UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM 10-K

(Mark One)

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2024

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to

Commission File Number 001-38360



Solid Biosciences Inc.

(Exact name of Registrant as specified in its Charter)

Delaware	**90-0943402**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
500 Rutherford Avenue, Third Floor	
Charlestown, MA	**02129**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 337-4680

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol**	**Name of exchange on which registered**
Common Stock $0.001 par value per share	SLDB	The Nasdaq Global Select Market

Securities registered pursuant to Section 12(g) of the Act:

None

(Title of class)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES ☐ NO ☒

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. YES ☐ NO ☒

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES ☒ NO ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). YES ☒ NO ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒	Smaller reporting company	☒
Emerging growth company	☐		

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☐

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to §240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Act). YES ☐ NO ☒

As of June 28, 2024, the last business day of the registrant's most recently completed second fiscal quarter, the aggregate market value of the registrant's common stock held by non-affiliates was $130.3 million, based on the last reported sale price of such stock on the Nasdaq Global Select Market as of such date.

The number of shares of the registrant's common stock outstanding as of March 3, 2025 was 77,492,959.

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Annual Report on Form 10-K includes forward-looking statements, which involve risks and uncertainties. These forward-looking statements can be identified by the use of forward-looking terminology, including the terms "believe," "estimate," "project," "anticipate," "expect," "seek," "predict," "aim," "continue," "possible," "intend," "may," "might," "will," "could," "would" or "should" or, in each case, their negative, or other variations or comparable terminology. These forward-looking statements include all matters that are not historical facts. They appear in a number of places throughout this Annual Report on Form 10-K. We derive many of our forward-looking statements from our operating budgets and forecasts, which are based upon many detailed assumptions. While we believe that our assumptions are reasonable, we caution that it is very difficult to predict the impact of known factors, and, of course, it is impossible for us to anticipate all factors that could affect our actual results. All forward-looking statements are based upon information available to us on the date of this Annual Report on Form 10-K.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- the timing, progress, design and results of ongoing and planned preclinical studies and clinical trials for our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates;
- our ability to establish or maintain collaborations or strategic relationships;
- our ability to obtain and maintain U.S. and foreign regulatory approval of our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates, and the timing and scope thereof;
- the timing and outcomes of regulatory interactions;
- the size of the patient populations and potential market opportunity for our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including capacity constraints and the scalability and commercial viability of our manufacturing methods and processes;
- our plans to develop and commercialize our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates, if approved;
- the pricing and reimbursement of our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates we may develop, if approved;
- the establishment of sales, marketing and distribution capabilities and entry into agreements with third parties to market and sell our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates, if approved;
- the rate and degree of market acceptance and clinical utility of our neuromuscular (e.g., SGT-003, SGT-212), cardiac (e.g., SGT-401, SGT-501, SGT-601) or other future candidates, if approved;
- our plans to develop our platform technologies;
- our expectations related to our use of capital resources;
- our estimates regarding expenses, ongoing losses, future revenue, capital requirements, and need for and ability to obtain additional financing;
- our intellectual property position;
- our competitive and market position;
- developments relating to our competitors and our industry;
- our ability to continue as a going concern; and
- the impact of laws, regulations, and global political and economic developments on our business, operations, strategy and goals.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition, business and prospects may differ materially from those made in or suggested by the forward-looking statements contained in this Annual Report on Form 10-K. In

addition, even if our results of operations, financial condition, business and prospects are consistent with the forward-looking statements contained in this Annual Report on Form 10-K, those results may not be indicative of results to be expected in subsequent periods.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to this Annual Report on Form 10-K completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements. The forward-looking statements contained in this Annual Report on Form 10-K are made as of the date of this Annual Report on Form 10-K, and we do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law.

This Annual Report on Form 10-K includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties as well as our own estimates of potential market opportunities based on our analysis of these data, research, surveys and studies. All of the market data used in this Annual Report on Form 10-K involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. Our estimates of the potential market opportunities for our candidates include a number of key assumptions based on our industry knowledge, industry publications and third-party research, surveys and studies, which may be based on a small sample size and fail to accurately reflect market opportunities. While we believe that our internal assumptions are reasonable, no independent source has verified such assumptions.

As used in this Annual Report on Form 10-K, the terms “Solid,” “the Company,” “we,” “us” and “our” refer to Solid Biosciences Inc., and its consolidated subsidiaries, unless the context indicates otherwise.

RISK FACTOR SUMMARY

Our business is subject to a number of risks that if realized could materially affect our business, operating results and financial condition and the trading price of our common stock could decline. These risks are discussed more fully below. These risks include the following:

- We have incurred significant net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future and may never achieve or maintain profitability.
- We will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.
- We have never generated revenue from product sales and do not expect to do so for the foreseeable future, if ever.
- Our limited operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.
- Unfavorable global economic conditions could harm our business, financial condition or results of operations.
- Our pipeline of gene transfer candidates, which we refer to collectively as our Candidates, utilize novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union.
- One of our prior clinical trials had been placed on clinical hold by the Food and Drug Administration, or FDA, in the past, and we cannot guarantee that similar events will not happen in ongoing, planned and future clinical trials for our Candidates.
- We have never completed a clinical trial and may be unable to do so for any Candidate, including SGT-003 and other Candidates.
- Our Candidates may cause one or more adverse events or other undesirable side effects or have properties that could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay, denial or withdrawal of regulatory approval by the FDA or other comparable foreign regulatory authorities, limit the Candidates commercial potential or result in significant negative consequences following any potential marketing approval.
- Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.

- Preliminary or interim data that we announce or publish from time to time may change as more data becomes available and are subject to audit and verification procedures that could result in material changes in the final data.
- We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.
- We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of SGT-003, SGT-212 or our other Candidates.
- Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize our Candidates and the approval may be for a narrower indication than we seek.
- We face significant competition and our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our ability to develop, successfully market or commercialize our Candidates. Changes within the competitive landscape could lead us to alter our clinical trial strategy, baseline eligibility criteria or make other modifications to clinical trial designs.
- We may not be successful in finding strategic collaborators for continuing development of our Candidates or platform technologies, or for successfully commercializing or competing in the market for certain indications.
- We have limited gene therapy manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-003, SGT-212, SGT-501, or other current and future Candidates. In addition, changes to manufacturing sites or processes, or formulations for our Candidates may result in additional cost or delay.
- We expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.
- Our gene transfer approach utilizes capsids derived from a virus, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of gene transfer Candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our Candidates.
- We heavily rely on certain in-licensed patents and other intellectual property rights in connection with our development of our Candidates and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize our Candidates.
- If we are unable to obtain and maintain patent protection for our Candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our Candidates may be adversely affected.
- Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.
- The price of our common stock has been, and in the future is likely to be, volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

PART I

Item 1. Business.

Overview

We are a life sciences company focused on advancing a portfolio of current and future gene therapy candidates, which we refer to collectively as our Candidates, including SGT-003 for the treatment of Duchenne muscular dystrophy, or Duchenne, SGT-212 for the treatment of Friedreich's ataxia, or FA, SGT-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia, or CPVT, SGT-601 for the treatment of TNNT2-mediated dilated cardiomyopathy, or TNNT2 DCM, and additional assets for the treatment of genetic cardiac and other diseases, at different stages of development, with varying levels of investment. We are advancing our diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, our mission is to improve the daily lives of patients living with these devastating diseases.

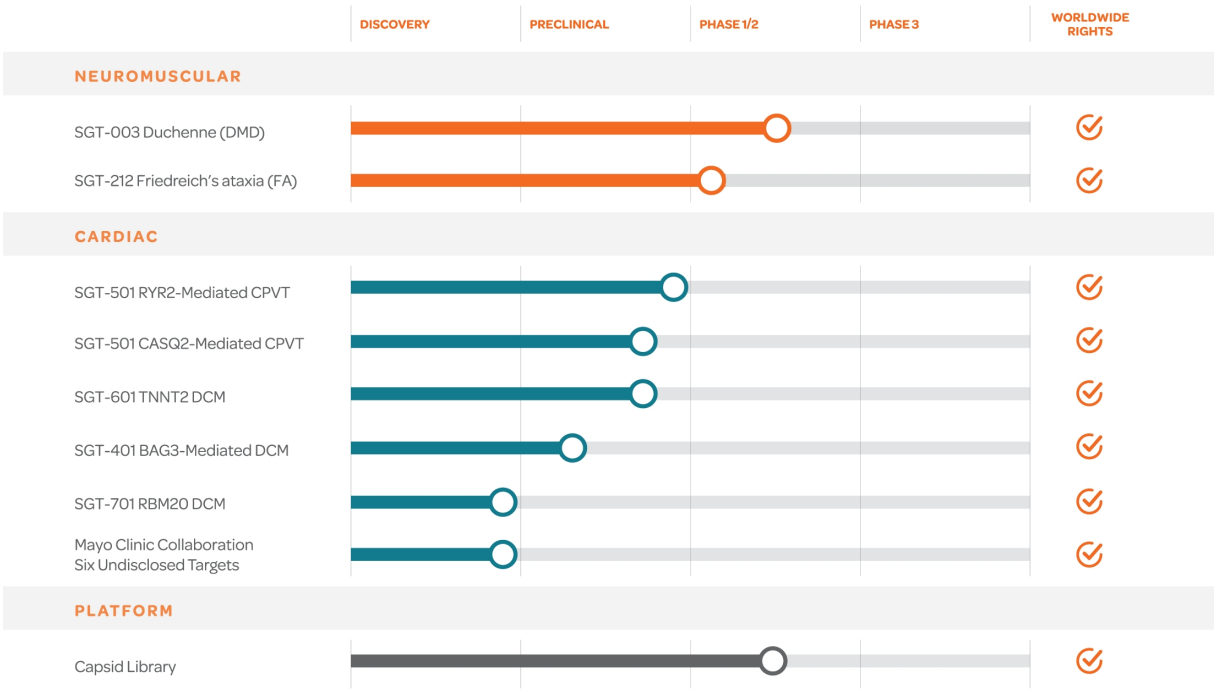
Solid was purpose-built to advance the best science and accelerate the discovery and development of treatments that may benefit all patients with Duchenne. As we expand to bring meaningful treatments to patients living with other neuromuscular and cardiac diseases, the values and guiding principles that drive us continue. Our corporate vision is to build an innovation platform enabling the discovery and development of high-value genetic medicines for neuromuscular and cardiac diseases by integrating internal capabilities, including a vector core, use of validated animal models, optimized expression cassettes, novel capsids and regulatory expertise, and collaborations with leaders in related clinical and research fields. Our mission, which guides our operations, is to treat and change the course of neuromuscular and cardiac diseases at all stages. Underscoring this mission, our disease-focused business model is founded on the following fundamental principles:

- identify and develop meaningful therapies for underserved patients with sometimes fatal neuromuscular and cardiac diseases;
- build innovative libraries of delivery capsids and other enabling technologies with the potential to have broad impact on the gene therapy field at large;
- bring together the leading experts in neuromuscular and cardiac diseases, science, technology, disease management and care; and
- be guided by the needs of these patients.

Our Pipeline

We are focused on developing transformative treatments to improve the lives of patients with rare neuromuscular and cardiac diseases. The majority of our current programs are designed to treat these diseases with gene transfer products. Gene transfer, a type of gene therapy, is designed to address diseases caused by mutated genes through the delivery of functional versions of genes, called transgenes. The transgenes are then utilized by the body to produce proteins that act therapeutically to treat the condition. In addition to a transgene, our gene transfer Candidates include a viral capsid or vector (a protein shell utilized as a vehicle to deliver a transgene to cells in the body) and a promoter (a specialized DNA sequence that directs cells to produce the protein in specific tissues). The capsid is modified to no longer self-replicate yet still retain its ability to introduce new genetic material directly into patients' cells. Adeno-associated virus, or AAV, capsids have been approved for use to deliver transgenes to patients, including via systemic delivery as well as stereotactic neurosurgical administration to the brain. The use of AAV capsids to deliver gene therapies has also been extensively studied by third parties in human clinical trials for multiple disease indications.

The following pipeline chart summarizes the development stages of our Candidates.



Neuromuscular Programs

About Duchenne Muscular Dystrophy

Duchenne is a genetic muscle-wasting disease predominantly affecting boys, with symptoms that usually manifest between three and five years of age. Duchenne is a progressive, irreversible, and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated prevalence of 5,000 to 15,000 cases in the United States alone. Duchenne is caused by mutations in the dystrophin gene, which result in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Dystrophin protein also serves as the cornerstone of the dystrophin glycoprotein complex, or DGC, a group of proteins that links the inner and outer components of muscle cells to ensure proper muscle function. Without functioning dystrophin and DGC, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. More than 1,000 dystrophin gene mutations, which can be inherited or can occur spontaneously, have been identified in people with Duchenne. By their early teens, Duchenne patients typically lose their ability to walk and become dependent on a wheelchair for mobility. By their 20s, patients essentially become paralyzed from the neck down and require a ventilator to breathe. Though disease severity and life expectancy vary, a patient's quality of life dramatically decreases over time, with death typically occurring by early adulthood from either cardiac or respiratory complications.

There is no cure for Duchenne. Glucocorticoid treatment, the current standard-of-care, has been shown to temporarily improve muscle strength, prolong the period of ambulation and slow the progression of Duchenne. However, glucocorticoid use is associated with well-known adverse side effects, including: severe weight gain, stunted growth, weakening of bone structure and metabolic dysfunctions, among others. The most commonly used glucocorticoids include prednisone and deflazacort (EMFLAZA).

Despite recent therapeutic advances, including the FDA approval of ELEVIDYS®, a gene transfer therapy for patients with Duchenne, Duchenne represents a significant societal and economic burden. The economic burden includes costs associated with hospital admissions, medication, frequent doctor visits and investment in assistive devices, as well as indirect costs, which account for approximately 45% of the total economic burden, related to productivity losses for the caregivers and costs due to pain, anxiety and social handicap. Only a small proportion of Duchenne patients are employed and many caregivers reduce their hours or stop working altogether to care for their children, who progressively require more help with everyday tasks, such as eating, dressing and using the bathroom. In some cases, patients also experience serious mental health issues that require additional support and treatment.

We are actively involved in engaging with the Duchenne patient, clinical and research communities to support advancement of therapies for patients with Duchenne. In November 2021, in collaboration with REGENXBIO Inc., we formally launched the Pathway Development Consortium, or the PDC, a multi-stakeholder initiative which aims to identify, develop, expand and maintain pathways to effective therapies for patients diagnosed early in life with rare diseases, including Duchenne. The PDC seeks to achieve these goals by bringing together a broad and diverse group of stakeholders from the rare disease and AAV gene therapy communities, including patients, industry, regulators, academia and payers, among others, for meaningful scientific and policy discussions.

SGT-003

Background

Our lead neuromuscular gene transfer candidate, SGT-003, is designed to address the underlying genetic cause of Duchenne by delivering a synthetic transgene that produces microdystrophin, a dystrophin-like protein that retains critical components of the full-size dystrophin gene, yet is small enough to fit within AAV packaging constraints, and is primarily expressed in muscles of the body, including skeletal, cardiac and respiratory muscles. AAV-SLB101, the capsid used in our Duchenne candidate, was generated by modifying a naturally occurring, non-pathogenic virus called AAV, with the goal of enhancing its ability to efficiently enter skeletal, diaphragm and cardiac muscle tissues.

Our microdystrophin is based on three decades of development and optimization work at the University of Missouri and the University of Washington as well as other academic institutions. In preclinical studies, the laboratories of Jeffrey Chamberlain, Ph.D., from the University of Washington, and Dongsheng Duan, Ph.D., from the University of Missouri, identified a proprietary configuration of genetic components that, when administered systemically, produces functional microdystrophin protein expression that not only stabilizes muscle membranes and protects muscle against injury, but also simultaneously restores the localization of DGC to the muscle membrane, notably increasing neuronal Nitric Oxide Synthase, or nNOS, concentration. In published studies, Dr. Duan and Dr. Chamberlain demonstrated in animal models that, in comparison to earlier configurations, nNOS-restoring microdystrophins were more effective in improving muscle function and resistance to fatigue.

We believe the unique functionality of our proprietary microdystrophin has the potential to result in functional benefits including diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle.

The expression of our microdystrophin is regulated by a modified, synthetic muscle-specific promoter cassette called CK8, which is derived from the naturally occurring muscle creatine kinase promoter. Regulatory cassettes, such as CK8, are used to drive gene expression specifically in muscle tissues. In comparison to other regulatory cassettes, CK8 is small in size and is able to drive microdystrophin transgene expression in skeletal, diaphragm and cardiac muscle tissues. In our preclinical studies in small and large animal models, CK8 restricted microdystrophin transgene expression to these muscles.

SGT-003 is a clinical-stage candidate designed to preserve muscle function in Duchenne patients after a single administration. SGT-003 utilizes an updated construct, combining our proprietary microdystrophin containing nNOS with AAV-SLB101, a novel, rationally designed capsid derived from AAV9 and designed for enhanced muscle tropism, reduced liver uptake and to more selectively deliver the drug to target tissue. We believe the SGT-003 construct is meaningfully differentiated from other approved and in development gene transfer candidates and may provide differentiated clinical benefit.

Preclinical Development

Following in vitro studies in mouse and human muscle cells, AAV-SLB101 was evaluated in a head-to-head study against AAV9 with the CK8-microdystrophin construct in the dystrophin-negative mouse model of Duchenne (mdx mouse). Separate groups of mice were administered a single intravenous dose of either construct, and the biodistribution, microdystrophin protein expression, and biomarker analyses were performed at the conclusion of the study. Overall, the in vivo study mdx mouse data supported the results seen from in vitro assays and further demonstrated the potential benefits of SGT-003. The mdx mice dosed with the novel AAV-SLB101 capsid showed increased biodistribution (vector genome copies) in representative muscle tissues and increased microdystrophin expression compared to those administered the AAV9 capsid. Additionally, there were lower vector genome copies observed in the liver compared to AAV9-administered mice, with the data supporting a preferential distribution of the novel capsid towards muscle tissue and away from the liver. These data supported the proof of concept for the novel capsid microdystrophin construct in Duchenne and formed a basis for establishing and advancing the SGT-003 program.

In April 2022, we released additional preclinical data from reporter transgene studies in non-human primates, or NHPs, and both mdx and wild type mice suggesting that AAV-SLB101 may have meaningful advantages for the delivery of

muscle-related gene therapies. Data from the NHP study, which used a reporter transgene in AAV-SLB101 demonstrated increased muscle tropism, decreased liver biodistribution and improved efficiency compared with AAV9. The results from the NHP study are consistent with the data from the reporter transgene studies in both mdx and wild type mouse models, which suggested improved muscle tropism and reduced liver uptake.

Clinical Development

Based on our preclinical data, we submitted an investigational new drug application, or IND, for SGT-003 to the FDA, which was cleared in November 2023. Patient dosing in the Phase 1/2 INSPIRE DUCHENNE trial of SGT-003 began in the second quarter of 2024. The INSPIRE DUCHENNE trial is a Phase 1/2 first-in-human, open-label, single-dose, multicenter trial designed to evaluate the safety, tolerability and efficacy of SGT-003 in pediatric patients with Duchenne at a dose of 1E14vg/kg. SGT-003 is administered as a one-time intravenous infusion. In September 2024, we amended the INSPIRE DUCHENNE clinical trial protocol to increase the anticipated participant enrollment size, expand the participant cohort age groups, and extend the time points of certain secondary objective measurements. In connection with the expanded clinical trial, we have initiated work for additional GMP batches of SGT-003.

Enrollment in the INSPIRE DUCHENNE trial is ongoing, with at least 10 total participants in the trial anticipated to be dosed by early in the second quarter of 2025 and approximately 20 total participants anticipated to be dosed by the fourth quarter of 2025. The seventh participant in the trial was dosed on February 17, 2025.

INSPIRE DUCHENNE currently has a total of six active clinical sites in the United States and Canada and approved clinical trial applications in the United Kingdom and Italy. We expect to activate additional trial sites by the end of 2025. In mid-2025, we plan to request a meeting with the U.S. Food and Drug Administration, or the FDA, to discuss the potential for accelerated approval regulatory pathways for SGT-003.

The FDA has granted orphan drug designation, Fast Track designation and Rare Pediatric Disease designation for SGT-003 for the treatment of Duchenne.

Initial Clinical Data from INSPIRE DUCHENNE Trial of SGT-003

On February 18, 2025, we announced positive initial data from the Phase 1/2 INSPIRE DUCHENNE trial as of the data cutoff date of February 11, 2025. Interim 90-day biopsy data reported in the first three participants showed an average microdystrophin expression of 110%, as measured by western blot, and improvements in multiple biomarkers that are indicators of muscle integrity, health and resilience.

The 90-day data reported as of the data cutoff date of February 11, 2025 includes: microdystrophin expression, measures of restoration and activation of key elements of the dystrophin-associated protein complex, key muscle integrity biomarker evaluation, in each case, from the first three participants dosed in the INSPIRE DUCHENNE trial, and interim safety findings from the first six participants dosed in the INSPIRE DUCHENNE trial. The first three participants are two 5-year-old boys and one 7-year-old boy at the time of dosing. The second three participants are a 6-year-old boy and two 7-year-old boys at the time of dosing.

Microdystrophin Expression and Other Measures at Day 90 (N=3)

	Mean (N=3)	Participant 1	Participant 2	Participant 3
Microdystrophin Expression % Normal (Western Blot)	110%	135%	112%	84%
Microdystrophin Expression % Normal (Mass Spectrometry)	108%	119%	152%	53%
% Dystrophin Positive Fibers (Immunofluorescence)	78%	77%	88%	70%
Vector Copies/Nucleus	18.7	19.8	28.6	7.6
nNOS (neuronal nitric oxide synthase) % Positive Fibers	42%	48%	53%	25%
Beta Sarcoglycan % Positive Fibers	70%	60%	88%	63%

Muscle Integrity Biomarker Evaluation at Day 90 (N=3)

- Mean reductions observed in markers of muscle injury and stress:
  - Serum creatine kinase (CK) (IU/L) -57%
  - Serum aspartate aminotransferase (AST) (IU/L): -45%

    - Serum alanine transaminase (ALT) (IU/L): -54%
    - Serum lactate dehydrogenase (LDH) (IU/L): -60%
- Mean reductions observed in markers of muscle breakdown and dystrophic regeneration:
    - Serum titin (pmol/L): -42%
    - Embryonic myosin heavy chain (eMHC) positive fibers: -59%

Measure of Cardiac Biomarkers:

- At Day 180, we observed a mean cardiac function increase of 8% (N=2) from baseline as measured by left ventricular ejection fraction (LVEF). The third participant had not reached Day 180 follow up as of the data cutoff date of February 11, 2025.
- We observed a reduction in serum cardiac hs-troponin I (hs-cTnI) of -36% at Day 90 in one participant who entered the trial with elevated hs-cTnI levels. Two of the first three participants entered the trial with normal baseline cTnI levels.
    - Two participants in total (N=6) had elevated troponin at baseline that reduced below initial baseline values post-dose.

Safety Update for the First Six Participants Dosed

SGT-003 has been well tolerated in the six participants dosed as of February 11, 2025. As of the cutoff date, the first six participants have reached at least 20 days post SGT-003 treatment. Adverse events observed after SGT-003 treatment were consistent with those observed in AAV gene therapy. No serious adverse events and no suspected unexpected serious adverse reactions were observed, and there was no evidence of thrombotic microangiopathy, atypical hemolytic uremic syndrome, or hemolysis. No AEs of acute liver injury were observed, including no elevated gamma-glutamyl transferase levels. All treatment-related AEs resolved with no sequelae and none of the AEs that were observed required the use of additional immunomodulatory agents such as eculizumab, sirolimus or rituximab. The most common AEs observed were nausea and vomiting; transient thrombocytopenia (including one Grade 3 episode that resolved within days without intervention and with no evidence of hemolysis observed); infusion related hypersensitivity reaction (including one Grade 3 episode of prolonged fever that resolved within days without intervention); and fever. One adverse event of special interest was observed, which was a Grade 1, mild, transient hs-troponin I elevation that resolved without intervention. There was no clinical evidence of myocarditis and no EKG or echocardiographic changes observed in such participant.

About Friedreich's ataxia

Friedreich's ataxia (FA) is a serious, life threatening, progressive multi system disease that is classically known to affect both cardiac and neurological systems but also involves endocrine, musculoskeletal and other organ systems. FA affects 1 in 40,000 people with an average onset between ages 10 and 15 and average lifespan of less than 40 years. It is estimated that approximately 5,000 patients in the United States and 25,000 patients in the European Union are affected by FA. The disease is due to autosomal recessive variants in the frataxin, or FXN, gene. Specifically, the majority of disease-causing variants are guanine-adenine-adenine expansion repeats located in intron 1 of the FXN gene on chromosome 9; the expansion range varies greatly from 70 to over 1700 repeats. This expansion causes a severe decrease in the expression of frataxin, a 210 amino-acid protein that is expressed as a precursor protein. Frataxin is imported into the mitochondrial matrix where it undergoes proteolytic cleavage to the 130 amino acid mature form, and is involved in iron-sulfur protein production, storage and transport. Alternatively, approximately 5% of patients will have another non-repeat variant in FXN. Frataxin, although ubiquitously expressed, exhibits tissue-specific differences in the levels of expression that partially correlate with sites of disease pathology. Dorsal root ganglia, or DRG, Purkinje and Granule cells in the cerebellum all exhibit high levels of expression. Frataxin expression is also high in non-neuronal tissues such as the heart and pancreas. FA is strongly associated with a high incidence of left ventricular, or LV, wall thickening, cardiomyopathy and diabetes. Frataxin is also highly expressed in tissues not affected in FA, such as liver, kidney and brown fat. FA is associated with a wide range of clinical manifestations including neurological and cardiovascular impact.

Neurological symptoms are key features and are highly penetrant in FA with ataxia and dysarthria being prominent features of the disease. Cerebellar lesions and sensory neuropathy result in ataxia and loss of balance. Visual and hearing impairments

also occur. It has been postulated that the onset of sensory neuropathy coincides with the beginning of inflammatory infiltration of the DRG. Modest reductions of frataxin are detrimental to the development of the spinal cord and DRG.

Cardiovascular complications are the most common cause of death in patients with FA, with comorbid cardiomyopathy found in at least 60% of fatalities. Cardiac involvement in FA manifests as cardiac LV wall thickening and resultant diastolic dysfunction. As the disease progresses, the heart becomes fibrotic and dilated, with damage to both the muscle and conduction system. Ultimately, FA cardiac involvement may result in reduced cardiac function (low ejection fraction), cardiac arrhythmias, heart failure, and death. As myocardial fibrosis is believed to be an irreversible end-stage complication, early intervention is key to preventing this most common cause of FA mortality.

There is no cure for FA. On February 28, 2023, Biogen's SKYCLARYS® (omaveloxolone) was the first drug approved in the United States and European Union for people with FA. While SKYCLARYS® positively effects cellular energy production, there continues to be significant unmet needs for patients with FA and the underlying cause of the disease, FXN deficiency is not addressed.

SGT-212

SGT-212 is a recombinant non-replicating AAVhu68 containing a codon-optimized cDNA that encodes FXN under control of the CB7 promoter and enhancer elements, flanked by AAV2 ITRs.

The vector capsid exhibits broad tissue tropism for effective transduction of a wide range of host cell types including cardiomyocytes and neurons. The expression of the cDNA is under the control of a ubiquitous CB7 promoter, which should lead to increased expression of FXN in many tissue types. Restoration of FXN levels is expected to address the underlying mitochondrial dysfunction, which is a hallmark of FA.

SGT-212 is intended for a dual route of administration: intradentate nuclei, or IDN, infusion using a magnetic resonance imaging (MRI) guided device followed by an IV infusion to increase therapeutic FXN levels in the cerebellar dentate nuclei, or DN, and in the cardiomyocytes, respectively. If functional FXN can be safely restored in these tissues, the mortality mediated by cardiovascular events as well as the neurologic and cardiovascular morbidity of the disease may be able to be treated simultaneously. Systemic administration in the non-clinical models resulted in poor penetration of the DN; therefore, we believe that a dual route of systemic and IDN infusion is needed to appropriately deliver the transgene to the desired tissues.

In January 2025, we announced that the FDA cleared our IND for SGT-212 for the treatment of FA. We anticipate initiating an open-label, multi-center Phase 1b clinical trial of SGT-212 in non-ambulatory and ambulatory adult patients living with FA in the second half of 2025. In the trial, SGT-212 will be administered by dual route of administration: via bilateral infusion to the DN followed by an IV infusion. The planned Phase 1b clinical trial is designed to evaluate the safety and tolerability of SGT-212. Exploratory objectives include assessment of preliminary efficacy and pharmacodynamic activity.

The FDA has granted Fast Track designation to SGT-212 for the treatment of FA.

Cardiac Programs

Genetic cardiac disease, or inherited cardiac conditions, is an umbrella term to describe cardiac diseases caused by mutations in one or more genes. Primary inherited arrhythmia syndromes present as abnormal cardiac arrhythmia, including life threatening ventricular arrhythmia, in the setting of structurally normal hearts and are in general genetically determined. Cardiomyopathy is a disease of the heart muscle that impairs the ability of the heart to pump blood to the rest of the body, resulting in arrhythmias, backup of blood into the lungs and other parts of the body, and ultimately heart failure. Forms of cardiomyopathy include DCM, hypertrophic and arrhythmogenic cardiomyopathy.

About CPVT

Our lead cardiac program is directed to the primary inherited arrhythmia syndrome CPVT. CPVT is a rare, serious and life-threatening disease which primarily manifests in children in the first and second decades of life, with the mean onset of CPVT symptoms being between seven and nine years of age. CPVT is an inherited cardiac arrhythmia syndrome characterized by adrenergically induced polymorphic arrhythmias in the presence of a normal resting sinus rhythm and a structurally normal heart. It is estimated that approximately 33,000 persons in the United States are affected by CPVT.

CPVT manifestations typically involve syncope, cardiac arrest and/or sudden cardiac death. The most common symptoms/signs include syncope (52-100%), cardiac arrest (8–48%), seizure-like events (40%), and hypoxic-ischemic encephalopathy (20%). CPVT is a significant cause of sudden death at a young age and mortality is high (historically up to 50% by the age of 35). Data from the recent Pediatric and Congenital Electrophysiology Society CPVT registry suggest that

three of every four children with CPVT present with life-threatening symptoms, which often occur during resting wakeful activities highlighting the unpredictable nature of CPVT.

To date, there are no medicines approved for the treatment of CPVT and management is directed toward manifestations of the disease with the goal of reducing arrhythmias or eliminating the incidence of life-threatening arrhythmias. Current treatments for CPVT include lifestyle management changes, such as restriction of rigorous physical exercise and avoidance of emotional distress, which are very challenging in the pediatric population, as well as pharmacotherapies requiring strict compliance (e.g. beta-blockers or flecainide alone, or as a combination therapy). Despite available pharmacotherapy options, the occurrence of breakthrough arrhythmia in approximately 30% of patients demonstrates that lifelong compliance is a critical problem. In addition, in some cases implantable cardioverter-defibrillators and/or left cardiac sympathetic denervation are used as treatment for symptomatic CPVT patients, but both are associated with attendant morbidity.

SGT-501

SGT-501 is a gene therapy candidate for the treatment of CPVT, which is caused by a gain of function mutation in the ryanodine receptor 2 (coded for by the RYR2 gene), referred to as RYR2-mediated CPVT. Mutations in RYR2 genes disrupt cardiac calcium, or Ca^{++}, release into the cytoplasm triggering abnormal contraction and relaxation leading to arrhythmias.

Our approach focuses on AAV-mediated therapeutic overexpression, or augmentation, of CASQ2, a calcium-binding protein which, through its role in Ca^{++}regulation, is integral to excitation-contraction coupling in the heart and in regulating the rate of heart beats. CASQ2 expression via AAV is intended to provide durable and continual protection from Ca^{++} leaks seen in patients with RYR2-mediated CPVT.

SGT-501 uses AAV8, a muscle tropic capsid, to deliver a functional CASQ2 transgene. Collectively, overexpression of CASQ2 in CPVT patients converges on a mechanism that drives buffering of free sarcoplasmic reticulum luminal calcium such that diastolic calcium leaks through the RYR2 into the cytosol are less likely. This mechanism of action is intended to support maintenance of normal cardiac rhythm and protect against triggered activity and arrhythmias.

Non-clinical mouse studies have demonstrated proof of concept for CASQ2 gene replacement using a recombinant AAV8 serotype capsid encoding CASQ2 to mitigate effects associated with an RYR2 mutation.

We have conducted preclinical studies of SGT-501, including three and six-month good laboratory practices, or GLP, toxicology studies. IND-enabling GLP toxicology studies of SGT-501 in non-human primates, including the in-life portion of the six-month toxicology study, were completed in the first quarter of 2025. We anticipate submitting an IND to the FDA for SGT-501 for the treatment of patients with RYR2-mediated CPVT in the first half of 2025.

The FDA and the European Medicines Agency, or EMA, have granted orphan drug designation for SGT-501 for the treatment of CPVT. The FDA recently granted Rare Pediatric Disease designation for SGT-501.

Other Cardiac Candidates

SGT-601

We are currently developing a preclinical stage product candidate, SGT-601, for the treatment of TNNT2 DCM. TNNT2 DCM is a rare cardiac disease characterized by mutations in the gene that codes for cardiac troponin T protein, which helps coordinate contraction of the heart muscle. TNNT2 gene mutations lead to reduced cardiac troponin T protein levels and DCM, which ultimately lead to heart failure. There are no approved therapies addressing the underlying cause of TNNT2 DCM. It is estimated that approximately 27,000 persons in the United States are affected by TNNT2 DCM. TNNT2 DCM is typically diagnosed between ages 20 and 50, with approximately 50% of onset seen by age 30, and has a mortality rate of 50%.

Our approach utilizes an AAV-delivered human TNNT2 transgene with a cardiac-selective promoter to restore functional levels of the troponin T protein. Preclinical studies in mice demonstrated SGT-601's ability to elicit robust, dose-dependent, cardiac-selective expression of human TNNT2 that was properly localized to the heart cell sarcomere. Efficacy studies in mice suggest that SGT-601 treatment resulted in a restoration of ejection fraction function and a stabilization in cardiac function over time. We anticipate submitting an IND to the FDA for SGT-601 for the treatment of TNNT2 DCM in the second half of 2026.

Other

We also have two cardiac pipeline gene transfer programs, SGT-401 for BAG3-mediated DCM, which is in early preclinical development, and SGT-701 for RBM20 DCM, which is in the discovery stage.

BAG3-mediated DCM is a rare cardiac disease characterized by mutations in the BAG3 gene, which codes for the BCL-2-associated athanogene 3, or BAG3, protein. Sufficient levels of functional BAG3 are required for healthy cardiac function. BAG3 gene mutations lead to reduced BAG3 protein levels and ultimately DCM. Deletions and truncations in the BAG3 protein that result in haplo-insufficiency have been associated with the development of DCM resulting from myofilament damage, poor contraction, left ventricular dysfunction, dilatation and heart failure.

RBM20 DCM is a rare inherited cardiac disease characterized by mutations in the RBM20 gene, a cardiac splicing factor that regulates alternative splicing and codes for RNA binding motif protein 20. RBM20 mutations can cause a clinically aggressive form of DCM that is correlated with high rates of heart failure, arrhythmias, and sudden cardiac death.

Platform Technologies

In addition to our gene transfer candidates, we have development programs focusing on platform technologies, including novel capsid libraries, genetic regulators including promoters, UTRs, and introns, immunomodulation technologies, manufacturing purity, and dual gene expression, a technology that allows us to package multiple transgenes into one capsid. These programs are part of our ongoing research efforts to develop innovative technologies that we believe may hold potential to translate into meaningful treatments, and drive future pipeline expansion, which we may seek to out-license to or develop through partnerships and collaborations with other biotechnology companies.

Novel Capsid Programs

Our novel capsid programs are directed toward developing skeletal muscle and cardiac capsid libraries using two approaches designed to enhance skeletal muscle and/or cardiac muscle tropism: rational design and directed evolution. Preclinical data in both wild-type and disease animal models demonstrated that we have developed a library of novel capsids that have shown increased muscle tropism with concomitant decreased liver biodistribution, resulting in improved efficiency compared to AAV9.

We have developed a rationally designed library of proprietary AAV capsids, including AAV-SLB101, the novel capsid used in SGT-003 for Duchenne, with the goal of improving skeletal muscle tropism. We approached this through the insertion of unique peptide sequences into traditional capsids and initially evaluated these candidates through an in vitro screening platform. The primary goal of developing this library was to generate capsids that preferentially target and transduce skeletal muscle cells, compared to traditional capsids such as AAV9. Candidate novel capsids were packaged with our microdystrophin transgene under the control of a muscle-specific promoter, such as CK8, and used to transduce muscle cells. In vitro studies performed in mouse muscle cell lines utilizing numerous novel capsid candidates showed multiple-fold increases in microdystrophin expression over AAV9. Further in vitro characterization of these capsids was performed in human Duchenne muscle cell lines. Results from these studies showed similar findings of multiple-fold increases in expression for novel capsid candidates over AAV9.

Non-specific novel capsids were packaged comprised of a bioluminescent protein (luciferase) under the control of a ubiquitous promoter in order to allow expression across a wide range of tissue types. These constructs were further evaluated in vivo in both mdx and wild-type mice to understand the potential broader applicability of these capsids for other indications. Results from this study support preferential targeting of muscle, with increases in biodistribution and expression over AAV9 across muscle tissues and decreased biodistribution and expression compared to AAV9 in the liver, and the potential applicability to a wide variety of indications that may benefit from such a targeting profile, in addition to Duchenne.

Using both directed evolution and rational design, we are developing multiple additional libraries of novel AAV capsids with the goal of enhancing cardiac tissue tropism and avoiding liver transduction.

We are using non-human primates, pigs, and mice as selection models to screen libraries at the level of RNA expression. Selection for effective transduction in cardiac tissue across different mammalian species is intended to identify capsid variants that potentially utilize conserved transduction mechanisms and may therefore be more likely to exhibit efficacy in humans. Our first cardiac-tropic capsid library has completed evaluation in all three mammalian species, with anticipated final capsid selection expected in the fourth quarter of 2025.

Manufacturing and Supply

Currently, we are working to develop and optimize a transient transfection manufacturing process for producing drug product for our current and future Candidates. This process will build on industry-wide accepted practices and is expected to increase the yield, robustness and scalability of our current methods.

SGT-003, SGT-212 and SGT-501 are manufactured using transient transfection which requires processing steps that are more complex than those required for most chemical pharmaceuticals. We also intend to use transient transfection manufacturing for our other Candidates. We selected a manufacturing process that we believe will be scalable to support clinical and commercial production needs for SGT-003, SGT-212 and SGT-501. The transient transfection process was selected to efficiently advance SGT-003, SGT-212 and SGT-501 along their respective development timelines.

We currently rely on third-party manufacturers for supply of SGT-003, SGT-212 and SGT-501 and plan to rely on third-party manufacturers for our other Candidates. In October 2021, we announced a partnership with a cell and gene therapy-focused contract development and manufacturing organization for the development and clinical stage manufacture of SGT-003. As part of our recently completed asset purchase from FA212 LLC, or FA212, we also acquired sufficient GMP clinical materials from FA212 to supply the planned Phase 1b clinical trial for SGT-212.

We are supplying, and expect to continue to supply, our ongoing and future preclinical and clinical development programs with drug produced at a cGMP compliant facility located at one of our contract manufacturing organizations. We ultimately intend to establish the capability and capacity to supply Candidates at commercial scale in alignment with program timeframes.

Intellectual Property

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection for our pipeline programs, our platform technologies and other know-how, to operate without infringing, misappropriating or otherwise violating the intellectual property rights of others, and to prevent others from infringing, misappropriating or otherwise violating our intellectual property rights. We also rely on patents, trade secrets, know-how, confidentiality procedures and agreements, and continuing technological innovation to develop and maintain our proprietary and intellectual property position.

We own and in-license various patents, patent applications, know-how and trade secrets relating to the development and commercialization of our gene therapy candidates and platform technologies. As of February 28, 2025, our patent portfolio includes both owned and in-licensed patent families relating to our gene therapy programs and platform technologies.

For some patents, substantive prosecution of our patent applications has not yet commenced at the U.S. Patent and Trademark Office, or USPTO. We cannot predict whether such pending patent applications will result in the issuance of patents that effectively protect our candidates and our platform technologies, or if such issued patents or any of our licensor's issued patents will effectively prevent others from commercializing competitive products. In any event, patent prosecution is a lengthy process, during which the scope of the claims initially submitted for examination by the patent offices in various jurisdictions are often significantly narrowed by the time they issue, if they issue at all.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent or patent application. The term of a patent that covers a drug or biological product may also be eligible for patent term extension when FDA approval is granted, subject to certain limitations and provided statutory and regulatory requirements are met (for more information, please see "Business— Government Regulation and Product Licensure —U.S. Patent Term Restoration"). In the future, if and when our candidates receive approval from the FDA or foreign regulatory authorities, we expect to apply for patent term extensions on issued patents we may obtain in the future covering those products, depending upon the length of the clinical trials for each product and other factors. There can be no assurance that any of our pending patent applications will issue or that we will benefit from any patent term extension or favorable adjustment to the term of any of our patents.

As with other biotechnology and pharmaceutical companies, our ability to maintain and solidify our proprietary and intellectual property position for our candidates will depend on our success in obtaining effective patent claims and enforcing those claims if granted. However, our owned and licensed pending patent applications, and any patent applications that we may in the future file or license from third parties may not result in the issuance of patents. We also cannot predict the breadth of claims that may be allowed or enforced in our patents. Any issued patents that we may receive in the future may be challenged, invalidated or circumvented. In addition, because of the extensive time required for clinical development and regulatory review of a candidate we may develop, it is possible that, before any of our candidates can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby limiting protection such patent would afford the respective product or products and any competitive advantage such patent may provide.

In addition to patents, we rely upon unpatented trade secrets and know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary information, in part, by executing

confidentiality agreements with our collaborators and scientific advisors, and non-competition, non-solicitation, confidentiality, and invention assignment agreements with our employees and consultants. We have also executed agreements requiring assignment of inventions with selected scientific advisors and collaborators. The confidentiality agreements we enter into are designed to protect our proprietary information and the agreements or clauses requiring assignment of inventions to us are designed to grant us ownership of technologies that are developed through our relationship with the respective counterparty. We cannot guarantee, however, that these agreements will afford us adequate protection of our intellectual property and proprietary information rights.

We also seek trademark protection in the United States and internationally where available and when appropriate. We currently own U.S. federal registrations for the marks SOLID, SOLID BIOSCIENCES and SOLID BIOSCIENCES logo, as well as registrations in the European Union, United Kingdom, Japan, and Hong Kong for the mark SOLID BIOSCIENCES, registrations in the European Union and United Kingdom for the marks SOLID BIOSCIENCES logo and SOLID GT. We also own pending trademark applications in the U.S. and in foreign jurisdictions for the mark AAVANTIBIO and a pending trademark application in the U.S. for the mark AAVANTIBIO logo.

Duchenne

Exclusive of our platform technologies, our Duchenne program includes three patent families with respect to microdystrophin and promoter sequences. We have filed one pending U.S. non-provisional patent application, and ten pending patent applications in foreign jurisdictions, and have also exclusively licensed three issued U.S. patents, one pending U.S. non-provisional patent application, and eighteen granted patents and seven pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire between 2028 and 2036, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent applications would be projected to expire between 2036 and 2042, excluding any patent term adjustments and any patent term extensions.

FA

Exclusive of our platform technologies, our FA program includes eight patent families. We have filed one pending U.S. non-provisional patent application, and have also exclusively licensed two U.S. patents, four pending U.S. non-provisional patent applications, and 16 pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire in 2036, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent applications would be projected to expire between 2036 and 2044, excluding any patent term adjustments and any patent term extensions.

CPVT

Exclusive of our platform technologies, our CPVT program includes two patent families. We have exclusively licensed four issued U.S. patents, one pending U.S. non-provisional patent application, 21 granted patents and six pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire in 2032, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent applications currently pending would be projected to expire in 2039, excluding any patent term adjustments and any patent term extensions.

Platform Technologies

We own or license patents, patent applications and know-how related to various platform technologies. Certain of these technologies may be applicable to one or more of our current or future gene therapy candidates.

Our capsid program includes two patent families related to modified AAV capsids. We have filed one pending U.S. patent application and ten pending patent applications in foreign jurisdictions. Any U.S. patents that may issue from the pending U.S. non-provisional patent application would be projected to expire in 2040, excluding any patent term adjustments and any patent term extensions.

Strategic Partnerships and Collaborations/Licenses

We have certain obligations under licensing agreements with third parties that include annual maintenance fees and payments that are contingent upon achieving various development, commercial and regulatory milestones. Pursuant to many of these license agreements, we are required to make milestone payments if certain development, regulatory and commercial sales milestones are achieved, and may have certain additional research funding obligations. Also, pursuant to the terms of many

of these license agreements, when and if commercial sales of a licensed product commence, we must pay royalties to our licensors on net sales of the respective licensed products.

FA212 LLC Asset Purchase Agreement

In September 2024, we entered into a purchase agreement with FA212 for the purchase of certain intellectual property including patents and assigned licenses, related to a preclinical drug candidate, which we now refer to as SGT-212, assigned manufacturing contracts as well as research and development materials such as manufactured materials and samples. The assets include an exclusive license for the gene therapy underlying SGT-212 from the University of Pennsylvania.

SGT-212 is designed as a dual-route administration AAV gene therapy aimed at treating the major manifestations of FA. SGT-212 is designed to be delivered via both IV and IDN infusion administration, which offers a novel approach to address FA's complex pathology.

Under the terms of the agreement, we made an upfront payment of $1.0 million to FA212. Additionally, FA212 is eligible to receive development milestone payments of up to $34.0 million and cumulative sales milestone payments of up to $21.0 million, upon achievement of specified milestone events, and tiered royalties on net sales in the low-single-digits. Certain development milestone payments are payable in either cash, equity, or a combination of both at our discretion. We also assumed from FA212 contingent development milestone payments of up to $4.2 million, regulatory milestone payments of up to $13.0 million, cumulative sales milestone payments of up to $27.5 million, and tiered royalties on worldwide net sales in the mid-single digits, each of which are payable to the University of Pennsylvania.

In February 2025, we made the first milestone payment of 975,496 shares of our common stock to FA212 following the FDA's clearance of our IND for SGT-212 for the treatment of FA.

As part of the asset purchase, we also acquired sufficient GMP clinical materials from FA212 to supply the planned Phase 1b clinical trial of SGT-212.

Maugeri License Agreement

In June 2023 we entered into a license agreement, or the Maugeri License Agreement, with ICS Maugeri S.p.A. SB, or Maugeri, to focus on our development and commercialization of cardiac-related products based on Maugeri's inventions. Pursuant to the Maugeri License Agreement, Maugeri granted us an exclusive worldwide sublicensable license in certain Maugeri patent rights, including existing patent rights, and those in any improvements or know-how made in performance of the Maugeri License Agreement, and a non-exclusive worldwide sublicensable license in certain Maugeri know-how, including existing know-how, and on any improvement thereto, in each case, subject to certain conditions, that is necessary or reasonably useful to develop licensed products under the terms of the Maugeri License Agreement. We will conduct certain activities agreed to by the parties with respect to the research and development of licensed products. A condition precedent to the effectiveness of the Maugeri License Agreement was regulatory review in Italy, which was completed in the third quarter of 2023 and, upon the completion of the condition precedent, the Maugeri License Agreement became effective.

We paid Maugeri an upfront license fee of €1.5 million, which was recorded as research and development expense during the second quarter of 2023. Additionally, we agreed to cumulative developmental, regulatory, and commercial milestone payments of up to €15.0 million, cumulative sales milestone payments of up to €15.0 million, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits. There were no milestones achieved during the years ended December 31, 2024 and December 31, 2023.

The Maugeri License Agreement continues until the latest expiry of (i) the last valid claim (as defined in the Maugeri License Agreement), (ii) regulatory exclusivity, and (iii) all payment obligations. Either party may terminate the Maugeri License Agreement for the other party's uncured material breach. We may also terminate the Maugeri License Agreement in our sole discretion upon 60 days' prior written notice to Maugeri and payment of a fee.

University of Washington License Agreement

In 2015, we entered into a license agreement with the University of Washington, acting through UW CoMotion, under which we obtained an exclusive, royalty-bearing, sublicensable, worldwide license under certain patent applications owned by the University of Washington relating to novel micro-dystrophins to develop, manufacture, and commercialize products for use in the treatment of Duchenne and related disease indications caused by a lack of functional dystrophin. We have the right to grant sublicenses to third parties contingent upon written approval by the University of Washington prior to executing such sublicense, which approval may not be unreasonably withheld.

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We are required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2024 and 2023. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017. In October 2020, the license agreement was amended such that we were required to pay the University of Washington $375 thousand in connection with the execution of the collaboration and license agreement, or the Collaboration Agreement, with Ultragenyx Pharmaceutical Inc., or Ultragenyx, in October 2020. This payment was recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that we are required to pay an aggregate of approximately $3.4 million upon the achievement of certain milestones. We must also pay royalties of a low single digit percentage of future sales by us and our sublicensees of products developed under the licensed patent rights. In addition, we must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to us and our sublicensees.

We are obligated to use our commercially reasonable efforts, consistent with sound and reasonable business practices and judgment, to commercialize the inventions covered by the licensed patent rights and to make and sell products based on that patent as soon as practicable and maximize sales thereof.

The University of Washington controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents, the University of Washington may prosecute and maintain such licensed patents at its own cost. We have the first right to enforce such licensed patents at our expense. However, we may not enter into any settlement in any manner relating to the licensed patents without the University of Washington's prior written consent.

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. We may terminate the agreement at any time upon providing sixty days' written notice to the University of Washington. The University of Washington may terminate the agreement upon our uncured, material breach of the agreement or if we enter into an insolvency-related event.

The University of Missouri License Agreement

In 2015, we entered into a license agreement with the Curators of the University of Missouri, or the University of Missouri, a public corporation of Missouri, under which we obtained an exclusive, royalty-bearing, sublicensable, worldwide license under certain patents and patent applications owned by the University of Missouri relating to a novel synthetic microdystrophin gene to make, sell and distribute products for use in the treatment of Duchenne and related disease indications resulting from a lack of functional dystrophin.

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We were required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones for each product developed based on the licensed patents.

Under the agreement, in the event we grant a sublicense to another party, we are required to pay the University of Missouri a percentage of the consideration received. The license agreement was amended such that we were required to pay, and did pay, the University of Missouri $0.8 million in February 2021 and $1.3 million in February 2022 as a result of the execution of the Collaboration Agreement with Ultragenyx in October 2020. These amounts were recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that we are required to make aggregate milestone payments of approximately $1.9 million upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2024 and 2023. We must pay a royalty of a low single digit percentage of future sales or by its sublicensees of products developed using the licensed patents. In addition, we must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

Under the agreement, we granted the University of Missouri a non-exclusive, royalty-free, irrevocable, paid-up license, with the right to grant sublicenses to non-profit, academic, educational or governmental institutions, to practice and use improvements made by us using the licensed patent rights, solely for non-commercial research purposes.

We are obligated to use our reasonable best efforts to introduce products based on the licensed patent rights into the commercial market as soon as possible, consistent with sound and reasonable business practices and judgment, and thereafter to keep such products reasonably available to the public.

The University of Missouri controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents, the University of Missouri may prosecute and maintain such licensed patents at its own cost. We have the first right to enforce such licensed patents at our expense. However, any settlement, consent judgment or other voluntary disposition of litigation that materially limits the scope, validity or enforceability of the licensed patent or admits fault or wrongdoing on the part of the University of Missouri must be pre-approved in writing by the University of Missouri. The license agreement remains in effect until the expiration of the last-to-expire patent or the abandonment of the last to be abandoned patent application licensed under the agreement. The University of Missouri may terminate the agreement, or render the license granted thereunder non-exclusive, in individual countries if we and our sublicensees fail to achieve certain milestones. We may terminate the license agreement at any time upon providing six months' written notice to the University of Missouri and paying a termination fee. Each of the University of Missouri and we may also terminate the agreement for an uncured default or breach of the agreement by the other party. Our ability to cure such breach only applies to the first two notices of such breach provided by the University of Missouri, and thereafter, the University of Missouri may terminate the agreement for our default or breach of the agreement upon thirty days' written notice without an opportunity to cure such default or breach.

University of Florida License Agreements

We, and our subsidiary AavantiBio Inc., or AavantiBio, have entered into several license agreements with the University of Florida Research Foundation, Inc., or UFRF. Broadly, the agreements relate to FA and certain early-stage cardiac candidates, including SGT-401, SGT-601 and SGT-701. Under each agreement we obtained an exclusive, royalty-bearing, sublicensable, world-wide license to certain patents and patent applications and a royalty-bearing non-exclusive license under the know-how, to make, have made, use, see, have sold, import and export licensed products. UFRF retains the right to practice the patent rights and know-how for internal non-commercial research, including research sponsored by commercial entities, and educational purposes.

In consideration for the rights granted under each agreement, AavantiBio paid a one-time non-refundable license fee. In connection with each agreement, we are required to pay an annual license maintenance fee until the first commercial sale of a licensed product after which time a minimum annual royalty will replace such maintenance fees. Under each agreement, we are required to reimburse UFRF for costs incurred in applying for, prosecuting and maintaining patents, pay up to an aggregate of approximately $2.9 million upon the achievement of certain intellectual property, clinical and regulatory milestones for each licensed product under the agreement and pay a low, single digit royalty on annual net sales by us and our sublicensees of licensed products on a licensed-product-by-licensed product basis. For any licensed product covered by both of these agreements, we are only obligated to make one payment for each milestone achieved and royalty payment due. Under each agreement, in the event we grant a sublicense to another party, we are required to pay UFRF a percentage of the consideration received.

Under each agreement, we have the right to grant sublicenses to third parties through multiple tiers, to the extent we are in compliance with our diligence obligations under the agreement and that sublicensee is subject to the terms of such agreement.

Under each agreement, we are obligated to use commercially reasonable efforts to develop and commercialize products covered by the licensed patent rights or know-how and to achieve certain regulatory and commercialization milestones within estimated time periods. Under the agreements entered in 2023 and 2024, we agreed to pay to UFRF cumulative sales milestones of up to $8.5 million and $27.0 million, respectively upon achievement of specified milestone events and tiered royalties on worldwide net sales in the low-to-mid-single digits.

Under each agreement, UFRF controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents in a particular country or jurisdiction, the license granted to such patent rights will terminate in such country or jurisdiction. We have the first right to enforce such licensed patents at our expense.

Each of the agreements terminates on a licensed product-by-licensed product basis on the later of: (i) expiration of the patent rights covering such licensed product or (ii) ten (10) years from the first commercial sale of such licensed product. After five years, we may terminate an agreement for any reason giving advance written notice and reason for termination. UFRF may terminate an agreement for our uncured default or breach of the agreement. UFRF may immediately terminate an agreement if we bring or assist others in bringing a patent challenge against the licensed patent rights. If UFRF sends us a written demand to terminate a sublicense agreement due to such sublicensee bringing or assisting a patent challenge, UFRF may terminate such agreement if we do not terminate the license with such sublicensee.

Mayo Clinic Collaboration and License Agreement

In December 2024, we entered into a collaboration, patent and know-how license agreement with the Mayo Foundation for Medical Education and Research, or Mayo, to further advance our research and development efforts in the field of genetic therapies, particularly for rare and debilitating cardiac diseases. This partnership focuses on leveraging Mayo's expertise in clinical research and our cutting-edge gene therapy platforms to explore innovative treatment options.

As part of the collaboration, we will be providing manufactured viral materials and chemistry, manufacturing and controls know-how while Mayo will be responsible for supporting all preclinical research through IND-enabling studies for six programs. We will then be responsible for the clinical development of programs chosen by us to develop. Under the terms of the collaboration, we and Mayo agreed to share intellectual property rights arising from the collaboration, while we retain exclusive rights to commercialize any resulting approved therapies.

In connection with the entry into the agreement, we made a one-time upfront payment of $0.6 million in cash to Mayo and issued 364,990 shares of our common stock to Mayo in December 2024. Additionally, Mayo is eligible to receive cumulative developmental and regulatory milestones of $7.0 million, cumulative sales milestone payments of up to $18.0 million, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-high-single-digits for each licensed product developed by us under the agreement. We have also agreed to pay an annual $0.6 million know-how access fee to Mayo.

The agreement also includes provisions for the potential sublicensing of certain intellectual property rights. We will also be responsible for reimbursing Mayo for costs incurred in the prosecution and maintenance of any patents resulting from the collaboration.

The collaboration remains in effect for the duration of the intellectual property rights associated with any therapies developed under the agreement. Either party may terminate the agreement with proper notice should specific terms be breached or should an insolvency-related event occur.

Competition

The biotechnology and pharmaceutical industries are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. This is also true in treatments of neuromuscular diseases, such as Duchenne and FA, cardiac diseases, such as CPVT, as well as in gene therapy. While we believe that our focus, strength of team, expertise in gene therapy, scientific knowledge and intellectual property provide us with competitive advantages, we face competition from several different sources, including large and small biopharmaceutical companies, academic research institutions, government agencies and public and private research institutions. Not only must we compete with other companies that are focused on gene transfer technology, but any product candidates that we successfully develop and commercialize will compete with existing therapies and new therapies that may become available in the future.

Many of our competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, clinical trials, regulatory approvals and product marketing than we do. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, enrolling patients in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We are aware of a number of companies and research institutions developing gene transfer programs progressing in Duchenne. For example, in June 2023, Sarepta Therapeutics, Inc., or Sarepta, announced that it had received accelerated approval for its gene therapy candidate ELEVIDYS® for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne. In June 2024 Sarepta announced an expanded US approval of ELEVIDYS® for patients who are at least 4 years of age including full approval for ambulatory Duchenne patients and accelerated approval for non-ambulatory Duchenne patients. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including, Genethon with a product candidate currently being evaluated in a Phase 1/2/3 clinical trial and REGENXBIO Inc. with a product candidate in Phase 1/2/3 clinical development.

We are also aware of a number of companies and research institutions developing gene transfer programs in FA. For example, Lexeo Therapeutics is developing an IV gene therapy to treat the cardiac manifestations of FA. Other competitors currently developing gene therapies to treat FA are in preclinical development, including Neurocrine Biosciences in collaboration with Voyager Therapeutics. We are also aware of other companies developing non-gene therapies for FA, such as Design Therapeutics, Larimar Therapeutics, and PTC Therapeutics who has a PDUFA target action date of August 19,

2025. Biogen's SKYCLARYS® (omaveloxolone) was approved for the treatment of FA in adults and adolescents aged 16 and older by the FDA and the European Commission in February 2023 and February 2024, respectively.

We are also aware of several companies and research institutions conducting clinical trials in small molecule product candidates focused on CPVT, including Armgo Pharmaceuticals, Inc. with an orally administered Rycal in a Phase 2 clinical trial and Cardurion Pharmaceuticals, Inc. with an orally administered CAMKII-delta inhibitor candidate in a Phase 2 clinical trial.

Government Regulation and Product Licensure

U.S. Government Regulation and Product Licensure

In the United States, biologic products including gene therapy products, such as our lead candidates, are licensed for marketing by the FDA under the Public Health Service Act, or PHS Act, and regulated by the FDA under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, as well as by other federal, state and local statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding rules and regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, recordkeeping, distribution, marketing, pricing, post-approval monitoring, reporting, advertising and other promotional practices involving biologic products. FDA approval must be obtained before conducting human clinical testing of biologic products. FDA must license a biologic product before it may be marketed within the United States.

U.S. Biologic Products Development Process

A company, institution, or organization which takes responsibility for the initiation and management of a clinical development program for such products is referred to as a sponsor. A sponsor seeking approval to market and distribute a new biological product in the United States must typically secure the following:

- completion of preclinical laboratory tests and in vivo studies according to the FDA's GLP requirements and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- design of a clinical protocol and submission to the FDA of an application for an IND, which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by an institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- approval by an institutional biosafety committee, or IBC, assessing the safety of the clinical research and identifying any potential risk to public health or the environment;
- performance of adequate and well controlled human clinical trials according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety, potency and purity of the proposed biologic product for each of its intended uses;
- preparation and submission to the FDA of a BLA for marketing approval that includes substantive evidence of safety, purity and potency from results of preclinical testing and clinical trials, and detailed information about the chemistry, manufacturing and controls, or CMC, for the product, reports of the outcomes and full data sets of the clinical trials and proposed labeling and packaging for the product;
- review of the product candidate by an FDA advisory committee, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biologic product candidate is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality and purity;
- potential FDA audit of the non-clinical and clinical trial sites that generated the data in support of the BLA;
- payment of user application and program fees;
- FDA review and licensure of the BLA for particular indications in the United States; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

Preclinical Studies and Investigational New Drug Application

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as non-clinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as in vivo studies to assess the potential safety and activity of the product candidate and to establish a rationale for therapeutic use. These studies are generally referred to as IND-enabling studies. The conduct of certain non-clinical studies must comply with federal regulations and requirements, including GLPs and the U.S. Department of Agriculture's Animal Welfare Act, if applicable.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, and long-term toxicity studies, may continue after the IND is submitted. With passage of the FDA's Modernization Act 2.0 in December 2022, Congress eliminated provisions in both the FDCA and PHSA that required animal testing in support of a BLA. While animal testing may still be conducted, the FDA was authorized to rely on alternative non-clinical tests, including cell-based assays, microphysiological systems or bioprinted or computer models.

An IND is an exemption from the FD&C Act that allows an unapproved product to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer an investigational product to humans. Such authorization must be secured prior to interstate shipment and administration of any product candidate that is not the subject of an approved BLA. In addition to reviewing an IND to assure the safety and rights of patients, the FDA also focuses on any CMC issues and the quality of the investigation. Some preclinical tests may continue even after the IND is submitted.

The IND becomes effective 30 days after receipt by the FDA, unless the FDA notifies the sponsor of deficiencies that require correction before human studies can begin. The sponsor cannot initiate studies until the FDA notifies the sponsor that the submitted corrections are satisfactory. The FDA may also place the clinical trial on a full clinical hold or partial clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin.

In addition, following the clearance of an IND, the FDA may impose a full or partial clinical hold at any time during clinical trials. A partial clinical hold is a delay or suspension of only part of the clinical work requested under the IND (e.g., a specific protocol or part of a protocol is not allowed to proceed; however, other protocols or parts of the protocol are allowed to proceed under the IND). If the FDA requires that progress to the next study is contingent on (i) FDA review of additional data and (ii) subsequent specific permission for the study to proceed, this represents a partial clinical hold.

Human Clinical Trials Under an IND

Clinical trials involve the administration of the biologic product candidate to healthy volunteers or subjects under the supervision of qualified investigators, generally physicians not employed by, or under the control of, the trial sponsor. Clinical trials are conducted under written study protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent.

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative, reviews and approves the study protocol and must monitor the clinical trial until completed.

Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring committee, or DMC. This group provides authorization as to whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study. Clinical trials involving recombinant DNA also must be reviewed by an IBC a local institutional committee that reviews and oversees basic and clinical research and utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials are typically conducted in three sequential phases that may overlap or be combined:

- *Phase 1*. The investigational biologic product is initially introduced into a small group of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients. Phase 1 clinical trials of gene therapies are typically conducted in patients rather than healthy volunteers.
- *Phase 2*. The biologic product candidate is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- *Phase 3*. Phase 3 clinical trials are commonly referred to as "pivotal" studies, which typically denotes a study that presents the data that the FDA or other relevant regulatory agency will use to determine whether or not to approve a biologic product. In Phase 3 clinical trials, the investigational biologic product is administered to an expanded patient population, generally at multiple geographically dispersed clinical trial sites in adequate and well controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes also referred to as post-marketing clinical trials, may be conducted after initial approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

A clinical trial may combine the elements of more than one phase and the FDA often requires more than one Phase 3 trial to support marketing approval of a product candidate. A company's designation of a clinical trial as being of a particular phase is not necessarily indicative that the study will be sufficient to satisfy the FDA requirements of that phase because this determination cannot be made until the protocol and data have been submitted to and reviewed by the FDA. Generally, pivotal trials are Phase 3 trials, but they may be Phase 2 trials if the design provides a well-controlled and reliable assessment of clinical benefit, particularly in an area of unmet medical need.

In December 2022, with the passage of Food and Drug Omnibus Reform Act, or FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. Specifically, action plans must include the sponsor's goals for enrollment, the underlying rationale for those goals, and an explanation of how the sponsor intends to meet them. In January 2024, the FDA issued draft guidance setting out its policies for the collection of race and ethnicity data in clinical trials. Unlike most guidance documents issued by the FDA, the diversity action plan guidance, when finalized, will have the force of the law because FDORA specifically dictates that the form and manner for submission of diversity action plans are specified in FDA guidance. In January 2025, in response to an executive order issued by President Trump on Diversity, Equity and Inclusion programs, the FDA removed this draft guidance from its website. The implications of this action are not yet known.

In June 2023, the FDA issued draft guidance with updated recommendations for GCPs aimed at modernizing the design and conduct of clinical trials. The updates are intended to help pave the way for more efficient clinical trials to facilitate the development of medical products. The draft guidance is adopted from the International Council for Harmonisation's recently updated E6(R3) draft guideline that was developed to enable the incorporation of rapidly developing technological and methodological innovations into the clinical trial enterprise. In addition, the FDA issued draft guidance outlining recommendations for the implementation of decentralized clinical trials.

The FDA or the sponsor or its DSMB/DMC may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biologic product candidate has been associated with unexpected serious harm to patients.

Finally, sponsors of certain clinical trials are required to register and disclose certain clinical trial information on a public registry (clinicaltrials.gov) maintained by the NIH. In particular, information related to the product, patient population, phase of investigation, study sites and investigators and other aspects of the clinical trial is made public as part of the registration of the clinical trial. The PHSA grants the Secretary of Health and Human Services the, or HHS, authority to issue a notice of noncompliance to a responsible party to failure to submit clinical trial information as required. The responsible party, however, is allowed 30 days to correct the noncompliance and submit the required information. Although sponsors are also obligated to disclose the results of their clinical trials after completion, disclosure of the results can be delayed in some cases for up to two years after the date of completion of the trial. As of December 19, 2024, the FDA has issued six notices of

non-compliance, signaling the government's willingness to enforce these requirements against non-compliant clinical trial sponsors. While these notices of non-compliance did not result in civil monetary penalties, the failure to submit clinical trial information to clinicaltrials.gov, as required, is a prohibited act under the FDCA with violations subject to potential civil monetary penalties of up to $10,000 for each day the violation continues. Violations may also result in injunctions and/or criminal prosecution or disqualification from federal grants.

Interactions with the FDA During the Clinical Development Program

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical trial investigators. Written IND safety reports must be promptly submitted to the FDA, the IRB and the investigators for serious and unexpected adverse events, any findings from other trials, in vivo laboratory tests or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

In addition, sponsors are given opportunities to meet with the FDA at certain points in the clinical development program. Specifically, sponsors may meet with the FDA prior to the submission of an IND, or pre-IND application meeting, at the end of a Phase 2 clinical trial, or EOP2 meeting, and before a BLA is submitted, or pre-BLA meeting. Meetings at other times may also be requested. There are five types of meetings that occur between sponsors and the FDA. Type A meetings are those that are necessary for an otherwise stalled product development program to proceed or to address an important safety issue. Type B meetings include pre-IND application and pre-BLA meetings, as well as Type B EOP2 meetings. A Type C meeting is any meeting other than a Type A or Type B meeting regarding the development and review of a product. A Type D meeting is focused on a narrow set of issues (and should be limited to no more than two focused topics) and should not require input from more than three disciplines or divisions. Finally, INTERACT meetings are intended for novel products and development programs that present unique challenges in the early development of an investigational product.

These meetings provide an opportunity for the sponsor to share information about the data gathered to date with the FDA and for the FDA to provide advice on the next phase of development. At the conclusion of these meetings, the FDA will typically provide its responses to questions posed by the sponsor regarding the clinical development program. The FDA will not indicate whether an NDA or BLA will be approved, but it will provide guidance to the sponsor on various questions, including whether an application should be submitted in the first place on the basis of the studies and data proposed by the sponsor. The agency may also generally express support for the sponsor's approach in the clinical development program but indicate that questions concerning whether the data support approval will be subject to review by the agency following its acceptance for filing of the NDA or BLA. The FDA has indicated that its responses, as conveyed in meeting minutes and advice letters, only constitute mere recommendations and/or advice made to a sponsor and, as such, sponsors are not bound by such recommendations and/or advice. Nonetheless, from a practical perspective, a sponsor's failure to follow the FDA's recommendations for design of a clinical program may put the program at significant risk of failure.

Clinical Studies Outside the United States in Support of FDA Approval

In connection with our clinical development program, we may conduct trials at sites outside the United States. When a foreign clinical trial is conducted under an IND, all IND requirements must be met unless waived. When a foreign clinical trial is not conducted under an IND, the sponsor must ensure that the study complies with certain regulatory requirements of the FDA in order to use the study as support for an IND or application for marketing approval. Specifically, the studies must be conducted in accordance with GCP requirements, including undergoing review and receiving approval by an independent ethics committee and seeking and receiving informed consent from subjects. GCP requirements encompass both ethical and data integrity standards for clinical trials. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical trials, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign trials are conducted in a manner comparable to that required for IND studies.

The acceptance by the FDA of study data from clinical trials conducted outside the United States in support of U.S. approval may be subject to certain conditions or may not be accepted at all. In cases where data from foreign clinical trials are intended to serve as the sole basis for marketing approval in the U.S., the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means.

In addition, even where the foreign study data are not intended to serve as the sole basis for approval, the FDA will not accept the data as support for an application for marketing approval unless the study is well-designed and well-conducted in accordance with GCP requirements and the FDA is able to validate the data from the study through an onsite inspection if deemed necessary. Many foreign regulatory authorities have similar approval requirements. In addition, such foreign trials are subject to the applicable local laws of the foreign jurisdictions where the trials are conducted.

Expanded Access to an Investigational Drug for Treatment Use

Expanded access, sometimes called "compassionate use," is the use of investigational new drug products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational drugs for patients who may benefit from investigational therapies. FDA regulations allow access to investigational drugs under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the drug under a treatment protocol or Treatment IND Application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical trials that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its investigational products available for expanded access; however, as required by the 21st Century Cures Act, or the Cures Act, passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase 2 or Phase 3 trial; or 15 days after the drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a drug manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act, but the manufacturer must develop an internal policy and respond to patient requests according to that policy.

Special Regulations and Guidance governing Gene Therapy Products

The FDA has defined a gene therapy product as one that mediates its effects by transcription and/or translation of transferred genetic material and/or by integrating into the host genome and which is administered as nucleic acids, viruses, or genetically engineered microorganisms. The products may be used to modify cells in vivo or transferred to cells ex vivo prior to administration to the recipient.

Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. CBER's Office of Therapeutic Products is responsible for the review of gene therapy and related products, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The NIH also advises the FDA on gene therapy issues and other issues related to emerging technologies. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documents must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA. However, many companies and other institutions, not otherwise subject to the NIH Guidelines, voluntarily follow them.

The FDA has issued various guidance documents regarding gene therapies, including final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, gene therapies for rare

diseases and gene therapies for retinal disorders, a final guidance in October 2022 for Human Gene Therapy for Neurodegenerative Diseases, as well as a draft guidance in July 2023 on comparability requirements for manufacturing changes in gene therapy products. In December 2023, a draft guidance on potency assurance for cellular and gene therapy products was released. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy product candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, for AAV capsids specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a 5-year period. Other types of gene therapy or gene editing products may require longer follow up, potentially up to a maximum 15-year period.

Finally, for a gene therapy product and where applicable, the FDA also will not approve the product if the manufacturer is not in compliance with good tissue practices, or GTP. These standards are found in FDA regulations and guidance that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue-based products, or HCT/Ps, which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that T-cell and tissue-based products are manufactured in a manner designed to prevent the introduction, transmission, and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing.

Pediatric Studies

Under the Pediatric Research Equity Act of 2003, or PREA, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the sponsor plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The sponsor, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the sponsor may request an amendment to the plan at any time.

For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of a sponsor, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments. In addition, the FDA will meet early in the development process to discuss pediatric study plans with sponsors and FDA must meet with sponsors by no later than the end-of-phase 1 meeting for serious or life-threatening diseases and by no later than 90 days after the FDA's receipt of the study plan.

The FDA may, on its own initiative or at the request of the sponsor, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. The FDA is required to send a PREA Non-Compliance letter to sponsors who have failed to submit their pediatric assessments required under PREA, have failed to seek or obtain a deferral or deferral extension or have failed to request approval for a required pediatric formulation. It further requires the FDA to publicly post the PREA Non-Compliance letter and sponsor's response. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation, although FDA has recently taken steps to limit what it considers abuse of this statutory exemption. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the Food and Drug Administration Safety and Innovation Act, or FDASIA. The FDA also maintains a list of diseases that are exempt from the requirements PREA, due to low prevalence of disease in the pediatric population. In May 2023, the FDA issued new draft guidance that further describes the pediatric study requirements under the PREA.

Compliance with cGMP Requirements

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products also must register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic, unannounced inspections by government authorities to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a government entity

placing restrictions on a product, manufacturer or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The PREVENT Pandemics Act, which was enacted in December 2022, clarifies that foreign drug manufacturing establishments are subject to registration and listing requirements even if a biologic undergoes further manufacture, preparation, propagation, compounding, or processing at a separate establishment outside the United States prior to being imported or offered for import into the United States.

Concurrent with clinical trials, companies usually complete additional preclinical studies and must also develop additional information about the physical characteristics of the biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other requirements, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

Submission and Filing of a BLA

After the completion of clinical trials of a biologic product, FDA licensure of a BLA must be obtained before commercial marketing of the biologic product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. In addition, under the PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biologic product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers.

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. Under federal law, the submission of most BLAs is subject to an application user fee, which for federal fiscal year 2025 is approximately $4.3 million for an application requiring clinical data. The sponsor of an approved BLA is also subject to an annual program fee, which for federal fiscal year 2025 is currently $403,889 per eligible prescription product. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

The FDA reviews a BLA within 60 days of submission to determine if it is substantially complete before the agency accepts it for filing, and it must so notify the sponsor of that determination within the 60 days. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In the event that FDA determines that an application does not satisfy this standard, it will issue a Refuse to File, or RTF, determination to the sponsor. The BLA may be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA.

With filing of the application, the FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Moreover, the FDA will review a sponsor's financial relationship with the principal investigators who conducted the clinical trials in support of the BLA. That is because, under certain circumstances, principal investigators at a clinical trial site may also serve as scientific advisors or consultants to a sponsor and receive compensation in connection with such services. Depending on the level of that compensation and any other financial interest a principal investigator may have in a sponsor, the sponsor may be required to report these relationships to the FDA. The FDA will then evaluate that financial relationship and determine whether it creates a conflict of interest or otherwise affects the interpretation of the trial or the integrity of the data generated at the principal investigator's clinical trial site. If so, the FDA may exclude data from the clinical trial site in connection with its determination of safety and efficacy of the investigational product.

During the biologic product approval process, the FDA also will determine whether a REMS, is necessary to assure the safe use of the biologic product. REMS use risk minimization strategies beyond the professional labeling to ensure that the

benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events and whether the product is a new molecular entity. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

In connection with its review of a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements to ensure the integrity of the clinical data. cGMP, GLP and GCP compliance requires significant expenditure of time, money and effort in the areas of training, recordkeeping, production and quality control.

With passage of FDORA, Congress clarified FDA's authority to conduct inspections by expressly permitting inspection of facilities involved in the preparation, conduct, or analysis of clinical and non-clinical studies submitted to FDA as well as other persons holding study records or involved in the study process.

Decisions on a BLA

After evaluating the application and all related information, including the advisory committee recommendations, if any, and inspection reports of manufacturing facilities and clinical trial sites, the FDA will issue either a Complete Response Letter, or CRL, or an approval letter. To reach this determination, the FDA must determine that the expected benefits of the proposed product outweigh its potential risks to patients. This "benefit-risk" assessment is informed by the extensive body of evidence about the product in the BLA.

A CRL indicates that the review cycle of the application is complete, and the application will not be approved in its present form. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. A CRL generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. The deficiencies identified may be minor, for example, requiring labeling changes; or major, for example, requiring additional clinical trials. Additionally, the CRL may include recommended actions that the sponsor might take to place the application in a condition for approval. If a CRL is issued, the sponsor may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application. For those seeking to challenge FDA's CRL decision, the agency has indicated that sponsors may request a formal hearing on the CRL or they may file a request for reconsideration or a request for a formal dispute resolution.

If a product receives regulatory approval, the FDA will issue an approval letter. The approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing or dispensing in the form of a REMS, or otherwise limit the scope of any approval. In addition, the FDA may require post-marketing clinical trials, designed to further assess a biologic product's safety, purity and potency, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

The FDA has agreed to specified performance goals in the review of BLAs under the PDUFA. One such goal is to review standard BLAs in ten months after the FDA accepts the BLA for filing, and priority BLAs in six months, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date.

Biosimilars and Reference Product Exclusivity

The Patient Protection and Affordable Care Act and the companion Health Care and Education Reconciliation Act, or the Health Care Reform Law, which was signed into law on March 23, 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. That Act established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars. To date, the FDA has approved a number of biosimilars and it has also issued numerous guidance documents outlining its approach to reviewing and licensing biosimilars and interchangeable biosimilars under the PHS Act.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the agency must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. In December 2022, Congress clarified through FDORA that FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the first day on which such a product is approved as interchangeable with the reference product.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of first licensure of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was first licensed. Even if a product is considered to be a reference product eligible for regulatory exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. There have been recent government proposals to reduce the 12-year reference product regulatory exclusivity period, but none has been enacted to date. Since passage of the BPCIA, many states have passed laws or amendments to laws, which address pharmacy practices involving biosimilar products.

As of December 27, 2020 (enacted as part of the Consolidated Appropriations Act, 2021), the "patent dance" lists became public information as listed in the Purple Book (FDA's "Database of Licensed Biological Products"). In particular, reference product BLA holders must submit to the FDA within 30 days of exchanging a patent list (patents with expiry dates) with a biosimilar applicant, as well as any supplemental lists. This information was previously maintained as confidential as between the BLA holder and biosimilar applicant. Despite publication of these lists, a BLA holder may assert other patents against future filers, and does not exclude enforcement of newly granted patents.

Additionally, under the Act, the FDA must now publish in the Purple Book the following information about patented biological products:

- a list of each biological product, by nonproprietary name, for which a biologics license is in effect;
- the date of licensure and the application number;
- the licensure status and, as available, the marketing status; and
- exclusivity periods.

The FDA must publish in the Purple Book all of the above information in the first instance within 180 days of enactment and update every 30 days.

Pediatric Exclusivity

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of regulatory exclusivity to the term of any existing regulatory exclusivity, including reference product and orphan exclusivity. This six-month exclusivity may be granted if an application sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity cover the product are extended by six months. Thus, pediatric exclusivity adds six months to existing exclusivity periods applicable to biological products under the BPCIA—namely, the four-year period during which the FDA will not consider an application for a biosimilar product, and the 12-year regulatory exclusivity period during which the FDA will not approve a biosimilar application.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product). Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent

and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. In addition, the FDA may not approve other applications to market the same drug or biologic product for the same indication for seven years unless the sponsor of the other product demonstrates that its product is clinically superior to the product with orphan drug exclusivity. Under Omnibus legislation enacted in December 2020, this clinical superiority requirement applies to drugs and biologics that received orphan drug designation before enactment of the FDA Reauthorization Act in 2017, but have not yet been approved or licensed by FDA.

Orphan exclusivity does not block the approval of a different product for the same rare disease or condition, nor does it block the approval of the same product for different indications. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA issued final guidance in September 2021 suggesting that it would not consider two gene therapy products to be different drugs solely based on minor differences in the transgenes or capsids. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity. Orphan medicinal product status in the European Union has similar, but not identical, benefits.

In September 2021, the Court of Appeals for the 11th Circuit held that, for the purpose of determining the scope of market exclusivity, the term “same disease or condition” in the statute means the designated “rare disease or condition” and could not be interpreted by the FDA to mean the “indication or use.” Thus, the court concluded, orphan drug exclusivity applies to the entire designated disease or condition rather than the “indication or use.” Although there have been legislative proposals to overrule this decision, they have not been enacted into law. In January 2023, FDA announced that, in matters beyond the scope of that court order, FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. More recently however, on February 14, 2025, a federal district court in Washington, DC fully embraced the reasoning of the court decision in another decision challenging the scope of orphan drug exclusivity. The implications of this decision, and its impact on the FDA’s implementation of the Orphan Drug Act, are unclear at this point.

Expedited Development and Review Programs

The FDA is authorized to expedite the review of BLAs in several ways. Under the Fast Track program, the sponsor of a biologic product candidate may request the FDA to designate the product for a specific indication as a Fast Track product concurrent with or after the filing of the IND. Biologic products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product candidate and the specific indication for which it is being studied. In addition to other benefits, such as the ability to have greater interactions with the FDA, the FDA may initiate review of sections of a Fast Track BLA before the application is complete, a process known as rolling review.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as breakthrough therapy designation, priority review and accelerated approval.

- *Breakthrough therapy designation.* To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient drug development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.
- *Priority review.* A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting product reaction, documented enhancement of patient compliance that may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. FDA aims to complete its review of priority review applications within six months as opposed to 10 months for standard review.

- *Accelerated approval.* Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

  With passage of FDORA, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to FDA every six months (until the study is completed) and use expedited procedures to withdraw accelerated approval of a BLA if certain conditions are not met, including where the confirmatory trial fails to verify the product's clinical benefit or where evidence demonstrates the product is not shown to be safe or effective under the conditions of use. The FDA may also use such procedures to withdraw an accelerated approval if a sponsor fails to conduct any required post-approval trial of the product with due diligence, including with respect to "conditions specified by the Secretary." The new procedures include the provision of due notice and an explanation for a proposed withdrawal, and opportunities for a meeting with the FDA Commissioner or the Commissioner's designee and a written appeal, among other things. In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to accelerated approval. Subsequently, in December 2024 and January 2025, the FDA issued additional draft guidance relating to accelerated approval. This guidance describes the FDA's views on what it means to conduct a confirmatory trial with due diligence and how the agency plans to interpret whether such a study needs to be underway at the time of approval. While this guidance is currently only in draft form and will ultimately not be legally binding even when finalized, sponsors typically observe the FDA's guidance closely to ensure that their investigational products qualify for accelerated approval.

- *Regenerative advanced therapy.* With passage of the Cures Act in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

None of these expedited programs change the standards for approval but they may help expedite the development or approval process of product candidates.

Rare Pediatric Disease Designation and Priority Review Vouchers

In 2012, Congress enacted the FDASIA, requiring the FDA to award priority review vouchers, or PRVs, to sponsors of certain rare pediatric disease product applications. This program is designed to encourage development of new drug and biological products for prevention and treatment of "rare pediatric diseases" by, upon initial approval of an application meeting certain specified criteria, providing companies with a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease product receiving a PRV may sell or otherwise transfer the voucher to another company. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted an application relying on the priority review voucher. The FDA may also revoke any PRV if the rare pediatric disease product for which the voucher was awarded is not marketed in the United States within one year following the date of approval.

In order to receive a PRV upon BLA approval, the product must receive designation from the FDA as a product for a rare pediatric disease prior to submission of the marketing application. A "rare pediatric disease" is a disease that is serious or life-threatening, in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and affects fewer than 200,000 people in the United States, or affects more than 200,000 people in the United States but there is no reasonable expectation that the cost of developing and making available in the United States a product for such disease or condition will be recovered from sales in the United States of such product. In addition to receiving rare pediatric

disease designation, in order to receive a PRV, the BLA must be given priority review, rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population, not seek approval for a different adult indication in the original rare pediatric disease product application and be for a product that does not include a previously approved active ingredient.

The Rare Pediatric Disease PRV program was scheduled to expire after September 30, 2020. After that, only drugs designated as rare pediatric treatments and approved by the FDA by October 1, 2022, could receive a voucher. In December 2020, however, Congress renewed the program as part of the 2021 Coronavirus Response and Relief Supplemental Consolidated Appropriations Act through the federal fiscal year 2024. Thus, under the current statutory sunset provisions, FDA may only award PRVs for approved rare pediatric disease product applications if sponsors have rare pediatric disease designation for the drug granted by September 30, 2024. The FDA may not award any rare pediatric disease PRVs after September 30, 2026.

Post-Approval Requirements

After regulatory approval of a product is obtained, there may be a number of post-approval requirements. For example, as a condition of approval of a BLA, the FDA may require post-marketing testing and surveillance to monitor the product's safety or efficacy. In addition, holders of an approved BLA are required to keep extensive records, to report certain adverse reactions and production problems to the FDA, to provide updated safety and efficacy information and to comply with requirements concerning advertising and promotional labeling for their products. Also, quality control and manufacturing procedures must continue to conform to cGMP regulations and practices, as well as the manufacturing conditions of approval set forth in the BLA. Changes to the manufacturing process are strictly regulated, and, depending on the significance of the change, may require prior regulatory approval before being implemented. Regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon us and any third-party manufacturers that we may decide to use. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP requirements, which impose certain procedural, substantive and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

Further, although physicians may prescribe legally available products for unapproved uses or patient populations, which are commonly referred to as "off-label uses," manufacturers may not market or promote such uses. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. In September 2021, the FDA published final regulations which describe the types of evidence that the FDA will consider in determining the intended use of a biologic. If a company is found to have promoted off-label uses, it may become subject to administrative and judicial enforcement by the FDA, the Department of Justice, or DOJ, or the Office of the Inspector General of the HHS, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

It may be permissible, under very specific, narrow conditions, for a manufacturer to engage in nonpromotional, non-misleading communication regarding off-label information, such as distributing scientific or medical journal information. For example, in January 2025, the FDA published final guidance outlining its policies governing the distribution of scientific information to healthcare providers about unapproved uses of approved products. The final guidance calls for such communications to be truthful, non-misleading and scientifically sound and to include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use of the approved product. If a company engages in such communications consistent with the guidance's recommendations, the FDA indicated that it will not treat such communications as evidence of unlawful promotion of a new intended use for the approved product. Moreover, with passage of the Pre-Approval Information Exchange Act in December 2022, sponsors of products that have not been approved may proactively communicate to payors certain information about products in development to help expedite patient access upon product approval. Previously, such communications were permitted under FDA guidance but the new legislation explicitly provides protection to sponsors who convey certain information about products in development to payors, including unapproved uses of approved products.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, and its implementing regulations, as well as the Drug Supply Chain Security Act, or DSCSA, which regulate the distribution and tracing of prescription drug samples at the federal level, and set minimum standards for the regulation of drug distributors by the states. The PDMA, its implementing regulations and state laws limit the distribution of prescription pharmaceutical product samples, and the DSCSA imposes requirements to ensure accountability in distribution and to identify and remove counterfeit and other illegitimate products from the market. Manufacturers were required by November 2023 to have such systems and processes in place to comply with the DSCSA, but, so as not to disrupt supply chains, the

FDA has granted certain exemptions from enhanced drug distribution security requirements for eligible trading partners for particular periods of time.

U.S. Patent Term Restoration

Depending upon the timing, duration and specifics of FDA approval of product candidates, some of a sponsor's U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent terms lost during product development and FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period generally is one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biologic product is eligible for the extension, the application for the extension must be submitted prior to the expiration of the patent, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Moreover, a given patent may only be extended once based on a single product. The USPTO in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Federal and State Data Privacy Laws

There are multiple privacy and data security laws that may impact our business activities, in the United States and in other countries where we conduct trials or where we may do business in the future. These laws are evolving and may increase both our obligations and our regulatory risks in the future. In the health care industry generally, under The Health Insurance Portability and Accountability Act, or HIPAA, the HHS has issued regulations to protect the privacy and security of protected health information used or disclosed by covered entities including certain healthcare providers, health plans, and healthcare clearinghouses. HIPAA also regulates standardization of data content, codes and formats used in healthcare transactions and standardization of identifiers for health plans and providers. HIPAA also imposes certain obligations on the business associates of covered entities that obtain protected health information in providing services to or on behalf of covered entities. HIPAA may apply to us in certain circumstances and may also apply to our business partners in ways that may impact our relationships with them. Our clinical trials will be regulated by the Common Rule, which also includes specific privacy-related provisions. In addition to federal privacy regulations, there are a number of state laws governing confidentiality and security of health information that may be applicable to our business. In addition to possible federal civil and criminal penalties for HIPAA violations, state attorneys general are authorized to file civil actions for damages or injunctions in federal courts to enforce HIPAA and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, state attorneys general (along with private plaintiffs) have brought civil actions seeking injunctions and damages resulting from alleged violations of HIPAA's privacy and security rules. State attorneys general also have authority to enforce state privacy and security laws. New laws and regulations governing privacy and security may be adopted in the future as well.

In addition to potential enforcement by HHS, we are also potentially subject to privacy enforcement from the FTC. The FTC has been particularly focused on the unpermitted processing of health and genetic data through its recent enforcement actions and is expanding the types of privacy violations that it interprets to be "unfair" under Section 5 of the FTC Act, as well as the types of activities it views to trigger the Health Breach Notification Rule (which the FTC also has the authority to enforce). The agency is also in the process of developing rules related to commercial surveillance and data security that may impact our business. We will need to account for the FTC's evolving rules and guidance for proper privacy and data security practices in order to mitigate our risk for a potential enforcement action, which may be costly. If we are subject to a potential FTC enforcement action, we may be subject to a settlement order that requires us to adhere to very specific privacy and data security practices, which may impact our business. We may also be required to pay fines as part of a settlement (depending on the nature of the alleged violations). If we violate any consent order that we reach with the FTC, we may be subject to additional fines and compliance requirements. Finally, both the FTC and HHS's enforcement priorities (as well as those of other federal regulators) may be impacted by the change in administration and new leadership. These shifts in enforcement priorities may also impact our business.

There are also increased restrictions at the federal level relating to transferring sensitive data outside of the United States to certain foreign countries. For example, in 2024, Congress passed H.B. 815, which included the Protecting Americans' Data from Foreign Adversaries Act of 2024. This law creates certain restrictions for entities that disclose sensitive data (including potential health data) to countries such as China. Failure to comply with these rules can lead to a potential FTC enforcement action. Additionally, the DOJ recently finalized a rule implementing Executive Order 14117, which creates similar

restrictions related to the transfer of sensitive US data to countries such as China. These data transfer restrictions (and others that may pass in the future) may create operational challenges and legal risks for our business.

In 2018, California passed into law the California Consumer Privacy Act, or the CCPA, which took effect on January 1, 2020, and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or the GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or the CPRA, which went into effect on January 1, 2023, and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency—the California Privacy Protection Agency—whose sole responsibility is to enforce the CPRA, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, a number of states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime over the next several years. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data, which includes health data in some cases. Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering or have already passed comprehensive privacy laws that will go into effect over the next several years. Other states will be considering similar laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. For example, the State of Washington passed the My Health My Data Act in 2023 which specifically regulated health information that is not otherwise regulated by the HIPAA rules, and the law also has a private right of action, which further increases the relevant compliance risk. Connecticut and Nevada have also passed similar laws regulating consumer health data, and more states are considering such legislation. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Plaintiffs' lawyers are also increasingly using privacy-related statutes at both the state and federal level to bring lawsuits against companies for their data-related practices. In particular, there have been a significant number of cases filed against companies for their use of pixels and other web trackers. These cases often allege violations of the California Invasion of Privacy Act and other state laws regulating wiretapping, as well as the federal Video Privacy Protection Act. The rise in these types of lawsuits creates potential risk for our business.

Because of the breadth of these laws and the narrowness of the statutory exceptions and regulatory safe harbors available under such laws, it is possible that some of our current or future business activities, including certain clinical research, sales and marketing practices, and the provision of certain items and services to our customers, could be subject to challenge under one or more of such privacy and data security laws. The heightening compliance environment and the need to build and maintain robust and secure systems to comply with different privacy compliance and/or reporting requirements in multiple jurisdictions could increase the possibility that a healthcare company may fail to comply fully with one or more of these requirements. If our operations are found to be in violation of any of the privacy or data security laws or regulations described above that are applicable to us, or any other laws that apply to us, we may be subject to penalties, including potentially significant criminal, civil, and administrative penalties, damages, fines, contractual damages, reputational harm, diminished profits and future earnings, additional reporting requirements and/or oversight if we become subject to a consent decree or similar agreement to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations. To the extent that any product candidates we may develop, once approved, are sold in a foreign country, we may be subject to similar foreign laws.

Government Regulation Outside of the U.S.

In addition to regulations in the United States, a manufacturer is subject to a variety of regulations in foreign jurisdictions to the extent it chooses to sell any products in those foreign countries. Even if a manufacturer obtains FDA approval of a product, it must still obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Because biologically sourced materials are

subject to unique contamination risks, their use may also be restricted in some countries. As in the United States, medicinal products can be marketed only if a marketing authorization from the competent regulatory agencies has been obtained. Similar to the United States, the various phases of preclinical and clinical research in the European Union are subject to significant regulatory controls.

With the exception of the European Union and European Economic Area, or EEA, applying the harmonized regulatory rules for medicinal products, the approval process and requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly between countries and jurisdictions and can involve additional testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Non-clinical Studies

Non-clinical studies are performed to demonstrate the health or environmental safety of new chemical or biological substances. Non-clinical (pharmaco-toxicological) studies must be conducted in compliance with the principles of good laboratory practice as set forth in EU Directive 2004/10/EC (unless otherwise justified for certain particular medicinal products – e.g., radio-pharmaceutical precursors for radio-labeling purposes). In particular, non-clinical studies, both in vitro and in vivo, must be planned, performed, monitored, recorded, reported and archived in accordance with the GLP principles, which define a set of rules and criteria for a quality system for the organizational process and the conditions for non-clinical studies. These GLP standards reflect the Organization for Economic Co-operation and Development requirements.

Clinical Trial Approval in the European Union

On January 31, 2022, the new Clinical Trials Regulation (EU) No 536/2014, or CTR, became effective in the European Union and replaced the prior Clinical Trials Directive 2001/20/EC. The new regulation aims at simplifying and streamlining the authorization, conduct and transparency of clinical trials in the European Union. Under the new coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one Member State of the European Union, or EU Member State, will only be required to submit a single application for approval. The submission will be made through the Clinical Trials Information System, a new clinical trials portal overseen by the EMA and available to clinical trial sponsors, competent authorities of the EU Member States and the public.

Beyond streamlining the process, CTR includes a single set of documents to be prepared and submitted for the application as well as simplified reporting procedures for clinical trial sponsors, and a harmonized procedure for the assessment of applications for clinical trials, which is divided in two parts. Part I is assessed by the competent authorities of all EU Member States in which an application for authorization of a clinical trial has been submitted (Member States concerned). Part II is assessed separately by each Member State concerned. Strict deadlines have been established for the assessment of clinical trial applications. The role of the relevant ethics committees in the assessment procedure will continue to be governed by the national law of the concerned EU Member State. However, overall related timelines will be defined by the CTR.

The CTR did not change the preexisting requirement that a sponsor must obtain prior approval from the competent national authority of the EU Member State in which the clinical trial is to be conducted. If the clinical trial is conducted in different EU Member States, the competent authorities in each of these EU Member States must provide their approval for the conduct of the clinical trial. Furthermore, the sponsor may only start a clinical trial at a specific study site after the applicable ethics committee has issued a favorable opinion.

The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. Clinical trials for which an application was submitted (i) prior to January 31, 2022 under the Clinical Trials Directive, or (ii) between January 31, 2022 and January 31, 2023 and for which the sponsor has opted for the application of the Clinical Trials Directive were governed by said directive until January 31, 2025. Beginning January 31, 2025, all clinical trials (including those which are ongoing) are subject to the provisions of the CTR. The failure to transition ongoing clinical trials to the Clinical Trials Regulation can result in corrective measures under Article 77 of the Clinical Trials Regulation, including revocation of the authorization of the clinical trial or suspension of the clinical trial as well as criminal sanctions and fines under national law of EU Member States.

Parties conducting certain clinical trials must post clinical trial information in the European Union at EudraCT website: https://eudract.ema.europa.eu.

PRIME Designation

In March 2016, the EMA, launched the PRIority MEdicines, or PRIME, initiative to foster research and development of medicines that may offer a major therapeutic advantage over existing treatments, or benefit patients without treatment options. PRIME aims to strengthen clinical trial designs to facilitate the generation of high-quality data for the evaluation of an application for marketing authorization. To be accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on preclinical and/or early clinical data. These medicines are considered priority medicines within the European Union.

After an investigational candidate has been selected for PRIME, developers are assigned a rapporteur from the Committee for Human Medicinal Products, or CHMP, to provide continuous support and help to build knowledge ahead of a marketing authorization application, or MAA. A multidisciplinary group of experts will provide broader guidance on the overall development plan and regulatory strategy of the product. Companies are also eligible for accelerated assessment at the time of their regulatory application.

Pediatric Studies

Sponsors developing a new medicinal product must agree upon a Pediatric Investigation Plan, or PIP, with the EMA's pediatric committee, or PDCO, and must conduct pediatric clinical trials in accordance with that PIP, unless a waiver applies (e.g., because the relevant disease or condition occurs only in adults). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which marketing authorization is being sought. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted by the PDCO of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults, in which case the pediatric clinical trials must be completed at a later date.

Marketing Authorization

In the European Union, marketing authorizations for medicinal products may be obtained through several different procedures founded on the same basic regulatory process.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid across the European Economic Area (i.e., the European Union as well as Iceland, Liechtenstein and Norway). The centralized procedure is compulsory for medicinal products produced by certain biotechnological processes, products designated as orphan medicinal products, and products with a new active substance indicated for the treatment of certain diseases. It is optional for those products that are highly innovative or for which a centralized process is in the interest of patients. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional written or oral information is to be provided by the sponsor in response to questions asked by the CHMP. Accelerated evaluation may be granted by the CHMP in exceptional cases. These are defined as circumstances in which a medicinal product is expected to be of a "major public health interest." Three cumulative criteria must be fulfilled in such circumstances: the seriousness of the disease, such as severely disabling or life-threatening diseases, to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In these circumstances, the EMA ensures that the opinion of the CHMP is given within 150 days.

The EMA's Committee for Advanced Therapies, or CAT, is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. Advanced-therapy medical products include gene therapy medicine, somatic cell therapy medicines and tissue-engineered medicines. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant EU guidelines. The EMA may issue new guidelines concerning the development and marketing authorization for somatic cell therapy medicinal products and require that we comply with these new guidelines. Similarly, complex regulatory environments exist in other jurisdictions in which we might consider seeking regulatory approvals for our product candidates, further complicating the regulatory landscape. As a result, the procedures and standards applied to gene therapy products and cell therapy products may be applied to any of our gene therapy or genome editing candidates, but that remains uncertain at this point.

Specifically, the grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC includes specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products, and tissue

engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to EMA which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

The decentralized procedure provides for approval by one or more other concerned EU Member States of an assessment of an application for marketing authorization conducted by one EU Member State, known as the reference EU Member State. In accordance with this procedure, a sponsor submits an application for marketing authorization to the reference EU Member State and the concerned EU Member States. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States which, within 90 days of receipt, must decide whether to approve the assessment report and related materials.

If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States. In accordance with the mutual recognition procedure, the sponsor applies for national marketing authorization in one EU Member State. Upon receipt of this authorization the sponsor can then seek the recognition of this authorization by other EU Member States. Authorization in accordance with either of these procedures will result in authorization of the medicinal product only in the reference EU Member State and in the other concerned EU Member States.

A marketing authorization may be granted only to a sponsor established in the European Union. Regulation No. 1901/2006 provides that, prior to obtaining a marketing authorization in the European Union, a sponsor must demonstrate compliance with all measures included in a PIP approved by the PDCO, covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, class waiver, or a deferral for one or more of the measures included in the PIP.

Conditional Marketing Authorization

In specific circumstances, European Union legislation on Conditional Marketing Authorizations for Medicinal Products for Human Use, or conditional marketing authorization, enables sponsors to obtain a conditional marketing authorization prior to obtaining the comprehensive clinical data required for an application for a full marketing authorization. Such conditional approvals may be granted for product candidates (including medicines designated as orphan medicinal products) if the risk-benefit balance of the product candidate is positive, it is likely that the sponsor will be in a position to provide the required comprehensive clinical trial data, the product fulfills unmet medical needs and the benefit to public health of the immediate availability on the market of the medicinal product concerned outweighs the risk inherent in the fact that additional data are still required. A conditional marketing authorization may contain specific obligations to be fulfilled by the marketing authorization holder, including obligations with respect to the completion of ongoing or new studies, and with respect to the collection of pharmacovigilance data.

Conditional marketing authorizations are valid for one year, and may be renewed annually, if the risk-benefit balance remains positive, and after an assessment of the need for additional or modified conditions and/or specific obligations. The timelines for the centralized procedure described above also apply with respect to the review by the CHMP of applications for a conditional marketing authorization.

Exceptional Circumstances

A marketing authorization may also be granted “under exceptional circumstances” when the applicant can show that it is unable to provide comprehensive data on the efficacy and safety under normal conditions of use even after the product has been authorized and subject to specific procedures being introduced. This may arise in particular when the intended indications are very rare and, in the present state of scientific knowledge, it is not possible to provide comprehensive information, or when generating data may be contrary to generally accepted ethical principles. This marketing authorization is similar to the conditional marketing authorization as it is reserved to medicinal products to be approved for severe diseases or unmet medical needs and the applicant does not hold the complete data set legally required for the grant of a marketing authorization. However, unlike the conditional marketing authorization, the applicant does not have to provide the missing data and will never have to. Although the marketing authorization “under exceptional circumstances” is granted definitively, the risk-benefit balance of the medicinal product is reviewed annually and the MA is withdrawn in case the risk-benefit ratio is no longer favorable. Under these procedures, before granting the marketing authorization, the EMA or the competent authorities of the EU Member States make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety, and efficacy.

Orphan Drug Designation and Exclusivity

The criteria for designating an orphan medicinal product in the European Union are similar in principle to those in the United States. Under Article 3 of Regulation (EC) 141/2000, a medicinal product may be designated as orphan if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition, (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the European Union to justify investment and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition. The term "significant benefit" is defined in Regulation (EC) 847/2000 to mean a clinically relevant advantage or a major contribution to patient care.

Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. During this ten year market exclusivity period, the EMA or the competent authorities of the Member States of the European Economic Area, or EEA, cannot accept an application for a marketing authorization for a similar medicinal product for the same indication. A similar medicinal product is defined as a medicinal product containing a similar active substance or substances as contained in an authorized orphan medicinal product, and which is intended for the same therapeutic indication. The application for orphan designation must be submitted before the application for marketing authorization. The sponsor will receive a fee reduction for the marketing authorization application if the orphan designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The ten-year market exclusivity in the European Union may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product for the same indication at any time if: (1) the second sponsor can establish that its product, although similar, is safer, more effective or otherwise clinically superior; (2) the sponsor consents to a second orphan medicinal product application; or (3) the sponsor cannot supply enough orphan medicinal product.

Pediatric Exclusivity

Products that are granted a marketing authorization with the results of the pediatric clinical trials conducted in accordance with the PIP are eligible for a six month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) even where the trial results are negative. In the case of orphan medicinal products, a two-year extension of the orphan market exclusivity may be available. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

Brexit and the Regulatory Framework in the United Kingdom

The United Kingdom's withdrawal from the European Union took place on January 31, 2020. As of January 1, 2021, the Medicines and Healthcare products Regulatory Agency, or the MHRA, became responsible for supervising medicines and medical devices in Great Britain, comprising England, Scotland and Wales under domestic law whereas Northern Ireland continues to be subject to EU rules under the Northern Ireland Protocol as amended by the "Windsor Framework". As of January 1, 2025, the MHRA is responsible for approving all medicinal products destined for the UK market (i.e., Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. The MHRA relies on the Human Medicines Regulations 2012 (SI 2012/1916) (as amended), or the HMR, as the basis for regulating medicines. The HMR has incorporated into the domestic law the body of EU law instruments governing medicinal products that pre-existed prior to the UK's withdrawal from the European Union.

As of January 1, 2024, a new international recognition procedure, or IRP, applies which intends to facilitate approval of pharmaceutical products in the UK. The IRP is open to applicants that have already received an authorization for the same product from one of the MHRA's specified Reference Regulators, or RRs. The RR notably include EMA and regulators in the EEA member states for approvals in the EU centralized procedure and mutual recognition procedure as well as the FDA (for product approvals granted in the U.S.). The RR assessment must have undergone a full and standalone review. RR assessments based on reliance or recognition cannot be used to support an IRP application. A CHMP positive opinion or a positive end of procedure outcome is an RR authorization for the purposes of IRP.

As with other issues related to withdrawal of the UK from the European Union, there are open questions about how personal data will be protected in the UK and whether personal information can transfer from the European Union to the UK. Following the withdrawal of the UK from the European Union, the UK Data Protection Act of 2018 applies to the processing

of personal data that takes place in the UK and includes parallel obligations to those set forth by the GDPR. While the Data Protection Act of 2018 in the UK that "implements" and complements the GDPR has achieved Royal Assent on May 23, 2018 and is now effective in the UK, it is still unclear whether transfer of data from the EEA to the UK will remain lawful under the GDPR. The UK government has already determined that it considers all EU and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the UK to the EU/EEA remain unaffected. In addition, a recent decision from the European Commission appears to deem the UK as being "essentially adequate" for purposes of data transfer from the European Union to the UK, although this decision may be re-evaluated in the future.

General Data Protection Regulation

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including personal health data, is subject to the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR.

Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield framework, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the United States. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the United States.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which would serve as a replacement to the EU-U.S. Privacy Shield. The European Commission initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022 and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the European Union to the United States. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue may further impact our business operations in the EU.

Following the withdrawal of the United Kingdom from the European Union, the U.K. Data Protection Act 2018 applies to the processing of personal data that takes place in the United Kingdom and includes parallel obligations to those set forth by GDPR, including in relation to data transfers.

Healthcare Law and Regulation

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, reporting of payments to physicians and teaching physicians and patient privacy laws and regulations and other healthcare laws and regulations that may constrain business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be

presented, to the federal government, claims for payment that are false, fictitious or fraudulent or knowingly making, using or causing to made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Health Care Reform Law, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the United States Department of Health and Human Services, information related to payments and other transfers of value made by that entity to physicians, other healthcare professionals and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Pharmaceutical Insurance Coverage and Health Care Reform

In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated health care costs. Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Thus, even if a product candidate is approved, sales of the product will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage and establish adequate reimbursement levels for, the product. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged, examining the medical necessity and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the Health Care Reform Law, which, among other things, includes changes to the coverage and payment for products under government health care programs. In addition, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013. Under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022,

made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory Pay-As-You-Go Act of 2010 sequester for two years, through the end of 2024. Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Consolidated Appropriations Act's health care offset title includes Section 4163, which extends the two percent Budget Control Act of 2011 Medicare sequester for six months into 2032 and lowers the payment reduction percentages in years 2030 and 2031.

The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our candidates for which we may obtain regulatory approval or the frequency with which any such candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%.

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the Patient Protection and Affordable Care Act, or PPACA, brought by several states without specifically ruling on the constitutionality of the PPACA. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

Pharmaceutical Prices

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to pharmaceutical pricing, review the relationship between pricing and manufacturer patient programs, and reduce the costs of pharmaceuticals under Medicare and Medicaid.

In addition, in October 2020, HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program, or SIP, to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Several states have passed laws allowing for the importation of drugs from Canada. Several states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. In January 2024, the FDA approved Florida's plan for Canadian drug importation. Florida now has authority to import certain products from Canada for a period of two years once certain conditions are met. Florida will first need to submit a pre-import request for each product selected for importation, which must be approved by the FDA. Florida will also need to relabel the products and perform quality testing of the products to meet FDA standards.

Further, on November 20, 2020, HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The final rule would also eliminate the current safe harbor for Medicare drug rebates and create new safe harbors for beneficiary point-of-sale discounts and pharmacy benefit manager service fees. It originally was set to go into effect on January 1, 2022, but with passage of the Inflation Reduction Act, or the IRA, has been delayed by Congress to January 1, 2032.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation; and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Medicare Part D drugs in 2027, 15 Medicare Part B or Part D drugs in 2028, and 20 Medicare Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a

single rare disease or condition. In August 2024, HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions and the prices will become effective January 1, 2026. In January 2025, CMS announced the next 15 drug and biologic prices that will be subject to the IRA's price negotiation provisions. CMS issued a public statement on January 2025, declaring that lowering the cost of prescription drugs is a top priority of the new administration and CMS is committed to considering opportunities to bring greater transparency in the negotiation program.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $2,000 a year beginning in 2025.

The IRA includes a provision exempting orphan drugs from Medicare price negotiation but this exclusion has been interpreted by CMS in final guidance issued in July 2023 to apply only to those orphan drugs with an approved indication (or indications) for a single rare disease or condition. The final guidance clarifies that CMS will consider only active designations/approvals when evaluating a drug for the exclusion, such that designations/indications withdrawn before the selected drug publication date will not be considered. CMS also clarified that, if a drug loses its orphan drug exclusion status, the agency will use the earliest date of approval/licensure to determine whether the product is a qualifying single source drug subject to price negotiations.

In addition, the IRA potentially raises legal risks with respect to individuals participating in a Medicare Part D prescription drug plan who may experience a gap in coverage if they required coverage above their initial annual coverage limit before they reached the higher threshold, or "catastrophic period" of the plan. Individuals requiring services exceeding the initial annual coverage limit and below the catastrophic period, must pay 100% of the cost of their prescriptions until they reach the catastrophic period. Among other things, the IRA contains many provisions aimed at reducing this financial burden on individuals by reducing the co-insurance and co-payment costs, expanding eligibility for lower income subsidy plans, and price caps on annual out-of-pocket expenses, each of which could have potential pricing and reporting implications.

On June 6, 2023, Merck & Co. filed a lawsuit against the HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. There have been various decisions by the courts considering these cases since they were filed. We expect that litigation involving these and other provisions of the IRA will continue, with unpredictable and uncertain results.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. A number of states, for example, require drug manufacturers and other entities in the drug supply chain, including health carriers, pharmacy benefit managers, wholesale distributors, to disclose information about pricing of pharmaceuticals. This is increasingly true with respect to products approved pursuant to the accelerated approval pathway. State Medicaid programs and other payers are developing strategies and implementing significant coverage barriers, or refusing to cover these products outright, arguing that accelerated approval drugs have insufficient or limited evidence despite meeting the FDA's standards for accelerated approval. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular product candidate to currently available therapies or so-called health technology assessments, in order to obtain reimbursement or pricing approval. For example, the European Union provides options for its EU Member States to restrict the range of products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. Member States may approve a specific price for a product, or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the product on the market. Other EU Member States allow companies to fix their own prices for products but monitor and control prescription volumes and issue guidance to physicians to limit prescriptions. Recently, many countries in the European Union have increased the amount of discounts required on pharmaceuticals and these efforts could continue as countries attempt to manage health care expenditures, especially in light of the severe fiscal and debt crises experienced by many countries in the European Union. The downward pressure on health care costs in general, particularly prescription products, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue

after reimbursement has been obtained. Reference pricing used by various EU Member States, and parallel trade, i.e., arbitrage between low-priced and high-priced EU Member States, can further reduce prices. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any products, if approved in those countries.

Environmental Regulations

We are also subject to regulation under the Occupational Safety and Health Act, the Environmental Protection Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and other present and potential federal, state or local regulations. These and other laws govern our use, handling and disposal of various biological and chemical substances used in, and waste generated by, our operations. Our research and development involves the controlled use of hazardous materials, chemicals and viruses.

Human Capital

We recognize that attracting, motivating and retaining talented employees is vital to our success. We value the health and wellness of our employees and their families. It is our goal to deliver innovative programs that provide choice, quality and value. We aim to create an equitable, inclusive and empowering environment in which our employees can grow and advance their careers, with the overall goal of developing, expanding and retaining our workforce to support our current pipeline and future business goals. Our success also depends on our ability to attract, engage and retain a talented group of employees. Our efforts to recruit and retain a talented and passionate workforce with different experiences, perspectives and backgrounds include providing competitive compensation and benefits packages.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards. We offer a comprehensive benefits program that provides resources to help employees manage their health, finances, and life outside of work.

As of December 31, 2024, we employed 100 full-time employees, including 65 in research and development and 35 in general and administrative positions, and of which 24 of our employees hold Ph.D. or M.D. degrees.

Corporate Information

Our principal executive offices are located at 500 Rutherford Avenue, Third Floor, Charlestown, Massachusetts 02129 and our telephone number is (617) 337-4680. Our website address is www.solidbio.com. The information contained in, or accessible through, our website does not constitute a part of this Annual Report on Form 10-K.

We make available free of charge on our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC.

Item 1A. Risk Factors.

You should carefully consider the following risk factors, in addition to the other information contained in this Annual Report on Form 10-K, including the section of this report titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes. If any of the events described in the following risk factors and the risks described elsewhere in this Annual Report on Form 10-K occurs, our business, operating results and financial condition could be seriously harmed and the trading price of our common stock could decline. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this Annual Report on Form 10-K. The risks and uncertainties described below are not the only ones we face. Additional risks not presently known to us or other factors not perceived by us to present significant risks to our business at this time also may impair our business operations.

Risks Related to our Financial Position and Need for Capital Requirements

We have incurred significant net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future and may never achieve or maintain profitability.

Since inception, we have incurred significant net losses. Our net losses were $124.7 million and $96.0 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had an accumulated deficit of $783.5 million. Prior to our acquisition of AavantiBio, Inc., or the Acquisition, we devoted substantially all of our efforts to research and development, including clinical development of SGT-001, which we are no longer developing, and preclinical development of SGT-003, as well as to building out our management team and infrastructure. Following the Acquisition, we also began devoting efforts to preclinical development of our other Candidates, clinical development of SGT-003, as well as building out our management team. We expect that it could be several years before we have a commercialized product, and we may never have a commercialized product. We expect to continue to incur significant expenses and see continued operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if, and as, we:

- continue to enroll patients in our INSPIRE DUCHENNE trial and advance clinical development of SGT-003;
- advance our other Candidates into clinical trials, including our planned Phase 1b clinical trial of SGT-212 in adult patients with FA;
- continue research and preclinical development of our Candidates and adjacent technologies such as assays;
- seek to identify additional Candidates;
- engage in regulatory interactions with the FDA and other regulatory authorities;
- submit regulatory filings relating to the development of our Candidates and seek marketing approvals for our Candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- scale up our manufacturing processes and arrange manufacturing for larger quantities of our Candidates for preclinical and clinical development and potential commercialization;
- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control and scientific personnel;
- build out new facilities or expand existing facilities to support our activities;
- acquire or in-license other drugs, drug candidates, technologies and intellectual property; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more Candidates with significant market potential. This will require us to be successful in a range of challenging activities, and our expenses will increase substantially as we continue to enroll patients in and conduct the INSPIRE DUCHENNE trial, initiate enrollment in our planned Phase 1b clinical trial of SGT-212, and continue to develop our pipeline and complete ongoing and planned preclinical studies and clinical trials of our Candidates, obtain marketing approval for our Candidates, develop adjacent technologies such as assays, develop and validate commercial-scale manufacturing processes, manufacture, market and sell

any future Candidates for which we may obtain marketing approval and satisfy any post-marketing requirements. Moreover, the manufacturing process requires materials which may fluctuate in cost or be limited or unavailable to us, as well as relationships with contract development and manufacturing organizations to facilitate the manufacturing process. We may never succeed in any of these activities and, even if we do, we may never generate revenue that is significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause stockholders to lose all or part of their investment.

We will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, conduct clinical trials of, and seek marketing approval for our Candidates. In addition, if we obtain marketing approval for our Candidates, we expect to incur significant expenses related to product sales, marketing, manufacturing and distribution. We also expect to continue to incur additional costs associated with operating as a public company. While we believe that our cash, cash equivalents and available-for-sale securities as of December 31, 2024, together with the net proceeds from our underwritten offering that closed on February 19, 2025, will be sufficient to fund our operating expenses and capital requirements into the first half of 2027, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate. In order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we anticipate that we will need additional funding to complete the development of our Candidates.

Our future capital requirements will depend on many factors, including:

- the progress, costs and results of the INSPIRE DUCHENNE trial, our planned Phase 1b clinical trial of SGT-212 and any future clinical trials of our Candidates;
- the costs, timing and outcome of regulatory review of our Candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for our Candidates;
- the costs associated with manufacturing and use of third-party manufacturers;
- the revenue, if any, received from commercial sale of our Candidates, should any of our future Candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining, defending and enforcing our intellectual property rights and defending intellectual property-related claims;
- the outcome of any lawsuits filed against us;
- the terms of our current and any future license agreements and collaborations;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to public health emergencies or pandemics and collateral consequences related thereto.

Identifying potential candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to submit a BLA or obtain marketing approval and achieve product sales. In addition, our Candidates, if approved, may not achieve commercial success. Our product revenue, if any, will be derived from or based on sales of our Candidates that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all, and may be impacted by the economic climate and market conditions. Our ability to raise additional funds may be adversely

impacted by general economic conditions, both inside and outside the U.S., including disruptions to, and instability and volatility in, the credit and financial markets in the U.S. and worldwide, heightened inflation, interest rate and currency rate fluctuations, and economic slowdown or recession as well as concerns related to public health emergencies or pandemics and geopolitical events, including civil or political unrest. In addition, market instability and volatility, high levels of inflation and interest rate fluctuations may increase our cost of financing or restrict our access to potential sources of future liquidity. Alternatively, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or Candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership of our common stock will be diluted and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or Candidates, or grant licenses on terms unfavorable to us.

We have never generated revenue from product sales and do not expect to do so for the foreseeable future, if ever.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize our Candidates. We do not anticipate generating revenue from product sales for the foreseeable future, if ever. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing research and development of our Candidates in a timely and successful manner;
- seeking and obtaining regulatory and marketing approvals for any of our Candidates for which we complete clinical trials;
- launching and commercializing Candidates for which we obtain regulatory and marketing approval by establishing a sales force and marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- maintaining and enhancing a commercially viable, sustainable, scalable, reproducible and transferable manufacturing processes for our Candidates that is compliant with cGMPs;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in terms of cost, amount and quality, products and services to support clinical development and the commercial demand for our Candidates, if approved;
- obtaining market acceptance, if and when approved, of our Candidates as a viable treatment option by patients, the medical community and third-party payors;
- qualifying for coverage and adequate reimbursement by government and third-party payors for our Candidates both in the U.S. and internationally;
- effectively addressing any competing technological and market developments;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations under such arrangements;
- maintaining, protecting, enforcing and expanding our portfolio of intellectual property rights, including patents, trademarks, trade secrets and know-how;
- avoiding and defending against intellectual property infringement, misappropriation and other claims;
- implementing additional internal systems and infrastructure, as needed; and
- attracting, hiring and retaining qualified personnel.

Our limited operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring rights to our technology, conducting research and development activities, establishing research and development collaborations, identifying and acquiring Candidates, establishing manufacturing arrangements and undertaking preclinical studies and clinical trials. As a company, we have limited experience in clinical development. We have not yet demonstrated the ability to complete clinical trials of any Candidate, obtain marketing approvals, manufacture at commercial-scale or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions our stockholders make about our prospects may not be as accurate as they could be if we had a longer operating history or prior experience integrating acquired businesses into our existing business.

Unfavorable global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn, including the impact of increased interest rates and inflation (such as the recent rise in inflation in the United States), could result in a variety of risks to our business, including weakened demand for our Candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

We hold a portion of our cash and cash equivalents that we use to meet our working capital and operating expense needs in deposit accounts that could be adversely affected if the financial institutions holding such funds fail.

We hold a portion of our cash and cash equivalents that we use to meet our working capital and operating expense needs in deposit accounts at multiple financial institutions. The balance held in these accounts may exceed the Federal Deposit Insurance Corporation, or FDIC, standard deposit insurance limit of $250,000. If a financial institution in which we hold such funds fails or is subject to significant adverse conditions in the financial or credit markets, we could be subject to a risk of loss of all or a portion of such uninsured funds or be subject to a delay in accessing all or a portion of such uninsured funds. Any such loss or lack of access to these funds could adversely impact our short-term liquidity and ability to meet our operating expense obligations, including payroll obligations.

For example, on March 10, 2023, Silicon Valley Bank, or SVB, and Signature Bank, were closed by state regulators and the FDIC was appointed receiver for each bank. The FDIC created successor bridge banks and all deposits of SVB and Signature Bank were transferred to the bridge banks under a systemic risk exception approved by the United States Department of the Treasury, the Federal Reserve and the FDIC. If financial institutions in which we hold funds for working capital and operating expenses were to fail, we cannot provide any assurances that such governmental agencies would take action to protect our uninsured deposits or investments in a similar manner.

We also maintain investment accounts with other financial institutions in which we hold our investments and marketable securities and, if access to the funds we use for working capital and operating expenses is impaired, we may not be able to sell investments or transfer funds from our investment accounts to other operating accounts on a timely basis sufficient to meet our operating expense obligations.

Risks Related to the Development of our Candidates

Our gene transfer Candidates utilize novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union.

Our future success depends on our successful development of our Candidates. Our risk of failure is high. We have experienced problems and delays in developing SGT-001, which we are no longer developing, and may in the future experience problems or delays in developing Candidates. Any such problems or delays would cause unanticipated costs, and any development problems may not be solved. For example, we or another party may uncover a previously unknown risk associated with our Candidates, the adeno-associated virus, or AAV, capsid, construct or other issues resulting in toxicity or lack of efficacy that may be more problematic than we currently believe and this may prolong the period of observation required for obtaining, or result in the failure to obtain, regulatory approval or may necessitate additional clinical testing.

In addition, our ability to conduct and complete our preclinical development testing and studies is contingent on our ability to source animals and other supplies required for the conduct of such testing and studies and the performance of animal models.

If we are unable to obtain all necessary animals and other supplies required for the conduct of our preclinical testing and studies, or the animal models do not perform as expected, we may be unable to complete such preclinical development testing and studies in a timely manner or at all. For example, some of our IND-enabling toxicology and other studies require certain non-human primates, or NHPs, that may be imported from countries in which trade relation with the U.S. are or may become challenging or through vendors who may not be able to timely source certain NHPs or at all, which may impair our ability to complete preclinical development testing and studies to support IND or similar applications or delay submission of such applications. Additionally, we may fail to demonstrate adequate Candidate efficacy and/or safety as required by regulatory authorities. We may fail to access relevant, adequate, or necessary animal models, including genetic models of disease and non-human primates in particular, for use in such studies as requested by regulatory authorities. We may also experience substantial delays as a result of our reliance on CROs to conduct all animal model experimentation necessary to assess the efficacy and safety of our Candidates. Any of these factors may result in delays to Candidate progression, inability to obtain regulatory approval, and/or substantial increases in Candidate development costs.

In addition, the product specifications and the clinical trial requirements of the FDA, the European Commission, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidate. The regulatory approval process for novel product candidates such as ours is unclear and can be more expensive and take longer than for other, better known or more extensively studied product candidates. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene transfer Candidates in either the United States or the European Union, if at all. Approvals by the European Commission may not be indicative of what the FDA may require for approval and vice versa.

Our gene transfer Candidates may cause undesirable side effects or have other properties that could delay or prevent their clinical development, regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

In the second quarter of 2024, patient dosing commenced in our INSPIRE DUCHENNE trial. Our other current Candidates have not yet been studied in human patients. During the conduct of clinical trials, patients may experience changes in their health, including illnesses, injuries, discomforts or a fatal outcome. Often, it is not possible to determine whether the Candidate being studied caused these conditions. For instance, we reported a serious adverse event in IGNITE DMD, which resulted in a clinical hold in November 2019, which has since been resolved. In April 2021, a patient treated with SGT-001 in IGNITE DMD experienced a systemic inflammatory response classified as a serious adverse event and considered by the investigator to be drug related.

In addition, it is possible that as we test Candidates in larger, longer and more extensive clinical programs, or as use of these Candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier clinical trials, as well as conditions that did not occur or went undetected in previous clinical trials, will be reported by subjects. Many times, side effects are only detectable after investigational products are tested in large-scale, Phase 3 clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. If additional clinical experience indicates that a Candidate has side effects or causes serious or life-threatening side effects, the development of the Candidate may fail or be delayed, or, if the Candidate has received regulatory approval, such approval may be withdrawn.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other clinical trials. The FDA convened the Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 to discuss toxicity risks of AAV based gene therapy products and discussed risks including oncogenicity risks due to capsid genome integration, hepatotoxicity, thrombotic microangiopathy, and neurotoxicity (especially related to dorsal root ganglion toxicity). While new recombinant capsids have been developed with the intent to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. There have been reports of significant adverse side effects, including muscle weakness and myocarditis, in clinical trials of other gene therapy treatments for Duchenne that may be related to the type and location of the specific gene mutation causing the disease. One clinical trial sponsor reported a death, preceded by hypovolemia and cardiogenic shock, of a non-ambulatory Duchenne subject with advanced disease and cardiac dysfunction. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients. Additionally, in previous clinical trials involving AAV capsids for gene therapy, some subjects experienced the development of a positive ELISPOT test associated with T-cell responses, which is of unclear clinical translatability. If T-cells are activated, the cellular immune

response system may trigger the removal of transduced cells. If our gene transfer Candidates demonstrate a similar effect or other undesirable side effects, we may decide or be required to halt or delay further clinical development of our Candidates involving AAV capsids for gene therapy.

Adverse side effects may be observed following administration of any AAV gene therapy, including SGT-003 or other Candidates. Not all contemplated AAV delivery systems have been validated in human clinical trials previously, such as AAV-SLB101, which is a novel capsid. If a delivery system does not meet the safety criteria or cannot provide the desired efficacy results, then we may be forced to suspend or terminate our development of SGT-003 or other Candidates. If certain adverse side effects were to occur in the future and we are unable to demonstrate that they were not caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, SGT-003 or other Candidates for any or all targeted indications. Even if we are able to demonstrate that any serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the clinical trial. Patients will also create antibodies to the AAV capsid and a second administration of gene transfer might not be safe or successful.

Additionally, if one or more of our Candidates receive marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy, or REMS, to ensure that the benefits outweigh the risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our Candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such a Candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a Candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

One of our prior clinical trials had been placed on clinical hold by the FDA in the past, and we cannot guarantee that similar events will not happen in ongoing, planned and future clinical trials for our Candidates.

In November 2019, the FDA placed a clinical hold on SGT-001 following a serious adverse event in IGNITE DMD. The third patient in the 2E14 vg/kg cohort of IGNITE DMD, dosed in late October 2019, experienced a serious adverse event deemed related to the study drug that was characterized by complement activation, thrombocytopenia, decrease in red blood cell count, acute kidney injury, and cardio-pulmonary insufficiency. In April 2021, an eighth patient was treated with SGT-001. The patient experienced a systemic inflammatory response which has since fully resolved. The event was classified as a serious adverse event and considered by the investigator to be drug related. While SGT-003 utilizes a different capsid than SGT-001 and includes other changes to the construct and manufacturing process to help avoid or mitigate any such events, we cannot guarantee that similar serious adverse events or clinical holds will not happen in ongoing and future clinical trials of SGT-003. We also cannot guarantee that similar serious adverse events or clinical holds will not happen in planned and future clinical trials of any of our other Candidates.

Delays in the completion of, or our inability to conduct, any clinical trial of SGT-003 or any other Candidate, as a result of similar serious adverse events or clinical holds or otherwise, will increase our costs, slow down or cease our Candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of SGT-003 or other Candidates.

We have never completed a clinical trial and may be unable to do so for any Candidate, including SGT-003 and other Candidates.

We are early in our development efforts and we have never completed a clinical trial. In the second quarter of 2024, patient dosing commenced in our INSPIRE DUCHENNE trial. We anticipate initiating a Phase 1b clinical trial of SGT-212 in adults with FA in the second half of 2025. Our other current Candidates are still in preclinical development. Preclinical studies involve a lengthy and expensive process with an uncertain outcome. There are many potential preclinical models to test for different disease states, and we could fail to choose the best or a predictive preclinical model to determine proof of concept and potential safety and efficacy of our Candidates. We may decide to suspend further testing on our Candidates or technologies if, in the judgment of our management and advisors, the preclinical test results do not support further development.

We will need to successfully initiate our planned clinical trials and complete our ongoing clinical trials in order to obtain FDA approval to market SGT-003, SGT-212 and other Candidates. We have limited experience in preparing, submitting and prosecuting regulatory submissions, and have not previously submitted a BLA for any Candidate. We cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin or to begin as proposed, or that, once begun, issues will not arise that suspend or terminate such clinical trials. Carrying out later-stage clinical trials and the submission of a successful BLA is a complicated process. This may be particularly true for design of a pivotal trial for the treatment of Duchenne as the FDA has not given clear guidance as to the necessary endpoints for approval of a treatment for Duchenne. In addition, we cannot be certain how many clinical trials of SGT-003, SGT-212 or other Candidates will be required or how such trials should be designed. Consequently, we may be unable to successfully and efficiently execute and complete necessary clinical trials in a way that leads to BLA submission and approval of SGT-003, SGT-212 or other Candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of Candidates that we develop. Failure to commence or complete, or delays in, clinical trials, could prevent us from or delay us in commercializing SGT-003, SGT-212 and other Candidates.

Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.

Results from preclinical studies or early clinical trials are not necessarily predictive of results from future clinical trials and are not necessarily indicative of final results. Our preclinical studies for certain Candidates in animals have been limited. There is a high failure rate for gene therapy and biologic products proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical studies and clinical trials are subject to varying interpretations, which may delay, limit or prevent regulatory approval. We also may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our Candidate development. Our Candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies. This failure could cause us to abandon any of our Candidates.

Preliminary or interim data that we announce or publish from time to time may change as more data becomes available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may announce or publish preliminary or interim data from clinical trials. Positive preliminary or interim data may not be predictive of such trial's subsequent or overall results. Preliminary or interim data are subject to the risk that one or more of the outcomes may materially change as more data becomes available. Additionally, preliminary or interim data are subject to the risk that one or more of the biologic or clinical outcomes may materially change as patient enrollment continues and more patient data becomes available. Therefore, positive preliminary or interim data in any ongoing clinical trial, including the INSPIRE DUCHENNE trial, may not be predictive of such results in the completed trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully evaluate all data. As a result, preliminary or interim data that we report may differ from future results from the clinical trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Preliminary or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary or interim data we previously published. As a result, preliminary or interim data should be viewed with caution until the final data are available. Material adverse changes in the final data compared to preliminary or interim data could significantly harm our business prospects.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our Candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the Candidate for its intended indications. Clinical testing is expensive, time consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in obtaining animals in sufficient quantities to run our preclinical studies;
- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement with the appropriate external parties on dose escalation;
- delays in enrolling patients in clinical trials;

- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board or independent ethics committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials, including because such trials have restrictive eligibility criteria or may be placebo-controlled trials and patients are not guaranteed to receive treatment with our Candidates, or as a result of alternative therapies or competing trials;
- difficulty in finding suitable animal models to demonstrate a disease specific phenotype;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with FDA good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union and other countries;
- delays in the testing, validation, manufacturing and delivery of our Candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in subjects completing participation in a trial or returning for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event, or after an inspection of our clinical trial operations, trial sites or manufacturing facilities or otherwise;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- delays as a result of public health emergencies or pandemics or other global instability; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Additionally, if the results of any clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our Candidates, we may:

- interrupt or halt clinical development;
- be delayed or fail in obtaining marketing approval for our Candidates;
- obtain approval for indications or patient populations that are not as broad as we intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way our products, if approved, are administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified REMS;
- be sued and held liable for harm caused to patients; or
- experience damage to our reputation.

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

Our product development costs will increase if we experience delays in testing or marketing approvals. In addition, if we make manufacturing or other changes to our Candidates, we may need to conduct additional studies to bridge our modified Candidates to earlier versions. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, which we have done in the past and which could result in delays. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to

commercialize our Candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our Candidates.

If our third-party clinical trial vendors fail to comply with strict regulations, any clinical trials for our Candidates may be delayed or unsuccessful.

We do not have the personnel capacity to conduct or manage the clinical trials that will be necessary for the development of our Candidates. For our INSPIRE DUCHENNE trial we are relying, and for any planned and future clinical trials we expect we will rely on third parties to assist us in managing, monitoring and conducting our clinical trials. If these third parties fail to comply with applicable regulations or do not adequately fulfill their obligations under the terms of our agreements with them, we may not be able to enter into alternative arrangements without undue delay or additional expenditures and, therefore, clinical trials for SGT-003 or other Candidates may be delayed or unsuccessful.

Furthermore, the FDA can be expected to inspect some or all of the clinical sites participating in our clinical trials to determine if our clinical trials are being conducted according to GCPs. If the FDA determines that these clinical sites are not in compliance with applicable regulations, we may be required to delay, repeat or terminate the clinical trials.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of SGT-003, SGT-212 or our other Candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-003, SGT-212 and our other Candidates are critical to our success. Because of our primary focus on rare diseases, we may have difficulty enrolling a sufficient number of eligible patients. The timing of any clinical trials depends on our ability to recruit patients to participate as well as complete required follow-up periods. If patients are unwilling or unable to participate in our gene therapy clinical trials, including because of negative publicity from adverse events related to our Candidates, other approved therapies, or due to competitive clinical trials or approvals for similar patient populations, clinical trials in products employing our capsid or our platform or for other reasons, the timeline for recruiting patients, conducting clinical trials and obtaining regulatory approval of SGT-003, SGT-212 or other Candidates may be delayed. We may also experience delays if patients withdraw from the clinical trial or do not complete the required monitoring period. Furthermore, we may face difficulties in recruiting patients to enroll in, or once enrolled, retaining patients in future clinical trials if they or their caretakers are affected by public health emergencies or pandemics or are fearful of traveling to, or are unable to travel to, our clinical trial sites because of public health emergencies or pandemics or other unforeseen events. These delays could result in increased costs, delays in advancing SGT-003 or other Candidates, delays in testing the effectiveness of our Candidates or termination of clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete any clinical trials in a timely manner. Patient enrollment and trial completion is affected by many factors, including:

- size of the patient population and the process for identifying subjects;
- design of the trial protocol;
- eligibility and exclusion criteria, including age, size and functional ability and pre-existing antibodies to AAV capsids that preclude subjects from being able to receive AAV-mediated gene transfer;
- restrictions on our ability to conduct clinical trials, including full and partial clinical holds on ongoing or planned clinical trials;
- perceived risks and benefits of the Candidate under study;
- perceived risks and benefits of gene therapy-based approaches to the treatment of diseases;
- release or disclosure of data from our completed or ongoing clinical trials;
- availability of competing therapies and clinical trials;
- severity of the disease;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians;

- ability to monitor subjects adequately during and after treatment; and
- in the case of pivotal trials, the risk that patients may opt not to enroll because they are not assured treatment with our Candidate.

If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted. For example, in December 2022, with the passage of FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. In June 2024, as mandated by FDORA, the FDA issued draft guidance outlining the general requirements for DAPs. Unlike most guidance documents issued by the FDA, the DAP guidance when finalized will have the force of law because FDORA specifically dictates that the form and manner for submission of DAPs are specified in FDA guidance. In January 2025, in response to an executive order issued by President Trump on Diversity, Equity and Inclusion programs, the FDA removed this draft guidance from its website. The implications of this action are not yet known.

We plan to conduct clinical trials at sites outside the United States. The FDA may not accept data from trials conducted in such locations, and the conduct of trials outside the United States could subject us to additional delays and expense.

We plan to conduct clinical trials, including the INSPIRE DUCHENNE trial, with one or more trial sites that are located outside the United States. Although the FDA may accept data from clinical trials conducted at sites outside the United States, acceptance of these data is subject to conditions imposed by the FDA. For example, where data from foreign clinical trial sites are not intended to serve as the sole basis for approval in the United States, the FDA will not accept the data as support for a marketing application unless the clinical trial was well designed and conducted in accordance with GCP requirements. The FDA must also be able to validate the data from the trial through an onsite inspection, if necessary. Where data from foreign clinical trial sites are intended to serve as the sole basis for marketing approval in the United States, the FDA will generally not approve the application on the basis of foreign data alone unless (i) the data are applicable to the U.S. population and U.S. medical practice; (ii) the trials were performed by clinical investigators of recognized competence and pursuant to GCP regulations; and (iii) the data may be considered valid without the need for an on-site inspection by the FDA, or if the FDA considers such inspection to be necessary, the FDA is able to validate the data through an on-site inspection or other appropriate means. In addition, these clinical trials are subject to the applicable local laws of the jurisdictions where the trials are conducted. There can be no assurance that the FDA will accept data from trials conducted outside of the United States. If the FDA does not accept the data from any trial that we conduct outside the United States, it would likely result in the need for additional trials, which would be costly and time-consuming and could delay or permanently halt our development of the applicable product candidates.

In addition, conducting clinical trials outside the United States could have a significant adverse impact on us. Risks inherent in conducting international clinical trials include:

- clinical practice patterns and standards of care that vary widely among countries;
- non-U.S. regulatory authority requirements that could restrict or limit our ability to conduct our clinical trials;
- compliance with foreign manufacturing, customs, shipment and storage requirements;
- administrative burdens of conducting clinical trials under multiple non-U.S. regulatory authority schema;
- foreign exchange fluctuations;
- diminished protection of intellectual property in some countries; and
- interruptions or delays resulting from geopolitical events, such as wars.

The EU Clinical Trials Regulation, or CTR, which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. The CTR aims to simplify and streamline the authorization, conduct and transparency of clinical trials in the EU. We have not previously secured authorization to conduct clinical trials in the European Union pursuant to the CTR and, accordingly, there is a risk that we may be delayed in commencing such studies. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted.

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize our Candidates and the approval may be for narrower indication than we seek.

We cannot commercialize our Candidates until the appropriate regulatory authorities have reviewed and approved the Candidate. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state and local statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals. Even if our Candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA advisory committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action or changes in regulatory authority policy during the period of product development, clinical trials and the regulatory review process.

Further, under the Pediatric Research Equity Act of 2003, a BLA or supplement to a BLA for certain biological products must contain data to assess the safety and effectiveness of the biological product in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective, unless the sponsor receives a deferral or waiver from the FDA. A deferral may be granted for several reasons, including a finding that the product or therapeutic Candidate is ready for approval for use in adults before pediatric trials are complete or that additional safety or effectiveness data needs to be collected before the pediatric trials begin. The applicable legislation in the European Union also requires sponsors to either conduct clinical trials in a pediatric population in accordance with a Pediatric Investigation Plan approved by the Pediatric Committee of the EMA or to obtain a waiver or deferral from the conduct of these studies by this Committee. For any of our Candidates for which we are seeking regulatory approval in the U.S. or the European Union, we cannot guarantee that we will be able to obtain a waiver or alternatively complete any required studies and other requirements in a timely manner, or at all, which could result in associated reputational harm and subject us to enforcement action, invalidation of the marketing application, and/or financial penalties. Our collaborators are also subject to similar requirements outside of the United States and the European Union and thus the attendant risks and uncertainties.

Even if we receive regulatory approval, regulatory authorities may approve a Candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or a REMS. Regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our Candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our Candidates.

Further, there is substantial uncertainty as to how measures being implemented by the new Trump Administration across the government will impact the FDA, CMS and other federal agencies with jurisdiction over our activities. For example, since taking office, President Trump has issued a number of executive orders, which could have a significant impact on the manner in which the FDA conducts its operations and engages in regulatory and oversight activities. If these or other orders or executive actions impose constraints on FDA's ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted. In addition, the loss of FDA personnel could lead to further disruptions and delays in FDA review and oversight of our product candidates. Similarly, efforts by the new administration to substantially reduce or delay research funding by the National Institutes of Health of medical research could have substantial direct or indirect impacts on our research activities.

Even if we obtain regulatory approval for a Candidate, our Candidates will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for any of our Candidates, we will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our Candidates may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or conditions of approval, or requirements for potentially costly post-marketing testing and surveillance to monitor the safety, purity, and potency of the biologic product. Advertising and promotional materials must comply with FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, later discovery of previously unknown adverse events or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have various consequences, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings on the labeling or marketing of a product;

- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions or the imposition of civil or criminal penalties; or
- litigation involving patients using our products.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs applicable to drug manufacturers or quality assurance standards applicable to medical device manufacturers, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, any contract manufacturers we may engage in the future, our future collaborators and their contract manufacturers will also be subject to other regulatory requirements, including submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements regarding the distribution of samples to clinicians, recordkeeping, and costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product such as the requirement to implement a REMS.

Finally, our ability to develop and market new drug products may be impacted by litigation challenging the FDA's approval of another company's drug product. In April 2023, the U.S. District Court for the Northern District of Texas invalidated the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various measures adopted under a REMS. The Court of Appeals for the Fifth Circuit declined to order the removal of mifepristone from the market but did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone, which the FDA authorized in 2016 and 2021, were arbitrary and capricious. In June 2024, the Supreme Court reversed that decision after unanimously finding that the plaintiffs did not have standing to bring this legal action against the FDA. On October 11, 2024, the Attorneys General of three states filed an amended complaint in the U.S. District Court for the Northern District of Texas challenging the FDA's actions. Depending on the outcome of this litigation our ability to develop new drug Candidates and to maintain approval of existing drug products could be delayed, undermined or subject to protracted litigation.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions. Further, similar restrictions apply to approved products in the EU. The holder of a marketing authorization is required to comply with a range of requirements applicable to the manufacturing, marketing, promotion and sale of medicinal products. These include: compliance with the EU's stringent pharmacovigilance or safety reporting rules, which can impose post-authorization studies and additional monitoring obligations; the manufacturing of authorized medicinal products, for which a separate manufacturer's license is mandatory; and the marketing and promotion of authorized drugs, which are strictly regulated in the EU and are also subject to EU Member State laws.

Accordingly, assuming we, or our collaborators, receive marketing approval for one or more of our Candidates, we, and our collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control. If we, and our collaborators, are not able to comply with post-approval regulatory requirements, our or our collaborators' ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the

cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any regulatory approval to market our products will be limited by indication. If we fail to comply or are found to be in violation of FDA regulations restricting the promotion of our products for unapproved uses, we could be subject to criminal penalties, substantial fines or other sanctions and damage awards.

The regulations relating to the promotion of products for unapproved uses are complex and subject to substantial interpretation by the FDA, EMA, Medicines and Healthcare products Regulatory Agency, or MHRA, and other government agencies. In September 2021, the FDA published final regulations which describe the types of evidence that the agency will consider in determining the intended use of a drug product. Physicians may nevertheless prescribe our products off-label to their patients in a manner that is inconsistent with the approved label. We intend to implement compliance and training programs designed to ensure that our sales and marketing practices comply with applicable regulations. Notwithstanding these programs, the FDA or other government agencies may allege or find that our practices constitute prohibited promotion of our products for unapproved uses. We also cannot be sure that our employees will comply with company policies and applicable regulations regarding the promotion of products for unapproved uses.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading, and non-promotional scientific communications concerning their products in certain circumstances. For example, in January 2025, the FDA published final guidance outlining its policies governing the distribution of scientific information to healthcare providers about unapproved uses of approved products. The final guidance calls for such communications to be truthful, non-misleading and scientifically sound and to include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use of the approved product. If a company engages in such communications consistent with the guidance's recommendations, the FDA indicated that it will not treat such communications as evidence of unlawful promotion of a new intended use for the approved product.

In addition, we could be adversely affected by several significant administrative law cases decided by the U.S. Supreme Court in 2024. In *Loper Bright Enterprises v. Raimondo*, for example, the court overruled *Chevron U.S.A., Inc. v. Natural Resources Defense Council, Inc*., which for 40 years required federal courts to defer to permissible agency interpretations of statutes that are silent or ambiguous on a particular topic. The Supreme Court stripped federal agencies of this presumptive deference and held that courts must exercise their independent judgment when deciding whether an agency, such as the FDA, acted within its statutory authority under the Administrative Procedure Act, or the APA. Additionally, in *Corner Post, Inc. v. Board of Governors of the Federal Reserve System*, the court held that actions to challenge a federal regulation under the APA can be initiated within six years of the date of injury to the plaintiff, rather than the date the rule is finalized. The decision appears to give prospective plaintiffs a personal statute of limitations to challenge longstanding agency regulations. Another decision, *Securities and Exchange Commission v. Jarkesy*, overturned regulatory agencies' ability to impose civil penalties in administrative proceedings. These decisions could introduce additional uncertainty into the regulatory process and may result in additional legal challenges to actions taken by federal regulatory agencies, including the FDA and CMS, that we rely on. In addition to potential changes to regulations as a result of legal challenges, these decisions may result in increased regulatory uncertainty and delays and other impacts, any of which could adversely impact our business and operations.

In recent years, a significant number of pharmaceutical and biotechnology companies have been the target of inquiries and investigations by various federal and state regulatory, investigative, prosecutorial and administrative entities in connection with the promotion of products for unapproved uses and other sales practices, including the Department of Justice and various U.S. Attorneys' Offices, the Office of Inspector General of the Department of Health and Human Services, or HHS, the FDA, the Federal Trade Commission, or the FTC, and various state Attorneys General offices. These investigations have alleged violations of various federal and state laws and regulations, including claims asserting antitrust violations, violations of the Federal Food, Drug, and Cosmetic Act, the False Claims Act, the Prescription Drug Marketing Act and anti-kickback laws and other alleged violations in connection with the promotion of products for unapproved uses, pricing and Medicare and/or Medicaid reimbursement. Many of these investigations originate as "*qui tam*" actions under the False Claims Act. Under the False Claims Act, any individual can bring a claim on behalf of the government alleging that a person or entity has presented a false claim or caused a false claim to be submitted to the government for payment. The person bringing a *qui tam* suit is entitled to a share of any recovery or settlement. *Qui tam* suits, also commonly referred to as "whistleblower suits," are often brought by current or former employees. In a *qui tam* suit, the government must decide whether to intervene and prosecute the case. If it declines, the individual may pursue the case alone.

If the FDA or any other governmental agency initiates an enforcement action against us or if we are the subject of a *qui tam* suit and it is determined that we violated prohibitions relating to the promotion of products for unapproved uses, we could be subject to substantial civil or criminal fines or damage awards and other sanctions such as consent decrees and corporate integrity agreements pursuant to which our activities would be subject to ongoing scrutiny and monitoring to ensure compliance with applicable laws and regulations. Any such fines, awards or other sanctions would have an adverse effect on our revenue, business, financial prospects and reputation.

Even if we obtain and maintain approval of one or more of our Candidates from the FDA, we may never obtain approval for our Candidates outside of the United States, which would limit our market opportunities and adversely affect our business.

Even if we receive FDA approval of one or more of our Candidates in the United States, approval of a Candidate in the United States by the FDA does not ensure approval of such Candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Future sales of our Candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approval. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. If we submit a marketing authorization application, or MAA, to the EMA for approval of SGT-003, SGT-212 or other Candidates in the European Union, obtaining such approval from the European Commission following the opinion of the EMA is a lengthy and expensive process. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our Candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for our Candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced, and our ability to realize the full market potential of our Candidates will be harmed.

Additionally, we could face heightened risks with respect to obtaining marketing authorization in the United Kingdom as a result of the withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. The United Kingdom is no longer part of the European Single Market and EU Customs Union. As of January 1, 2025 the MHRA is responsible for approving all medicinal products destined for the UK market (i.e., Great Britain and Northern Ireland), and the EMA will no longer have any role in approving medicinal products destined for Northern Ireland. Any delay in obtaining, or an inability to obtain, any marketing authorizations, as a result of Brexit or otherwise, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom for our Candidates, which could significantly and materially harm our business.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted. For instance, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products (including potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023 with several amendments requested in April 2024. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may, however, have a significant impact on the pharmaceutical industry and our business in the long term.

We expect that we will be subject to additional risks in commercializing any of our Candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

Regulatory requirements governing gene therapy products are periodically updated and may continue to change in the future.

Regulatory requirements governing gene therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of gene therapy products. For example, in the United States, the FDA has established the Office of Therapeutic Products within the Center for Biologics Evaluation and Research, or the CBER, to consolidate the review of gene therapy and related products,

and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials may also be subject to review and oversight by an institutional biosafety committee, a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

The FDA has issued various guidance documents regarding gene therapies, including final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, gene therapies for rare diseases and gene therapies for retinal disorders, a final guidance in October 2022 for Human Gene Therapy for Neurodegenerative Diseases, as well as a draft guidance in July 2023 on comparability requirements for manufacturing changes in gene therapy products. In December 2023, a draft guidance on potency assurance for cellular and gene therapy products was released. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy Candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, for AAV capsids specifically, the FDA typically recommends that sponsors continue to monitor participants for potential gene therapy-related adverse events for up to a 5-year period. Other types of gene therapy or gene editing products may require longer follow up, potentially up to a maximum 15-year period.

Similarly, the EMA may issue new guidelines concerning the development and marketing authorization for gene therapy products and require that we comply with these new guidelines. The grant of marketing authorization in the European Union for gene therapy products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC includes specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

Finally, ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that our Candidates are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our Candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

As we advance our Candidates through clinical development, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of Candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue.

We may not be able to obtain orphan drug exclusivity for one or more of our Candidates, and even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Under the Orphan Drug Act, the FDA may designate a product candidate as an orphan drug if it is a drug or biologic intended to treat a rare disease or condition. A similar regulatory scheme governs approval of orphan products by the EMA in the European Union. Generally, if a product candidate with an orphan drug designation subsequently receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the EMA from approving another marketing application for a similar product for the same therapeutic indication for that time period. The applicable period is seven years in the United States and ten years in the European Union. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation, in particular if the product is sufficiently profitable so that market exclusivity is no longer justified.

The FDA has granted orphan drug designation to SGT-003 for the treatment of Duchenne and the FDA and EMA have granted orphan drug designation to SGT-501 for the treatment of CPVT.

In order for the FDA to grant orphan drug exclusivity to one of our products, the FDA must find that the product is indicated for the treatment of a condition or disease with a patient population of fewer than 200,000 individuals annually in the United States. The FDA may conclude that the condition or disease for which orphan drug exclusivity is sought does not meet this standard. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different products can be approved for the same condition.

In addition, under the FDA September 2021 guidance for interpreting sameness of gene therapy products under the orphan drug regulations, even after an orphan drug is approved, the FDA can subsequently approve a similar product for the same condition if the FDA concludes that the later product is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. Orphan drug exclusivity may also be lost if the FDA or EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of the patients with the rare disease or condition.

The FDA Reauthorization Act of 2017, or FDARA, requires that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. FDARA reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority.

The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term "same disease or condition" means the designated "rare disease or condition" and could not be interpreted by the FDA to mean the "indication or use." Thus, the Court of Appeals concluded that orphan drug exclusivity applies to the entire designated disease or condition rather than the "indication or use." Although there have been legislative proposals to overrule this decision, they have not been enacted into law. In January 2023, the FDA announced that, in matters beyond the scope of that court order, FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. We do not know if, when, or how the FDA or Congress may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA or Congress may make to its orphan drug regulations and policies, our business could be adversely impacted.

We may seek a breakthrough therapy designation for one or more of our Candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

We may seek a breakthrough therapy designation for one or more of our Candidates; however, we cannot assure our stockholders that one or more of our Candidates will meet the criteria for that designation. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies and biologics that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review if supported by clinical data at the time the BLA is submitted to the FDA.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe that one of our Candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. Even if we receive breakthrough therapy designation, the receipt of such designation for a Candidate may not result in a faster development or regulatory review or approval process compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our Candidates qualifies as a breakthrough therapy, the FDA may later decide that the Candidate no longer meets the conditions for qualification or decide that the time period for FDA review or approval will not be shortened.

Accelerated approval by the FDA, even if granted for one or more of our Candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our Candidates will receive marketing approval.

We may seek approval of one or more of our Candidates using the FDA's accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful

advantage over available therapies. In addition, it must demonstrate an effect on a surrogate or intermediate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA or other applicable regulatory agency makes the determination regarding whether a surrogate or intermediate endpoint is reasonably likely to predict long-term clinical benefit. Given that expression of microdystrophin has not yet been established to predict long-term clinical benefit, it is not currently accepted, and it is possible the FDA and/or other applicable regulatory agencies could decide never to accept it, as a surrogate endpoint for the accelerated approval pathway for the treatment of Duchenne.

As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. These confirmatory trials must be completed with due diligence and may be required to be initiated prior to submission of the BLA. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Further, with passage of FDORA in December 2022, Congress modified certain provisions governing accelerated approval of drug and biologic products. Specifically, the new legislation authorized the FDA to: require a sponsor to have its confirmatory clinical trial underway before accelerated approval is awarded, require a sponsor of a product granted accelerated approval to submit progress reports on its post-approval studies to FDA every six months (until the study is completed) and use expedited procedures to withdraw accelerated approval of a BLA after the confirmatory trial fails to verify the product's clinical benefit.

There can be no assurance that the FDA or comparable foreign regulatory agencies will agree with our surrogate endpoints or intermediate clinical endpoints in any of our clinical trials, or that we will decide to pursue or submit any additional application for accelerated approval or any other form of expedited development, review or approval. Similarly, there can be no assurance that, after feedback from the FDA or comparable foreign regulatory agencies, we will continue to pursue or apply for accelerated approval or any other form of expedited development, review or approval. Furthermore, for any submission of an application for accelerated approval or application under another expedited regulatory designation, there can be no assurance that such submission or application will be accepted for filing or that any expedited development, review or approval will be granted on a timely basis, or at all.

A failure to obtain accelerated approval or any other form of expedited development, review or approval for our Candidates, or withdrawal of a Candidate, would result in a longer time period until commercialization of such Candidate, could increase the cost of development of such Candidate and could harm our competitive position in the marketplace.

A potential regenerative medicine advanced therapy designation by the FDA for our Candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our Candidates will receive marketing approval.

We may seek a regenerative medicine advanced therapy designation for some of our Candidates. A regenerative medicine advanced therapy is defined as cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues may meet the definition of a regenerative medicine therapy. The regenerative medicine advanced therapy program is intended to facilitate efficient development and expedite review of regenerative medicine advanced therapies, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A BLA for a regenerative medicine advanced therapy may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with the FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A regenerative medicine therapy that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical trials, patient registries, or other sources of real world evidence, such as electronic health records; the collection of larger confirmatory data sets; or post-approval monitoring of all patients treated with such therapy prior to its approval.

Designation as a regenerative medicine advanced therapy is within the discretion of the FDA. Accordingly, even if we believe one of our Candidates meets the criteria for designation as a regenerative medicine advanced therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a regenerative medicine advanced therapy designation for a Candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our Candidates qualify as regenerative medicine advanced therapies, the FDA may later decide that the biological products no longer meet the conditions for qualification.

We may seek PRIME Designation in the EU for one or more of our Candidates, but we might not receive such designations and, even if we do, such designations may not lead to a faster development or regulatory review or approval process.

In the EU, we may seek PRIME designation for our Candidates in the future. PRIME is a voluntary program aimed at enhancing the EMA's role to reinforce scientific and regulatory support in order to optimize development and enable accelerated assessment of new medicines that are of major public health interest with the potential to address unmet medical needs. The program focuses on medicines that target conditions for which there exists no satisfactory method of treatment in the EU or even if such a method exists, it may offer a major therapeutic advantage over existing treatments. PRIME is limited to medicines under development and not authorized in the EU and the sponsor intends to apply for an initial marketing authorization application through the centralized procedure. To be accepted for PRIME, a product candidate must meet the eligibility criteria in respect of its major public health interest and therapeutic innovation based on information that is capable of substantiating the claims.

The benefits of a PRIME designation include the appointment of a Committee for Medicinal Products for Human Use rapporteur to provide continued support and help to build knowledge ahead of a marketing authorization application, early dialogue and scientific advice at key development milestones, and the potential to qualify products for accelerated review, meaning reduction in the review time for an opinion on approvability to be issued earlier in the application process. PRIME enables a sponsor to request parallel EMA scientific advice and health technology assessment advice to facilitate timely market access. Even if we receive PRIME designation for any of our Candidates, the designation may not result in a materially faster development process, review or approval compared to conventional EMA procedures. Further, obtaining PRIME designation does not assure or increase the likelihood of EMA's grant of a marketing authorization.

We may seek a Rare Pediatric Disease Designation for our Candidates. However, a BLA for such Candidates may not meet the eligibility criteria for a priority review voucher upon approval.

With enactment of the Food and Drug Administration Safety and Innovation Act in 2012, Congress authorized the FDA to award priority review vouchers to sponsors of certain rare pediatric disease product applications that meet the criteria specified in the law. This provision is designed to encourage development of new drug and biological products for prevention and treatment of certain rare pediatric diseases. Specifically, under this program, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. The sponsor of a rare pediatric disease drug product receiving a priority review voucher may transfer (including by sale) the voucher to another sponsor. The voucher may be further transferred any number of times before the voucher is used, as long as the sponsor making the transfer has not yet submitted the application.

In order to receive a priority review voucher upon BLA approval, the product must receive designation from the FDA as a product for a rare pediatric disease prior to approval of the marketing application. A "rare pediatric disease" is a disease that is serious or life-threatening, in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years and affects fewer than 200,000 people in the United States, or affects more than 200,000 people in the United States but there is no reasonable expectation that the cost of developing and making available in the United States a product for such disease or condition will be recovered from sales in the United States of such product. In addition to receiving rare pediatric disease designation, in order to receive a priority review voucher, the BLA must be given priority review, rely on clinical data derived from studies examining a pediatric population and dosages of the product intended for that population, not seek approval for a different adult indication in the original rare pediatric disease product application and be for a product that does not include a previously approved active ingredient.

Under the current statutory sunset provisions for the Rare Pediatric Disease Priority Review Voucher Program, after September 30, 2024, the FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the drug, and that designation was granted by September 30, 2024.

After September 30, 2026, the FDA may not award any rare pediatric disease priority review vouchers. If we do not obtain approval of a BLA by these dates, and if the Rare Pediatric Disease Priority Review Voucher Program is not further extended by congressional action, we may not receive a Priority Review Voucher.

The FDA has granted rare pediatric disease designation to SGT-003 for the treatment of Duchenne and SGT-501 for the treatment of CPVT.

We may seek a fast track designation for one or more of our Candidates. However, such designation may not actually lead to a faster development or regulatory review or approval process. We might not receive such designation for one or more of our Candidates.

If a therapy is intended for the treatment of a serious condition and non-clinical or clinical data demonstrates the potential to address unmet medical need for this condition, a drug sponsor may apply for FDA fast track designation. However, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. The FDA has broad discretion with respect to whether or not to grant fast track designation to a product candidate, so even if we believe a particular Candidate is eligible for such designation, the FDA may decide not to grant it. Moreover, we may not experience a faster development or regulatory review or approval process with fast track designation compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program or if the unmet need has been fulfilled with the approval of another product. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

The FDA has granted fast track designation to SGT-003 for the treatment of Duchenne and to SGT-212 for the treatment of FA.

We may seek priority review designation for one or more of our Candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our Candidates, however, we cannot assume that one or more of our Candidates will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular Candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

Inadequate funding for the FDA, the SEC and other U.S. or foreign government agencies, including from government shut downs, or other disruptions to these agencies' operations, could hinder their ability to hire and retain key leadership and other personnel, prevent new products and services from being developed or commercialized in a timely manner or otherwise prevent those agencies from performing normal business functions on which the operation of our business may rely, which could negatively impact our business.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including government budget and funding levels, ability to hire and retain key personnel and accept the payment of user fees, and statutory, regulatory and policy changes. Average review times at the agency have fluctuated in recent years as a result. Disruptions at the FDA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. In addition, government funding of the SEC and other government agencies on which our operations may rely, including those that fund research and development activities, is subject to the political process, which is inherently fluid and unpredictable.

Disruptions at the FDA, EMA and other agencies may also slow the time necessary for new product candidates to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, in recent years, including 2018 and 2019, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA and the SEC, have had to furlough critical employees and stop critical activities. In addition, disruptions may result in events similar to the COVID-19 pandemic. During the COVID-19 pandemic, a number of companies announced receipt of complete response letters due to the FDA's inability to complete required inspections for their applications. In the event of a similar public health emergency in the future, the FDA may not be able to continue its current pace and review timelines could be extended. Regulatory authorities outside the United States facing similar circumstances may adopt similar restrictions or other policy measures in response to a similar public health emergency and may also experience delays in their regulatory activities.

If a prolonged government shutdown or other disruption occurs, it could significantly impact the ability of the FDA, EMA or other regulatory agency to review and process our regulatory submissions in a timely manner, which could have a material adverse effect on our business. Further, future government shutdowns or other disruptions affect other government agencies such as the SEC, which may also impact our business by delaying review of our public filings, to the extent such review is necessary and could impact our ability to access the public markets and obtain necessary capital in order to properly capitalize and continue our operations.

We face significant competition and our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our ability to develop, successfully market or commercialize our Candidates. Changes within the competitive landscape could lead us to alter our regulatory and/or clinical trial strategy, baseline eligibility criteria or make other modifications to clinical trial designs.

We operate in a highly competitive segment of the biopharmaceutical market. We face competition from many different sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, government agencies and private and public research institutions. Our Candidates, if successfully developed and approved, will compete with established therapies as well as with new treatments that may be introduced by our competitors. There are a variety of product candidates, including gene therapies, in development for Duchenne, CPVT, other cardiomyopathies or FA. Many of our competitors have significantly greater financial, product candidate development, manufacturing and marketing resources than we do. Large pharmaceutical and biotechnology companies have extensive experience in clinical testing and obtaining regulatory approval for their products, and mergers and acquisitions within these industries may result in even more resources being concentrated among a smaller number of larger competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials, enrolling patients in clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

We are aware of a number of companies and research institutions developing gene transfer programs progressing in Duchenne. For example, in June 2023, Sarepta Therapeutics, Inc., or Sarepta, announced that it had received accelerated approval for its gene therapy candidate ELEVIDYS® for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne. In June 2024, Sarepta announced an expanded US approval of ELEVIDYS® for patients who are at least 4 years of age including full approval for ambulatory Duchenne patients and accelerated approval for non-ambulatory Duchenne patients. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including Genethon with a product candidate currently being evaluated in a Phase 1/2/3 clinical trial and REGENXBIO Inc. with a product candidate in Phase 1/2/3 clinical development.

We are also aware of a number of companies and research institutions developing gene transfer programs in FA. For example, Lexeo Therapeutics is developing an IV gene therapy to treat the cardiac manifestations of FA. Other competitors currently developing gene therapies to treat FA are in preclinical development, including Neurocrine Biosciences in collaboration with Voyager Therapeutics. We are also aware of other companies developing non-gene therapies for FA, such as Design Therapeutics, Laminar Therapeutics, and PTC Therapeutics who has a PDUFA target action date of August 19, 2025. Biogen's SKYCLARYS® (omaveloxolone) was approved for the treatment of FA in adults and adolescents aged 16 and older by the FDA and the European Commission in February 2023 and February 2024, respectively.

We are also aware of several companies and research institutions conducting clinical trials in small molecule product candidates focused on CPVT, including Armgo Pharmaceuticals, Inc. with an orally administered Rycal in a Phase 2 clinical trial and Cardurion Pharmaceuticals with an orally administered CAMKII-delta inhibitor candidate in a Phase 2 clinical trial.

Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are first to market or are safer, more effective, have fewer or less severe side effects, have broader market acceptance, are more convenient or are less expensive than any Candidate that we may develop. Changes within the competitive landscape could lead us to alter regulatory and/or clinical trial strategy, baseline eligibility criteria or make other modifications to clinical trial designs.

We are aware of several companies focused on developing gene therapies in various indications, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against Candidates we develop.

We may fail to capitalize on other potential Candidates that may represent a greater commercial opportunity or for which there is a greater likelihood of success.

The success of our business depends upon our ability to develop and commercialize our Candidates. Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or Candidates or for indications that later prove to have greater commercial potential than our Candidates. For example, in September 2022, we announced that we would be pausing activities for SGT-001, which we are now no longer developing.

Our spending on current and future research and development programs may not yield any commercially viable Candidates. If we do not accurately evaluate the commercial potential for a particular Candidate, we may relinquish valuable rights to that

Candidate through strategic collaborations, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such Candidate. Alternatively, we may allocate internal resources to a Candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. If any of these events occur, we may be forced to abandon our development efforts with respect to a particular Candidate or fail to develop a potentially successful Candidate.

Risks Related to the Manufacturing and Commercialization of our Candidates

We have entered into, and may in the future enter into, collaborations with third parties for the development or commercialization of our Candidates. If our collaborations are not successful, we may not be able to capitalize on the market potential of these Candidates and our business could be adversely affected.

While we have retained all necessary rights to and are developing on our own SGT-003, SGT-212 and other Candidates, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to SGT-003, SGT-212 or other Candidates. Our likely collaborators for any such sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our Candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

Collaborations that we enter into may not be successful, and any success will depend heavily on the efforts and activities of such collaborators. Collaborations pose a number of risks, including the following:

- collaborators have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development of our Candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may not pursue commercialization of any Candidates that achieve regulatory approval or may elect not to continue or renew commercialization programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that may divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a Candidate, repeat or conduct new clinical trials or require a new formulation of a Candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding Candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such Candidates on a discretionary basis;
- collaborators could develop products that compete directly or indirectly with our Candidates and products pursuant to the collaboration;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our Candidates and products if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- Candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our Candidates;
- a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a Candidate or product;
- a collaborator with marketing and distribution rights to one or more of our Candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;

- disagreements with collaborators, including disagreements over intellectual property or proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of Candidates, might lead to additional responsibilities for us with respect to Candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly obtain, maintain, enforce, defend or protect our intellectual property or proprietary rights or may use our proprietary information in such a way as to potentially lead to disputes or legal proceedings that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property or proprietary rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable Candidates.

Collaboration agreements may not lead to development or commercialization of Candidates in the most efficient manner, or at all. If any collaborations that we enter into do not result in the successful development and commercialization of products or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of our Candidates could be delayed and we may need additional resources to develop our Candidates. All of the risks relating to product development, regulatory approval and commercialization described herein also apply to the activities of our collaborators.

Additionally, subject to its contractual obligations to us, if a collaborator of ours is involved in a business combination, the collaborator might deemphasize or terminate the development or commercialization of any Candidate licensed to it by us. If one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and our perception in the business and financial communities could be adversely affected.

We may not be successful in finding strategic collaborators for continuing development of our Candidates or platform technologies, or for successfully commercializing or competing in the market for certain indications.

We may seek to establish strategic partnerships for developing Candidates or platform technologies due to capital costs required to develop, manufacture and commercialize our Candidates or platform technologies. We may not be successful in our efforts to establish strategic partnerships or other alternative arrangements because, among other things, our research and development pipeline may be insufficient, Candidates or platform technologies may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our Candidates or platform technologies as having the requisite potential to demonstrate safety and efficacy. We cannot be certain that, following a strategic transaction, we will achieve an economic or business benefit that justifies such transaction. If we seek to but are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail, reduce or delay the development of a Candidate, delay its potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development, manufacturing or commercialization activities independently. If we elect to fund our own independent development or commercialization activities, we will need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development, manufacturing and commercialization activities, we may not be able to further develop our Candidates or platform technologies.

We have limited gene therapy manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-003, SGT-212, SGT-501, or our other Candidates. In addition, changes to manufacturing sites or processes, or formulations for our Candidates may result in additional cost or delay.

We have limited experience manufacturing SGT-003, SGT-212, SGT-501 and our other Candidates. The manufacturing process we have used historically and the manufacturing process we plan to use in the future to produce product for our Candidates are complex and our processes have not been validated for commercial use. As Candidates progress through preclinical studies and clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to enhance safety, quality, efficacy, yield, manufacturing batch size, minimize costs and achieve consistent results. For example, we have moved to a transient transfection-based manufacturing process for SGT-003 and while we have observed early positive

results in preclinical studies and in our ongoing INSPIRE DUCHENNE trial using this new manufacturing process, any further changes in manufacturing or formulation may result in effects and results that are different from those observed in our completed studies to date. Similarly, in the future we may further alter our existing process or introduce an alternative process or formulation of one or more of our Candidates during the course of our planned preclinical studies or clinical trials. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our Candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay initiation or completion of clinical trials, require the conduct of bridging studies or clinical trials or the repetition of one or more studies or clinical trials, increase development costs, delay approval of our Candidates and jeopardize our ability to commercialize our Candidates, if approved, and generate revenue.

The production of SGT-003, SGT-212 and SGT-501 uses a transient transfection-based process which requires processing steps that are more complex than those required for most chemical pharmaceuticals. We also intend to use transient transfection manufacturing for our other Candidates. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a gene therapy candidate such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we have and will continue to employ multiple steps to control our manufacturing processes to assure that the process works and that SGT-003, SGT-212, SGT-501 and our other Candidates are made strictly and consistently in compliance with such processes. We must supply all necessary documentation in support of an IND, BLA or MAA on a timely basis and must adhere to the FDA's and the European Union's cGMP requirements before we can obtain marketing approval for SGT-003, SGT-212, SGT-501, and other Candidates. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP requirements, by performing extensive audits of contract laboratories, manufacturers and suppliers.

We currently rely on multiple third-party manufacturers for supply of SGT-003, SGT-212 and SGT-501 and plan to rely on third-party manufacturers for our other Candidates. In order to produce sufficient quantities of Candidates for clinical trials and initial U.S. commercial demand, we have and will continue to further optimize and increase the capacity of our manufacturing process at our third-party manufacturers. We may need to make changes to our manufacturing processes, beyond implementation of a transient transfection-based manufacturing process. We may not be able to produce sufficient quantities of drug product due to several factors, including capacity constraints, equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, a public health issue (for example, an outbreak of a contagious disease such as the recent COVID-19 pandemic), disruption in utility services, human error or disruptions in the operations of our suppliers. We may experience variability with respect to the success and yield between lots that will require continued engagement in process development activities to improve the reproducibility, reliability, quality and consistency of yields of the manufacturing process. Additional manufacturing runs will be required to produce necessary or adequate supply for our future clinical trials and there is no guarantee that all of those runs will be within specifications or produce adequate supply. If we are not able to produce sufficient supply on the timeline expected, our overall development schedule for SGT-003, SGT-212, SGT-501 and other Candidates could be delayed, and we could incur additional expense. Any such failure could delay or prevent development and commercialization of SGT-003, SGT-212, SGT-501 or other Candidates.

If supply from a manufacturing facility is interrupted, including as a result of capacity constraints, equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, public health emergencies or pandemics, such as the recent COVID-19 pandemic, disruption in utility services or human error, there could be a significant disruption in supply of SGT-003 or other Candidates. In such instance, we may need to locate appropriate replacement third-party manufacturers, and we may not be able to enter into arrangements with such additional third-party manufacturers on favorable terms or at all. Use of new third-party manufacturers could increase the risk of delays in production or insufficient supplies of our Candidates as we transfer our manufacturing technology to these manufacturers and as they gain experience manufacturing our Candidates.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

In addition, the FDA, the EMA and other foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Lot failures or product recalls could cause us to delay or abandon clinical trials or product launches.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to oversee our manufacturing and quality control process, which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or facilities could make us a less attractive collaborator for potential partners, including biotechnology and pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in our manufacturing process or facilities also could restrict our ability to meet market demand for our Candidates.

We expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.

We do not independently manufacture material for our ongoing or planned clinical programs and we are utilizing and expect to utilize materials manufactured by cGMP-compliant third-party suppliers. If these third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture our Candidates in accordance with quality and regulatory requirements or if there are disagreements between us and these third-party manufacturers, we may not be able to complete, or may be delayed in completing, the clinical trials required for approval of our Candidates. In such instances, we may need to locate an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of our Candidates.

Additionally, we rely on our third-party manufacturers for their compliance with the cGMP and their maintenance of adequate quality control, quality assurance and qualified personnel. Furthermore, all of our third-party suppliers and manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes them to regulatory risks for the production of such materials and products. FDA inspections may identify compliance issues at third-party manufacturer facilities or at the facilities of third-party suppliers that may disrupt production or distribution, or require substantial resources to correct and prevent recurrence of any deficiencies, and could result in fines or penalties by regulatory authorities. In addition, discovery of problems with a product or the failure to comply with applicable requirements may result in restrictions on a product, manufacturer or holder of an approved BLA, including withdrawal or recall of the product from the market or other voluntary, FDA-initiated or judicial action, including fines, injunctions, civil penalties, license revocations, seizure, total or partial suspension of production or criminal penalties, any of which could significantly and adversely affect supplies of our Candidates.

In addition, we do not currently have long-term supply or manufacturing arrangements in place for the production of our Candidates at commercial scale. Although we intend to establish additional sources for long-term supply, from one or more third-party manufacturers, if the gene therapy industry were to grow, we may encounter increasing competition for the materials necessary for the production of Candidates. We may experience difficulties in scaling up production beyond clinical batches. Furthermore, demand for third-party cGMP manufacturing facilities may grow at a faster rate than existing manufacturing capacity, which could disrupt our ability to find and retain third-party manufacturers capable of producing sufficient quantities of our Candidates for future clinical trials or to meet initial commercial demand in the United States. We currently rely, and expect to continue to rely, on additional third parties to manufacture materials for our Candidates and to perform quality testing. We intend to maintain third-party manufacturers for these materials, as well as to serve as additional sources of our Candidates, which will expose us to risks including:

- reduced control of manufacturing activities;
- the inability of certain CMOs to produce our Candidates in the necessary quantities, or in compliance with current cGMP or in compliance with pertinent regulatory requirements and within our planned time frame and cost parameters;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturer and our and their suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier, natural disasters or public health issues.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize our Candidates. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of product manufacture.

If we are unable to establish sales, distribution and marketing capabilities or enter into agreements with third parties to market and sell our Candidates, we will be unable to generate any product revenue.

We currently have no sales, distribution or marketing organization. To successfully commercialize any Candidate that may result from our development programs, we will need to develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any Candidate we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaborations regarding our Candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize our Candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We will also face competition in our search for third parties to assist us with the sales and marketing efforts of any future products. Without an internal team or the support of a third party to perform marketing and sales functions, we will be unable to compete successfully against these more established companies.

If we are unable to establish medical affairs capabilities, we will be unable to establish an educated market of physicians to administer any future products.

We currently have no medical affairs team. If we are unable to successfully build a medical affairs team to address scientific and medical questions and provide expert guidance and education in the application, administration and utilization of any future products to physicians, we may not be able to establish an educated market for our products. The establishment and development of our own medical affairs team will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability.

If the market opportunities for any of our future products are smaller than we believe they are, our revenue prospects may be adversely affected and our business may suffer.

We currently focus our research and product development on treatments for rare genetic neuromuscular and cardiac indications. Our understanding of the patient population with these diseases is based on estimates in published literature and by disease-focused foundations. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our Candidates or patients may become increasingly difficult to identify and access.

Further, there are several factors that could contribute to reducing the actual number of patients who could receive our Candidates less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a degenerative disease such as Duchenne and FA up to the time of treatment will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell damage.

Certain patients' immune systems might prohibit the successful delivery of certain gene therapy products, thereby potentially limiting the population of patients amenable to gene transfer.

As with many AAV-mediated gene therapy approaches, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products, thereby potentially limiting the population of patients amenable to gene transfer. While we are working to better understand the prevalence of antibodies to AAV, or seroprevalence, as it relates to gene therapy, the exact seroprevalence is currently unknown and varies by AAV serotype and age. We may not be able to address these potentially limiting factors for gene therapy as a treatment for certain patients.

The commercial success of any of our Candidates, if approved, will depend upon market acceptance by physicians, patients, third-party payors and others in the medical community.

Even with the requisite approvals from the FDA in the United States, the European Commission in the European Union and other regulatory authorities internationally, the commercial success of our Candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and, in particular for each of our current

and future Candidate, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community due to ethical, social, medical and legal concerns. If our products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, our current and future Candidates, if approved for commercial sale, will depend on multiple factors, including:

- the efficacy and safety of our current and future Candidates as demonstrated in clinical trials;
- the efficacy and potential and perceived advantages of our Candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which our Candidates are approved by the FDA, the European Commission or other regulatory authorities, as applicable;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products to meet market demand;
- publicity concerning our Candidates or competing products and treatments;
- any restrictions on the use of our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

Even if a potential Candidate displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

Our efforts to educate the medical community and third-party payors on the benefits of our Candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential Candidates. If our Candidates are approved but fail to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenue from any such product.

Our gene transfer approach utilizes capsids derived from a virus, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of gene transfer Candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our Candidates.

Gene transfer remains a novel technology that faces many challenges imposed by the humoral immune response. The immunogenicity of AAV gene transfers is a very complex process that we and others continue to work understand through the extensive clinical experience that now exists over a broad spectrum of therapeutic areas and indications. Marked inflammatory toxicities have been observed, including complement activation, cytopenias, severe hepatotoxicity as well as transgene related toxicities representing part of the continuum of diverse aspects of clinical immune responses that can be observed post gene transfer.

In particular, our success will depend upon physicians who specialize in the treatment of our pipeline indications, prescribing treatments that involve the use of viral capsids in lieu of, or in addition to, other treatments with which they are more familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion may delay or impair the development and commercialization or demand for any Candidate we may develop. A public backlash developed against gene therapy following the death of a patient in 1999 during a gene therapy clinical trial of research subjects with ornithine transcarbamylase, or OTC, deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene. The death of the clinical trial subject was due to complications of adenovirus capsid administration. Dr. James M.

Wilson, former chair of our Scientific Advisory Board, was a co-investigator of the 1999 trial while he was Director of the Institute for Human Gene Therapy of the University of Pennsylvania. Serious adverse events in our clinical trials, including the events that led to the previously-lifted clinical holds on IGNITE DMD or other clinical trials involving gene transfer products or our competitors' products, even if not ultimately attributable to the relevant Candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our Candidates, stricter labeling requirements for our Candidates, if approved, and a decrease in demand for our Candidates.

Any contamination in our manufacturing process, shortages of materials or failure of any of our key suppliers to deliver necessary components could result in interruption in the supply of our Candidates and delays in our clinical development or commercialization schedules.

Given the nature of biologics manufacturing, there is a risk of contamination in our manufacturing processes. Any contamination could materially adversely affect our ability to produce our Candidates on schedule and could cause reputational damage.

Some of the materials required in our manufacturing process are derived from biologic sources. Such materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our Candidates could adversely impact or disrupt the manufacturing or the production of clinical material, which could materially and adversely affect our development timelines.

The insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our Candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We expect the cost of a single administration of gene transfer products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our future products, if approved, will depend substantially, both domestically and abroad, on the extent to which the costs of such Candidates will be paid by health maintenance, managed care, pharmacy benefit and similar health care management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective;
- durable and a one-time treatment, as applicable; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our future products, if approved. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

To our knowledge, only a limited number of gene transfer products have been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services, or the CMS, the agency responsible for administering the Medicaid program. It is difficult to predict what the CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these types of products either in the United States or the European Union. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union member states and vice versa. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our future products, if approved.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenue, if any.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In general, the prices of therapeutics outside the United States are substantially lower than in the United States. Other countries may allow companies to fix their own prices for therapeutics, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulations could restrict the amount that we are able to charge for our Candidates. Accordingly, in markets outside the United States, the reimbursement for our Candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenue.

Additionally, in countries where the pricing of gene therapy products is subject to governmental control, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has been obtained. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our Candidate to other available therapies. Reimbursement of our products may be unavailable or limited in scope or amount, which would adversely affect our revenue, if any.

If we obtain approval to commercialize our future products outside of the United States, in particular in the European Union, a variety of risks associated with international operations could materially adversely affect our business.

We expect that we will be subject to additional risks in commercializing our future products, if approved, outside the United States, including:

- different regulatory requirements for approval of therapeutics in foreign countries;
- reduced protection for intellectual property rights;
- the existence of additional third-party patent rights of potential relevance to our business;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- foreign reimbursement, pricing and insurance regimes;
- production shortages resulting from any events affecting material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires.

The failure to comply with applicable foreign regulatory requirements may result in, among other things, fines, suspension, variation or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

If we engage in future acquisitions or strategic collaborations, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may evaluate various acquisitions and strategic collaborations, including licensing or acquiring complementary products, intellectual property rights, technologies or businesses. Any potential acquisition or strategic collaboration may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;

- the diversion of our management's attention from our existing Candidates and initiatives in pursuing such acquisition or strategic collaboration;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or collaboration or even to offset transaction costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition or collaboration opportunities and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

Risks Related to our Business Operations

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on members of our executive team, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with certain of our executive officers, any of them could leave our employment at any time. We currently do not have "key person" insurance on any of our employees. The loss of the services of one or more of our current key employees might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, also will be critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and capsid manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, the failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives.

If we are unable to manage growth in the scale and complexity of our operations, our performance may suffer.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of our current and future Candidates and products that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and any future Candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. This could include violations of the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, other U.S. federal and state law, and requirements of non-U.S.

jurisdictions, including the European Union Data Protection Directive. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards, regulations, guidance or codes of conduct. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our Candidates and may affect the prices we may set.

Our business and financial prospects could be affected by changes in health care spending and policy in the United States and abroad. We operate in a highly regulated industry and new laws or judicial decisions, or new interpretations of existing laws or decisions, related to health care availability, the method of delivery or payment for health care products and services could negatively impact our business, operations and financial condition.

For example, in the United States there is significant interest in promoting health care reform, as evidenced by the enactment of the Patient Protection and Affordable Care Act and the companion Health Care and Education Reconciliation Act, or the Health Care Reform Law. The Health Care Reform Law increased federal oversight of private health insurance plans and included a number of provisions designed to reduce Medicare expenditures and the cost of health care generally, to reduce fraud and abuse, and to provide access to increased health coverage.

The Health Care Reform Law also imposed substantial changes to the U.S. system for paying for health care, including programs to extend medical benefits to millions of individuals who have lacked insurance coverage. Generally, implementation of the Health Care Reform Law has thus far included significant cost-saving, revenue and payment reduction measures with respect to, for example, several government health care programs that might cover our products in the United States, should they be commercialized, including Medicaid and Medicare. Additional downward pricing pressure associated with the Health Care Reform Law includes that the Health Care Reform Law established and provided significant funding for a Patient-Centered Outcomes Research Institute to coordinate and fund Comparative Effectiveness Research, as those terms are defined in the Health Care Reform Law. While the stated intent of Comparative Effectiveness Research is to develop information to guide providers to the most efficacious therapies, outcomes of Comparative Effectiveness Research could influence the reimbursement or coverage for therapies that are determined to be less cost-effective than others. Should any of our products be approved for sale, but then determined to be less cost-effective than alternative therapies, the levels of reimbursement for these products, or the willingness to reimburse at all, could be adversely impacted.

In addition to legislative changes resulting from the passage of the Health Care Reform Law, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, led to aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which will remain in effect through the first half of 2032. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester through 2031. These Medicare sequester reductions were suspended through June 2022, with the full 2% cut resuming thereafter. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our Candidates for which we may obtain regulatory approval or the frequency with which any such Candidate is prescribed or used. Indeed, under current legislation, the actual reductions in Medicare payments may vary up to 4%. The Consolidated Appropriations Act, which was signed into law by President Biden in December 2022, made several changes to sequestration of the Medicare program. Section 1001 of the Consolidated Appropriations Act delays the 4% Statutory Pay-As-You-Go Act of 2010 sequester for two years, through the end of calendar year 2024. Triggered by enactment of the American Rescue Plan Act of 2021, the 4% cut to the Medicare program would have taken effect in January 2023. The Consolidated Appropriations Act's health care offset title includes Section 4163, which extends the 2% Budget Control Act of 2011 Medicare sequester for six months into fiscal year 2032 and lowers the payment reduction percentages in fiscal years 2030 and 2031.

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act

of 2017, or the TCJA, Congress repealed the "individual mandate." The repeal of this provision of the Health Care Reform Law, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the Health Care Reform Law is an essential and inseverable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the Health Care Reform Law are invalid as well. The U.S. Supreme Court heard this case on November 10, 2020 and on June 17, 2021, dismissed this action after finding that the plaintiffs do not have standing to challenge the constitutionality of the statute. It is unclear how such litigation and other efforts to repeal and replace the Health Care Reform Law will impact the Health Care Reform Law and our business. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

Current and future legislative efforts may limit the prices for our products, if and when they are licensed for marketing and that could materially impact our ability to generate revenues.

The costs and prices of prescription pharmaceuticals have been the subject of considerable discussion in the United States. To date, there have been several U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the current administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

In addition, in October 2020, the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Several states have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. Florida now has authority to import certain products from Canada for a period of two years once certain conditions are met. Florida will first need to submit a pre-import request for each product selected for importation, which must be approved by the FDA. Florida will also need to relabel the products and perform quality testing of the products to meet FDA standards. Further, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law.

Further, on November 20, 2020, the HHS finalized a regulation removing safe harbor protection for price reductions from pharmaceutical manufacturers to plan sponsors under Medicare Part D, either directly or through pharmacy benefit managers, unless the price reduction is required by law. The rule also creates a new safe harbor for price reductions reflected at the point-of-sale, as well as a new safe harbor for certain fixed fee arrangements between pharmacy benefit managers and manufacturers, the implementation of which has been delayed until January 1, 2032 by the Inflation Reduction Act, or IRA.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation; and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of HHS to implement many of these provisions through guidance, as opposed to regulation, for the initial years.

Specifically, with respect to price negotiations, Congress authorized Medicare to negotiate lower prices for certain costly single-source drug and biologic products that do not have competing generics or biosimilars and are reimbursed under Medicare Part B and Part D. CMS may negotiate prices for ten high-cost drugs paid for by Medicare Part D starting in 2026, followed by 15 Part D drugs in 2027, 15 Part B or Part D drugs in 2028, and 20 Part B or Part D drugs in 2029 and beyond. This provision applies to drug products that have been approved for at least 9 years and biologics that have been licensed for 13 years, but it does not apply to drugs and biologics that have been approved for a single rare disease or condition. The first cycle of negotiations for the Medicare Drug Price Negotiation Program commenced in the summer of 2023. On August 15, 2024, HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions, including diabetes, chronic kidney disease, and rheumatoid arthritis. The prices of these ten drugs will become effective January 1, 2026. In August 2024, HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions and the prices will become effective January 1, 2026. In January 2025, CMS announced the next 15 drug and biologic prices that will be subject to the IRA's price negotiation provisions. This second cycle of negotiations with participating drug companies will occur during 2025, and any negotiated prices for this second set of drugs will be effective starting January 1, 2027. CMS issued a public statement on January 2025, declaring that lowering

the cost of prescription drugs is a top priority of the new administration and CMS is committed to considering opportunities to bring greater transparency in the negotiation program.

Further, the legislation subjects drug manufacturers to civil monetary penalties and a potential excise tax for failing to comply with the legislation by offering a price that is not equal to or less than the negotiated "maximum fair price" under the law or for taking price increases that exceed inflation. The legislation also requires manufacturers to pay rebates for drugs in Medicare Part D whose price increases exceed inflation. The new law also caps Medicare out-of-pocket drug costs at an estimated $2,000 a year beginning in 2025.

The IRA includes a provision exempting orphan drugs from Medicare price negotiation but this exclusion has been interpreted by CMS in final guidance issued in July 2023 to apply only to those orphan drugs with an approved indication (or indications) for a single rare disease or condition. The final guidance clarifies that CMS will consider only active designations/approvals when evaluating a drug for the exclusion, such that designations/indications withdrawn before the selected drug publication date will not be considered. CMS also clarified that, if a drug loses its orphan drug exclusion status, the agency will use the earliest date of approval/licensure to determine whether the product is a qualifying single source drug subject to price negotiations.

In June 2023, Merck filed a lawsuit against HHS and CMS asserting that, among other things, the IRA's Drug Price Negotiation Program for Medicare constitutes an uncompensated taking in violation of the Fifth Amendment of the Constitution. Subsequently, a number of other parties, including pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. There have been various decisions by the courts considering these cases since they were filed. We expect that litigation involving these and other provisions of the IRA will continue with unpredictable and uncertain results. Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. A number of states, for example, require drug manufacturers and other entities in the drug supply chain, including health carriers, pharmacy benefit managers, wholesale distributors, to disclose information about pricing of pharmaceuticals. This is increasingly true with respect to products approved pursuant to the accelerated approval pathway. State Medicaid programs and other payers are developing strategies and implementing significant coverage barriers, or refusing to cover these products outright, arguing that accelerated approval drugs have insufficient or limited evidence despite meeting the FDA's standards for accelerated approval. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, if approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our Candidates or additional pricing pressures.

It is likely that federal and state legislatures within the United States and foreign governments will continue to consider changes to existing health care legislation. We cannot predict the reform initiatives that may be adopted in the future or whether initiatives that have been adopted will be repealed or modified. The continuing efforts of the government, insurance companies, managed care organizations and other health care payors of to contain or reduce costs of health care may adversely affect:

- the demand for any Candidates for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to obtain coverage and reimbursement approval for a product;
- our ability to generate revenue and achieve or maintain profitability; and
- the level of taxes that we are required to pay.

Finally, in the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our Candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the

establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of healthcare and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our Candidates, restrict or regulate post-approval activities and affect our ability to commercialize our Candidates, if approved.

In markets outside of the United States and the European Union, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action in the United States, the European Union or any other jurisdiction. If we or any third parties we may engage are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we or such third parties are not able to maintain regulatory compliance, our Candidates may lose any regulatory approval that may have been obtained and we may not achieve or sustain profitability.

Our relationships with customers, physicians and third-party payors will be subject, directly or indirectly, to federal and state health care fraud and abuse laws, false claims laws, health information privacy and security laws, and other health care laws and regulations. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA approval for our current or future Candidates and begin commercializing one or more of those products in the United States, our operations will be directly or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal laws and the Physician Payment Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal health care program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The Health Care Reform Law amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. The Health Care Reform Law provides and recent government cases against pharmaceutical and medical device manufacturers support the view that Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any health care benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;

- federal transparency laws, including the federal Physician Payment Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the CMS information related to: (i) payments or other "transfers of value" made to physicians, other healthcare professionals and teaching hospitals and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other health care providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states; and
- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations.

The risk of our being found in violation of these laws is increased by the fact that many of them have not been fully interpreted by the regulatory authorities or the courts, and their provisions are open to a variety of interpretations. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. The shifting compliance environment and the need to build and maintain robust and expandable systems to comply with multiple jurisdictions with different compliance and/or reporting requirements increases the possibility that we may run afoul of one or more of the requirements.

We are subject to stringent privacy laws, information security laws, regulations, policies and contractual obligations related to data privacy and security and changes in such laws, regulations, policies, contractual obligations and failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

We are subject to data privacy and protection laws and regulations that apply to the collection, transmission, storage and use of personally-identifying information, which among other things, impose certain requirements relating to the privacy, security and transmission of personal information, including comprehensive regulatory systems in the United States, EU and UK. The legislative and regulatory landscape for privacy and data protection continues to evolve in jurisdictions worldwide, and there has been an increasing focus on privacy and data protection issues with the potential to affect our business. Failure to comply with any of these laws and regulations could result in enforcement action against us, including fines, imprisonment of company officials and public censure, claims for damages by affected individuals, damage to our reputation and loss of goodwill, any of which could have a material adverse effect on our business, financial condition, results of operations or prospects.

There are numerous U.S. federal and state laws and regulations related to the privacy and security of personal information. In particular, regulations promulgated pursuant to HIPAA establish privacy and security standards that limit the use and disclosure of individually identifiable health information, or protected health information, and require the implementation of administrative, physical and technological safeguards to protect the privacy of protected health information and ensure the confidentiality, integrity and availability of electronic protected health information. Determining whether protected health information has been handled in compliance with applicable privacy standards and our contractual obligations can be complex and may be subject to changing interpretation. These obligations may be applicable to some or all of our business activities now or in the future.

If we are unable to properly protect the privacy and security of protected health information, we could be found to have breached our contracts. Further, if we fail to comply with applicable privacy laws, including applicable HIPAA privacy and security standards, we could face civil and criminal penalties. HHS enforcement activity can result in financial liability and reputational harm, and responses to such enforcement activity can consume significant internal resources. In addition, state attorneys general are authorized to bring civil actions seeking either injunctions or damages in response to violations that threaten the privacy of state residents. We cannot be sure how these regulations will be interpreted, enforced or applied to our operations. In addition to the risks associated with enforcement activities and potential contractual liabilities, our ongoing efforts to comply with evolving laws and regulations at the federal and state level may be costly and require ongoing modifications to our policies, procedures and systems.

In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA and other California privacy laws, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities.

In addition to California, several other states have passed comprehensive privacy laws similar to the CCPA and CPRA. These laws are either in effect or will go into effect sometime over the next several years. Like the CCPA and CPRA, these laws create obligations related to the processing of personal information, as well as special obligations for the processing of "sensitive" data (which includes health data in some cases). Some of the provisions of these laws may apply to our business activities. There are also states that are strongly considering privacy laws that will go into effect over the next several years. Other states will be considering these laws in the future, and Congress has also been debating passing a federal privacy law. There are also states that are specifically regulating health information that may affect our business. These laws may impact our business activities, including our identification of research subjects, relationships with business partners and ultimately the marketing and distribution of our products.

Plaintiffs' lawyers are also increasingly using privacy-related statutes at both the state and federal level to bring lawsuits against companies for their data-related practices. In particular, there have been a significant number of cases filed against companies for their use of pixels and other web trackers. These cases often allege violations of the California Invasion of Privacy Act and other state laws regulating wiretapping, as well as the federal Video Privacy Protection Act. The rise in these types of lawsuits could create potential risk for our business.

Similar to the laws in the United States, there are significant privacy and data security laws that apply in Europe and other countries. The collection, use, disclosure, transfer, or other processing of personal data, including personal health data, regarding individuals who are located in the European Economic Area, or EEA, and the processing of personal data that takes place in the EEA, is regulated by the GDPR, which went into effect in May 2018 and which imposes obligations on companies that operate in our industry with respect to the processing of personal data and the cross-border transfer of such data. The GDPR imposes onerous accountability obligations requiring data controllers and processors to maintain a record of their data processing and policies. If our or our partners' or service providers' privacy or data security measures fail to comply with the GDPR requirements, we may be subject to litigation, regulatory investigations, enforcement notices requiring us to change the way we use personal data and/or fines of up to 20 million Euros or up to 4% of the total worldwide annual turnover of the preceding financial year, whichever is higher, as well as compensation claims by affected individuals, negative publicity, reputational harm and a potential loss of business and goodwill.

The GDPR places restrictions on the cross-border transfer of personal data from the EU to countries that have not been found by the European Commission to offer adequate data protection legislation, such as the United States. There are ongoing concerns about the ability of companies to transfer personal data from the EU to other countries. In July 2020, the Court of Justice of the European Union, or the CJEU, invalidated the EU-U.S. Privacy Shield, one of the mechanisms used to legitimize the transfer of personal data from the EEA to the U.S. The CJEU decision also drew into question the long-term viability of an alternative means of data transfer, the standard contractual clauses, for transfers of personal data from the EEA to the U.S. While we were not self-certified under the Privacy Shield, this CJEU decision may lead to increased scrutiny on data transfers from the EEA to the U.S. generally and increase our costs of compliance with data privacy legislation as well as our costs of negotiating appropriate privacy and security agreements with our vendors and business partners.

Following the withdrawal of the UK from the EU, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the UK and includes parallel obligations to those set forth by GDPR. In relation to data transfers, both the UK and the EU have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the UK Data Protection Act and the GDPR, respectively. The UK and the U.S. have also agreed to a U.S.-UK "Data Bridge", which functions similarly to the EU-U.S. Data Privacy Framework and provides an additional legal mechanism for companies to transfer data from the UK to the United States. In addition to the UK, Switzerland is also in the process of approving an adequacy decision in relation to the Swiss-U.S. Data Privacy Framework (which would function similarly to the EU-U.S. Data Privacy Framework and the U.S.-UK Data Bridge in relation to data transfers from Switzerland to the United States). Any changes or updates to these developments have the potential to impact our business.

Additionally, in October 2022, President Biden signed an executive order to implement the EU-U.S. Data Privacy Framework, which serves as a replacement to the EU-U.S. Privacy Shield. The European Commission initiated the process to adopt an adequacy decision for the EU-U.S. Data Privacy Framework in December 2022 and the European Commission adopted the adequacy decision on July 10, 2023. The adequacy decision permits U.S. companies who self-certify to the EU-U.S. Data Privacy Framework to rely on it as a valid data transfer mechanism for data transfers from the EU to the U.S. However, some privacy advocacy groups have already suggested that they will be challenging the EU-U.S. Data Privacy Framework. If these challenges are successful, they may not only impact the EU-U.S. Data Privacy Framework, but also further limit the viability of the standard contractual clauses and other data transfer mechanisms. The uncertainty around this issue has the potential to impact our business internationally.

Following Brexit, the UK Data Protection Act 2018 applies to the processing of personal data that takes place in the United Kingdom and includes parallel obligations to those set forth by GDPR. In relation to data transfers, both the United Kingdom and the EU have determined, through separate "adequacy" decisions, that data transfers between the two jurisdictions are in compliance with the UK Data Protection Act and the GDPR, respectively. Any changes or updates to these adequacy decisions have the potential to impact our business.

Beyond GDPR, there are privacy and data security laws in a growing number of countries around the world. While many loosely follow GDPR as a model, other laws contain different or conflicting provisions. These laws will impact our ability to conduct our business activities, including both our clinical trials and the sale and distribution of commercial products, through increased compliance costs, costs associated with contracting and potential enforcement actions.

While we continue to address the implications of the recent changes to data privacy regulations, data privacy remains an evolving landscape at both the domestic and international level, with new regulations coming into effect and continued legal challenges, and our efforts to comply with the evolving data protection rules may be unsuccessful. It is possible that these laws may be interpreted and applied in a manner that is inconsistent with our practices. We must devote significant resources to understanding and complying with this changing landscape. Failure to comply with laws regarding data protection would expose us to risk of enforcement actions taken by data protection authorities in the EEA and elsewhere and carries with it the potential for significant penalties if we are found to be non-compliant. Similarly, failure to comply with federal and state laws in the United States regarding privacy and security of personal information could expose us to penalties under such laws. Any such failure to comply with data protection and privacy laws could result in government-imposed fines or orders requiring that we change our practices, claims for damages or other liabilities, regulatory investigations and enforcement action, litigation and significant costs for remediation, any of which could adversely affect our business. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our business, financial condition, results of operations or prospects.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, be precluded from developing manufacturing and selling certain products outside the United States or be required to develop and implement costly compliance programs, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the U.K. Bribery Act 2010, or Bribery Act, the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. Compliance with the FCPA, in particular, is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted. If we expand our operations outside of the United States, we will need to dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, collectively referred to as the Trade Control laws. In addition, various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. If we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and Candidates outside of the United States, which could limit our growth potential and increase our development costs.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA or other legal requirements, including Trade Control laws. If we are not in compliance with the Bribery Act, the FCPA and other anti-corruption laws or Trade Control laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. The Securities and Exchange Commission also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions. Any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control laws by United Kingdom, U.S. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit commercialization of any Candidate that we may develop.

We face an inherent risk of product liability exposure related to the testing of SGT-003 and any of our current and future Candidates in preclinical studies and clinical trials and may face an even greater risk if we commercialize any Candidate that we may develop. If we cannot successfully defend ourselves against claims that our Candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any Candidate that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to commercialize any of our Candidates; and
- injury to our reputation and significant negative media attention.

Although we maintain product liability insurance coverage, such insurance may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we commence additional clinical trials and if we successfully commercialize any Candidate. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could have a material adverse effect on the success of our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and viruses and other biologic materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages. We also could incur significant costs associated with civil or criminal fines and penalties. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Although we maintain workers' compensation insurance for certain costs and expenses, we may incur due to injuries to our employees resulting from the use of hazardous materials or other work-related injuries, this insurance may not provide adequate coverage against potential

liabilities. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities.

Our internal computer systems, or those of our collaborators, contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our product development.

Despite the implementation of security measures, our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such systems are also vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, third-party vendors and/or business partners, or from cyber-attacks by malicious third parties. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial-of-service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient.

While we are not aware of any such material system failure, accident, cyber-attack or security breach to date, if such an event were to occur and cause interruptions in our or our collaborators', contractors' or consultants' operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from preclinical studies or clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our current and other future Candidates could be delayed.

Risks Related to our Intellectual Property

We heavily rely on certain in-licensed patents and other intellectual property rights in connection with our development of our Candidates and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize our Candidates.

Our ability to develop and commercialize our Candidates is heavily dependent on licenses to patent rights and other intellectual property granted to us by third parties. In particular, we have licensed certain patents and patent applications from the University of Missouri, the University of Washington and others that are important or necessary to the development of SGT-003, our other Candidates and other elements of our gene transfer program. We have further licensed certain intellectual property from the University of Pennsylvania and others that are important or necessary to the development of SGT-212 and from ICS Maugeri that are important or necessary to the development of SGT-501. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization obligations, milestone payments, royalties and other obligations on us. If we fail to comply with our obligations under our agreements, we may be subject to damages, which may be significant, and the licensor may have the right to terminate the license, in which event we may not be able to develop or market Candidates or technologies covered by the license. In addition, certain of these license agreements are not assignable by us without the consent of the respective licensor, which may have an adverse effect on our ability to engage in certain transactions.

Under certain of our existing license agreements, we do not have, and under future license agreements we may not have, the right to control the preparation, filing and prosecution of patent applications, or the maintenance, enforcement and defense of the patents and patent applications that we license from third parties. For example, under our inbound license agreements with the University of Missouri and the University of Washington, each of the applicable licensors controls the prosecution of patent applications and the maintenance of patents and patent applications. Therefore, we cannot be certain that the licensed patents and applications will be prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to maintain, enforce or defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our Candidates that are the subject of such licensed rights could be adversely affected. For more information, see Part I, Item 1, "Business—Strategic Partnerships and Collaborations/Licenses" of this Annual Report on Form 10-K.

Moreover, licenses to additional third-party intellectual property, technology and materials may be required for our development programs but may not be available in the future or may not be available on commercially reasonable terms. For example, third parties may claim that the constructs containing the gene or protein of interest and the AAV capsids we are developing for use in product candidates are covered by patents held by them. We believe that we would have valid defenses to any such claims; however, if any such claims were ultimately successful, we might require a license to continue to use and sell Candidates and such AAV capsids. Such licenses may not be available on commercially reasonable terms, or at all. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. Moreover, even if we are able to obtain such licenses, they may only be non-exclusive, which could permit competitors and other third parties to use the same intellectual property in competition with us.

We may collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. These institutions may provide us with an option to negotiate a license to any of the institution's licensable rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the required timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights, or successfully challenge such rights, to any third-party intellectual property rights that are required for the development and commercialization of our Candidates, and such third-party intellectual property rights are successfully asserted against us, we may be liable for damages, which may be significant, and we may be required to cease the development and commercialization of our Candidates.

If we are unable to obtain and maintain patent protection for our Candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our Candidates may be adversely affected.

Our success depends, in large part, on our and our licensors' ability to seek, obtain, maintain, enforce and defend patent rights in the United States and other countries with respect to our Candidates and our future innovation related to our manufacturing technology. Our licensors and we have sought, and we intend to continue to seek, to protect our proprietary position by filing patent applications in the United States and, in at least some cases, one or more countries outside the United States related to our Candidates that are important to our business. However, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents or whether the claims of any issued patents will provide us with a competitive advantage.

Moreover, although we have pending patent applications in the United States and abroad, we cannot predict whether or in which jurisdictions the pending applications will result in issuance of patents that effectively protect any of our Candidates or will effectively prevent others from commercializing competitive products. Further, each of the provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of each provisional patent application. If we do not timely file a non-provisional patent application in respect of a provisional patent application, we may lose our priority date with respect to such provisional patent application and any patent protection on the inventions disclosed in such provisional patent application. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether such future patent applications will result in the issuance of patents that effectively protect any of our Candidates or will effectively prevent others from commercializing competitive products.

In addition, foreign regulatory authorities may change their approval policies and new regulations may be enacted regarding non-patent exclusivity. For example, EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission's proposal for revision of several legislative instruments related to medicinal products, which may reduce the duration of regulatory data protection and exclusivity periods for orphan drugs, and revise the eligibility for expedited pathways in addition to other changes, was published on April 26, 2023. On April 10, 2024, the European Parliament adopted a position on the proposal requesting several amendments to the package. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may, however, have a significant impact on the pharmaceutical industry and our business in the long term.

We may not be able to file, prosecute, maintain, enforce, defend or license all patents that are necessary to our business.

The patent prosecution process is expensive, time-consuming and complex, and we and our licensors may not be able to file, prosecute, maintain, enforce, defend or license all necessary or desirable patents and patent applications at a reasonable cost or in a timely manner.

It is also currently unknown what claims may, if ever, issue from pending applications included in our patent rights. Additionally, certain of our in-licensed U.S. patent rights lack corresponding foreign patents or patent applications, and therefore we will be unable to obtain patent protection for our Candidates in certain jurisdictions. We or our licensors may not be able to obtain or maintain patent protection with respect to our Candidates.

Changes in either the patent laws or their interpretation in the United States and other countries may diminish our ability to protect our inventions, obtain, maintain and enforce our intellectual property rights, and more generally, could affect the value of our intellectual property rights or narrow the scope of our licensed patents or future owned patents.

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Patent applications included in our current and future patent rights may not result in patents being issued that protect our Candidates, effectively prevent others from commercializing competitive products or otherwise provide any competitive advantage. In fact, patent applications may not issue as patents at all. Even assuming patents issue from patent applications in which we have rights, changes in either the patent laws or interpretation of the patent laws in the United States and other jurisdictions may diminish the value of our patents or narrow the scope of our patent protection.

Other parties have developed products that may be related or competitive to our own and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to our Candidates. In addition, we cannot provide any assurances that any of the inventions disclosed in our patent applications will be found to be patentable, including over third-party or our own prior art patents, publications or other disclosures, or will issue as patents. Even if our patent applications issue as patents, we cannot provide any assurances that such patents will not be challenged or ultimately held to be invalid or unenforceable. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and in other jurisdictions are typically not published until 18 months after filing, or, in some cases, at all. Therefore, we cannot know with certainty whether the inventors of our licensed patents and applications were the first to make the inventions claimed in those patents or pending patent applications, or that they were the first to file for patent protection of such inventions. Similarly, should we own any issued patents or patent applications in the future, we may not be certain that we were the first to file for patent protection for the inventions claimed in such patents or patent applications. Furthermore, given the differences in patent laws in the United States, Europe and other foreign jurisdictions, for example, the availability of grace periods for filing patent applications and what can be considered as prior art, we cannot make any assurances that any claims in our pending and future patent applications in the United States or other jurisdictions will issue, or if they do issue, whether they will issue in a form that provides us with any meaningful competitive advantage. Similarly, we cannot make any assurances that if the patentability, validity, enforceability or scope of our pending or future patents and patent applications in the United States or foreign jurisdictions are challenged by any third party, that the claims of such pending or future patents and patent applications will survive any such challenge in a form that provides us with any meaningful competitive advantage. For example, we are aware of certain third-party patents and publications related to certain microdystrophin constructs. While we believe that our owned or in-licensed patents and patent applications claim novel and non-obvious features of microdystrophin constructs that are not described in such third-party patents or publications, such third-party patents and publications may have earlier priority or publication dates and may be asserted as prior art against our owned or in-licensed patents and applications. Any such challenge, if successful, could limit or eliminate patent protection for our products and Candidates or otherwise materially harm our business. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty.

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from

competing with us or otherwise provide us with any competitive advantage. Any patents that we license or may own in the future may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our Candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative products in a non-infringing manner.

The degree of patent protection we require to successfully compete in the marketplace may be unavailable. We cannot provide any assurances that any of the patents or patent applications included in our patent rights include or will include claims with a scope sufficient to protect our Candidates or otherwise provide any competitive advantage. In addition, the laws of foreign countries may not protect our proprietary rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Certain extensions may be available, however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new Candidates, patents protecting such Candidates might expire before or shortly after such Candidates are commercialized. As a result, our patent rights may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar or identical to our Candidates, including biosimilar versions of such products.

Our licensed patents, and any patents we may own in the future, may be challenged, narrowed, invalidated or held unenforceable.

Even if we acquire patent protection that we expect should enable us to maintain some competitive advantage, third parties, including competitors, may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. In litigation, a competitor could claim that our in-licensed patents or any patents we may own in the future are not valid or enforceable for a number of reasons. If a court agrees, we would lose our rights to those challenged patents. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such proceedings could result in the revocation or cancellation of or amendment to our licensed patents and any patents we may own in the future in such a way that they no longer cover our Candidates.

Even if issued, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our current and future patent rights may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of patents included in our patent rights. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of the pending patent applications included in our patent rights. We may become involved in opposition, derivation, revocation, reexamination, post-grant and *inter partes* review, or interference proceedings challenging one or more patents included in our patent rights. For example, competitors may claim that they invented the inventions claimed in patents or patent applications included in our patent rights, such as the microdystrophin we use in SGT-003, prior to the inventors of such patents or patent applications, or may have filed one or more patent applications before the filing of the patents or patent applications included in our patent rights. A competitor who can establish an earlier filing or invention date may also assert that we are infringing their patents and that we therefore cannot practice our technology related to our Candidates as claimed in the patents or patent applications included in our patent rights. Competitors may also contest patents or patent applications included in our patent rights by showing that the claimed subject matter was not patent-eligible, was not novel or was obvious or that the patent claims failed any other requirement for patentability or enforceability. In addition, we may in the future be subject to claims by our or our licensors' current or former employees or consultants asserting an ownership right in the patents or patent applications included in our patent rights as an inventor or co-inventor, as a result of the work they performed.

An adverse determination in any such submission or proceeding may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar therapeutics, without payment to us, or could limit the duration of the patent protection covering our Candidates. Such challenges may also result in our inability to manufacture or commercialize our Candidates without infringing third-party patent rights, and we may be required to obtain a license from third parties, which may not be available on commercially reasonable terms or at all, or we may need to cease the development, manufacture and commercialization of one or more of our Candidates. In addition, if the breadth or strength of protection provided by the patents and patent applications included in our patent rights is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future Candidates. Such proceedings also may result in substantial cost and require significant time from our scientists and management, even if the eventual outcome is favorable to us.

Even if they are unchallenged, the patents and pending patent applications included in our patent rights may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patent rights by developing similar or alternative therapeutics in a non-infringing manner. For example, a third party may develop a

competitive therapeutic that provides benefits similar to one or more of our Candidates but that uses a capsid or an expression construct that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we license or pursue with respect to our Candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our Candidates could be negatively affected.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

We currently depend, and will continue to depend, on our license, collaboration and other similar agreements. Further development and commercialization of our Candidates and platform technologies may require us to enter into additional license, collaboration or other similar agreements. The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, impact our ability to sublicense the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected Candidates.

If any of our licenses or material relationships are terminated or breached, we may:

- lose our rights to develop and market our Candidates;
- lose patent protection for our Candidates;
- experience significant delays in the development or commercialization of our Candidates;
- not be able to obtain any other licenses on acceptable terms, if at all; or
- incur liability for damages.

These risks apply to any agreements that we may enter into in the future for our Candidates.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We have certain obligations under licensing agreements with third parties that include annual maintenance fees and payments that are contingent upon achieving various development, commercial and regulatory milestones. Pursuant to many of these license agreements, we are required to make milestone payments if certain development, regulatory and commercial sales milestones are achieved, and may have certain additional research funding obligations. Also, pursuant to the terms of many of these license agreements, when and if commercial sales of a licensed product commence, we must pay royalties to our licensors on net sales of the respective licensed products.

We have entered into, or plan to enter into, license agreements with third parties and may need to obtain additional licenses from one or more of these same third parties or from others to advance our research or allow our commercialization of our Candidates. It is possible that we may be unable to obtain such licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to redesign Candidates or the methods for manufacturing them or to develop or license replacement products, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize our Candidates. We cannot provide any assurances that third-party patents or other intellectual property rights do not exist that might be enforced against our manufacturing methods, Candidates or any technologies we may develop, resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

Our existing license agreements provide the licensor sole control of patent prosecution of the licensed technology, and we may be required to reimburse the licensor for their costs of patent prosecution. Future license agreements may require the same. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Further, in certain of our license agreements our licensors have the first right to bring any actions against any third party for infringing on the patents we have licensed. Our

license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing Candidates. Disputes may arise regarding intellectual property subject to our licensing agreements, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our products or processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of licensed patented inventions.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize our Candidates. In spite of our best efforts, our licensors might conclude that we have materially breached our license agreements and might therefore terminate the license agreements, thereby resulting in disputes or litigation, which could cause us to incur substantial costs and distract management's time, and if we are unsuccessful, we could lose our ability to develop and commercialize products covered by these license agreements. If these licenses are ultimately terminated by the licensor, or if the underlying patents fail to provide the intended exclusivity, competitors would have the freedom to seek regulatory approval of, and to market, products identical to ours.

Third parties may initiate legal proceedings alleging that we are infringing, misappropriating or otherwise violating their intellectual property rights, the outcome of which would be uncertain and could have a material adverse effect on the success of our business.

Our commercial success depends upon our ability and the ability of our future collaborators to develop, manufacture, market and sell our Candidates without infringing, misappropriating or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We or our licensors may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our Candidates, including interference proceedings, post grant review and *inter partes* review before the USPTO. Our competitors or other third parties may assert infringement claims against us, alleging that, among other things, our therapeutics, manufacturing methods, formulations or administration methods are covered by their patents.

Given the vast number of patents in our field of technology, we cannot be certain or guarantee that a court would hold that any of our Candidates do not infringe an existing patent or a patent that may be granted in the future. Many companies and institutions have filed, and continue to file, patent applications related to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Furthermore, because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending that may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our Candidates and we may or may not be aware of such patents. If a patent holder believes the manufacture, use, sale or importation of one of our Candidates infringes its patent, the patent holder may sue us even if we have licensed other patent protection for our Candidates. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue and against whom our licensed patent portfolio may therefore have no deterrent effect.

It is also possible that we have failed to identify relevant third-party patents or applications for which we may need a license to develop and commercialize our Candidates. For example, applications filed before November 29, 2000, and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our Candidates because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our Candidates. In addition, we may be unaware of one or more issued

patents that would be infringed by the manufacture, sale or use of a current or future Candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our Candidates.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent or other intellectual property rights against us. For example, third parties may claim that gene or protein of interest, such as microdystrophin, or the AAV capsids we are developing for use in our Candidates are covered by patents held by them. Even if we believe such claim, or other intellectual property claims alleged by third parties, are without merit, there is no assurance that we would be successful in defending such claims. A court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially and adversely affect our ability to commercialize our Candidates covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. Similarly, there is no assurance that a court of competent jurisdiction would find that our Candidates did not infringe a third-party patent.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. If we are found, or believe there is a risk that we may be found, to infringe, misappropriate or otherwise violate a third party's intellectual property rights, and we are unsuccessful in demonstrating that such intellectual property rights are invalid or unenforceable, we could be required or may choose to obtain a license from such third party to continue developing, manufacturing and marketing our Candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing Candidate. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement, misappropriation or other violation of intellectual property rights, or claims that we have done so, could prevent us from manufacturing and commercializing our Candidates or force us to cease some or all of our business operations.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Litigation or other legal proceedings relating to intellectual property claims, with or without merit, is unpredictable and generally expensive and time-consuming. Competitors may infringe patents that we may own in the future or the patents of our licensing partners or we may be required to defend against claims of infringement. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities.

We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Accordingly, despite our efforts, we may not be able to prevent third parties from infringing or misappropriating or successfully challenging our intellectual property rights. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could have a material adverse effect on our ability to compete in the marketplace.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the USPTO and various government patent agencies outside of the United States over the lifetime of our

licensed patents and applications and any patents and patent applications we may own in the future. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable intellectual property law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could have a material adverse effect on our business.

Some intellectual property that we have in-licensed may have been discovered through government-funded programs and thus may be subject to federal regulations such as "march-in" rights, certain reporting requirements, and a preference for U.S. manufacturing. Compliance with such regulations may limit our exclusive rights, and limit our ability to contract with non-U.S. manufacturers.

Some of the intellectual property rights we have licensed, including such rights licensed from the University of Missouri, the University of Washington and the University of Florida, are stated to have been generated through the use of U.S. government funding and may therefore be subject to certain federal regulations. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future Candidates pursuant to the Bayh-Dole Act of 1980, or Bayh-Dole Act. These U.S. government rights in certain inventions developed under a government-funded program include a non-exclusive, non-transferable, irrevocable worldwide license to use inventions for any governmental purpose. In addition, the U.S. government has the right to require us to grant exclusive, partially exclusive or non-exclusive licenses to any of these inventions to a third party if it determines that: (i) adequate steps have not been taken to commercialize the invention, (ii) government action is necessary to meet public health or safety needs or (iii) government action is necessary to meet requirements for public use under federal regulations (also referred to as "march-in rights"). The U.S. government also has the right to take title to these inventions if we, or the applicable licensor, fail to disclose the invention to the government and fail to file an application to register the intellectual property within specified time limits. Intellectual property generated under a government funded program is also subject to certain reporting requirements, compliance with which may require us or the applicable licensor to expend substantial resources. In addition, the U.S. government requires that any products embodying the subject invention or produced through the use of the subject invention be manufactured substantially in the United States. The manufacturing preference requirement can be waived if the owner of the intellectual property can show that reasonable but unsuccessful efforts have been made to grant licenses on similar terms to potential licensees that would be likely to manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

We may not be able to protect our intellectual property and proprietary rights throughout the world.

Filing, prosecuting, maintaining, enforcing and defending patents on Candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. Although our license agreements grant us worldwide rights, certain of our in-licensed U.S. patents lack corresponding foreign patents or patent applications. For example, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States even in jurisdictions where we and our licensors pursue patent protection. Consequently, we and our licensors may not be able to prevent third parties from practicing our inventions in all countries outside the United States, even in jurisdictions where we and our licensors pursue patent protection, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our inventions in jurisdictions where we and our licensors have not pursued and obtained patent protection to develop their own products and may export otherwise infringing products to territories where we have patent protection, but where enforcement is not as strong as it is in the United States. These products may compete with our Candidates and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property rights, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents, if pursued and obtained, or the marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could (i) result in substantial costs and divert our efforts and attention

from other aspects of our business, (ii) put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and (iii) provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of the discovery and development processes of our Candidates or technology platforms that involve proprietary know-how, information or technology that is not covered by patents. Aspects of our manufacturing process are protected by trade secrets. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

We seek to protect our proprietary know-how, trade secrets and processes, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our employees, consultants, scientific advisors, CROs, manufacturers and contractors. These agreements typically limit the rights of third parties to use or disclose our confidential information. However, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, despite the existence generally of confidentiality agreements and other contractual restrictions. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary processes. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary know-how and trade secrets will be effective. If any of our employees, collaborators, CROs, manufacturers, consultants, advisors and other third parties who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive, and time-consuming, and the outcome is unpredictable. As a result, we could lose our trade secrets. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these security measures, they may still be breached, and we may not have adequate remedies for any breach.

In addition, our trade secrets may otherwise become known or be independently discovered by competitors. Competitors could purchase our Candidates, if approved, and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe, misappropriate or otherwise violate our intellectual property rights, design around our protected know-how and trade secrets, or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products and technologies, our competitive position could be adversely affected.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Certain of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors, as well as our academic partners. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. An inability to incorporate such technologies or features would have a material adverse effect on our business and may prevent us from successfully commercializing our Candidates. Moreover, any such litigation or the threat of such litigation may adversely affect our ability to hire employees or contract with independent contractors. A loss of key personnel or their work product could hamper or prevent our ability to commercialize our Candidates. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of

intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Moreover, individuals executing agreements with us may have preexisting or competing obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our Candidates.

Changes in either the patent laws or the interpretation of the patent laws in the United States could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. Prior to March 2013 in the United States, assuming that other requirements for patentability are met, the first to make the claimed invention was entitled to the patent, while outside the United States, the first to file a patent application was entitled to the patent. After March 2013, under the Leahy-Smith Act, the United States transitioned to a first inventor to file system in which, assuming that other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent on an invention regardless of whether a third party was the first to invent the invention. The Leahy-Smith Act also includes a number of significant changes that affect the way patent applications will be prosecuted and may also affect patent litigation. These include allowing third-party submission of prior art to the USPTO during patent prosecution and additional procedures to attack the validity of a patent through various post-grant proceedings administered by the USPTO. The USPTO developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business as, among other reasons, the USPTO must still implement various regulations. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and “gene patents” have been decided by the U.S. Supreme Court. On March 20, 2012, the U.S. Supreme Court issued a decision in *Mayo Collaborative Services v. Prometheus Laboratories, Inc.*, or Prometheus, a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the U.S. Supreme Court, the addition of well understood, routine or conventional activity such as “administering” or “determining” steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On July 3, 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the patent claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. On June 13, 2013, the U.S. Supreme Court issued its decision in Association for *Molecular Pathology v. Myriad Genetics, Inc.*, or Myriad, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent-eligible subject matter, but that complementary DNA may be patent-eligible.

In 2014, the USPTO issued a guidance to its patent examiners for evaluating claims for patent subject matter eligibility under the relevant statute (35 U.S.C. § 101). This guidance was in response to a series of decisions from the U.S. Supreme Court on patent claims reciting judicial exceptions, including Abstract Ideas, Laws of Nature/Natural Principles, Natural Phenomena and/or Natural Products. Based on judicial decisions and public feedback, several supplements to this guidance and additional memoranda and materials have since been issued and are continually being issued, while the current eligibility guidance has been incorporated into the latest (10th) edition of the MPEP (Manual for Patent Examination Procedure), last revised in June 2020. The current subject matter eligibility guideline instructs USPTO examiners to follow a two-part test, set forth in the U.S. Supreme Court decisions Alice/Mayo, as the only test that should be used to evaluate the eligibility of claims under examination, including claims directed to natural products and principles including all naturally occurring nucleic acids. Certain claims of our licensed patents and patent applications contain, and any future patents we may obtain may contain, claims that relate to specific recombinant DNA sequences that are naturally occurring at least in part and, therefore, could be the subject of future challenges made by third parties. In addition, the current USPTO subject matter eligibility guidance and the constantly evolving case law, together with contemplated congressional action, could all impact our ability to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure our stockholders that our efforts to seek patent protection for our Candidates will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the U.S. Supreme Court's decisions in Prometheus and Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could have a material adverse effect on our existing patent rights and our ability to protect and enforce our intellectual property in the future.

Moreover, although the U.S. Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter.

If we do not obtain patent term extension for patents relating to our Candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our Candidates, one or more U.S. patents that we license or may own in the future may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process based on the first regulatory approval for a particular drug or biologic. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may be able to enter the market sooner.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition and our business may be adversely affected.

We have registered trademarks with the USPTO for the marks "SOLID BIOSCIENCES", and "SOLID BIOSCIENCES" logo and registered marks in foreign jurisdictions for "SOLID BIOSCIENCES", "SOLID GT" and "SOLID BIOSCIENCES" logo. Once registered, our trademarks or trade names may be challenged, infringed, diluted, tarnished, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement, dilution or tarnishment claims brought by owners of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be adversely affected. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources.

Intellectual property rights and regulatory exclusivity rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our Candidates but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our current or future license partners or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent applications that we license or may own in the future;

- we, or our current and future license partners or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative products or duplicate any of our processes without infringing our owned or licensed intellectual property rights;
- others may circumvent our regulatory exclusivities, such as by pursuing approval of a competitive product candidate via the traditional approval pathway based on their own clinical data, rather than relying on the abbreviated pathway provided for biosimilar applicants;
- it is possible that our pending licensed patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to now or in the future may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- others may have access to the same intellectual property rights licensed to us;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know how, and a third party may subsequently file a patent covering such intellectual property.

If approved, our Candidates that are licensed and regulated as biologics may face competition from biosimilars approved through an abbreviated regulatory pathway.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Health Care Reform Law to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" based on its similarity to an approved biologic. Under the BPCIA, a reference biological product is granted 12 years of regulatory exclusivity from the time of first licensure of the product, and the FDA will not accept an application for a biosimilar or interchangeable product based on the reference biological product until four years after the date of first licensure of the reference product. In addition, the licensure of a biosimilar product may not be made effective by the FDA until 12 years from the date on which the reference product was first licensed. During this 12-year period of exclusivity, another company may still develop and receive approval of a competing biologic, so long as its BLA does not reply on the reference product, sponsor's data or submit the application as a biosimilar application.

In December 2022, Congress clarified through FDORA, that the FDA may approve multiple first interchangeable biosimilar biological products so long as the products are all approved on the same first day on which such a product is approved as interchangeable with the reference product and the exclusivity period may be shared amongst multiple first interchangeable products. More recently, in October 2023, the FDA issued its first interchangeable exclusivity determination under the BPCIA.

We believe that any of the Candidates we develop as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject Candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any one of the reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing. Nonetheless, the approval of a biosimilar to our Candidates would have a material adverse impact on our business due to increased competition and pricing pressure.

Risks Related to Ownership of our Common Stock

Our executive officers, directors and principal stockholders maintain the ability to control or significantly influence all matters submitted to our stockholders for approval.

Our executive officers and directors and principal stockholders, in the aggregate, beneficially own shares representing a significant percentage of our capital stock. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control or significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of voting power may:

- delay, defer or prevent a change in control;
- entrench our management and our Board of Directors; or
- delay or prevent a merger, consolidation, takeover or other business combination involving us on terms that other stockholders may desire.

A significant number of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is performing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that holders of a large number of shares intend to sell shares, could reduce the market price of our common stock. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended, or the Securities Act, or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates of ours. Moreover, holders of a substantial number of shares of our common stock have rights, subject to certain conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders.

In October 2020, in connection with the execution of our collaboration and license agreement with Ultragenyx, we issued and sold 521,719 shares of our common stock to Ultragenyx. For the ten-year period after date of such sale, subject to specified conditions, we have agreed to file a registration statement in order to register all or a portion of the shares sold to Ultragenyx.

In December 2024, in connection with the execution of our collaboration, patent and know-how license agreement with Mayo Foundation for Medical Education and Research, or Mayo, we issued 364,990 shares of our common stock to Mayo in a private placement. In February 2025, we made the first milestone payment of 975,496 shares of our common stock to FA212 following the FDA's clearance of our IND for SGT-212 for the treatment of FA. We have agreed to file resale registration statements in order to register all of the shares issued to Mayo and FA212.

In July 2019, December 2020 and January 2024, we completed private placements of shares of our common stock and pre-funded warrants to purchase shares of our common stock to several accredited investors. In December 2022, we also issued shares of our common stock in the Acquisition and in a related private placement to several accredited investors. We have filed registration statements covering the resale of these shares by the purchasers in these private placements, and the stock consideration issued in the Acquisition, and have agreed to keep such registration statements effective until the date the shares covered by the respective registration statement have been sold or can be resold without restriction under Rule 144 of the Securities Act.

In addition, we have filed registration statements registering all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to black-out periods and volume limitations applicable to affiliates.

We currently have on file with the SEC a universal shelf registration statement which allows us to offer and sell registered common stock, preferred stock, debt securities, depositary shares, warrants and/or units from time to time pursuant to one or more offerings at prices and terms to be determined at the time of sale.

The price of our common stock has been, and in the future is likely to be, volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

Our stock price has been, and in the future is likely to be, volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been

unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price they paid for their shares. The market price for our common stock may be influenced by many factors, including:

- our ability to successfully implement our proposed business strategy;
- results of or developments in preclinical studies and clinical trials of our Candidates or those of our competitors;
- the success of competitive products or technologies;
- the effect of public health emergencies or pandemics on both the healthcare system and the patient population;
- regulatory or legal developments in the United States, the European Union and other countries;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our Candidates, or our clinical development programs and our commercialization efforts;
- the results of our efforts to discover, develop, acquire or in-license additional Candidates;
- actual or anticipated changes in our development timelines;
- our ability to raise additional capital;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our Candidates;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of health care payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions;
- the liquidity for our stock and daily share volumes transacted;
- our ability to maintain our listing on the Nasdaq Global Select Market; and
- the other factors described in this “Risk Factors” section.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

In the past, following periods of volatility in the market price of a company’s securities, securities class-action litigation often has been instituted against that company. We and certain of our executive officers and board members have previously been named as defendants in purported class action lawsuits. Any such litigation instituted against us could cause us to incur substantial costs to defend such claims and divert management’s attention and resources.

An active trading market for our common stock may not be sustained.

Although our common stock is listed on the Nasdaq Global Select Market, given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for our stockholders to sell shares without depressing the market price for the shares, if at all.

We are a “smaller reporting company” and the reduced disclosure requirements applicable to smaller reporting companies may make our common stock less attractive to investors.

We are a smaller reporting company, and we will remain a smaller reporting company so long as the market value of our common stock held by non-affiliates is less than $250 million measured on the last business day of our second fiscal quarter,

or our annual revenues are more than $100 million during the most recently completed fiscal year and the market value of our common stock held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being permitted to provide only two years of audited financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations".

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in our filings with the SEC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

We incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to new compliance initiatives.

As a public company, we incur significant legal, accounting and other expenses. Those expenses will increase if we do not remain a smaller reporting company. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404 of the Sarbanes-Oxley Act, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain a smaller reporting company with less than $100 million in annual revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Provisions in our certificate of incorporation and our bylaws and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our Board of Directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our Board of Directors. Among other things, these provisions:

- establish a classified Board of Directors such that not all members of our board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our Board of Directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our Board of Directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;

- authorize our Board of Directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our Board of Directors; and
- require the approval of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, is the only sole source of gain for an investment in our common stock.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for an investor for the foreseeable future.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for such disputes with us or our directors, officers or employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim for breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, our certificate of incorporation or our bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. We do not intend to have this choice of forum provision apply to, and this choice of forum provision will not apply to, actions arising under the Securities Act or the Exchange Act. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

Item 1B. Unresolved Staff Comments.

None.

Item 1C. Cybersecurity

We have certain processes for assessing, identifying and managing cybersecurity risks, which are built into our information technology function and are designed to help protect our information assets and operations from internal and external cyber threats, as well as secure our networks, systems and data. Such processes include physical, procedural, and technical safeguards, employee training and incident simulations. We have the capacity to engage certain external parties, such as consultants, independent privacy assessors, computer security firms and risk management, peer companies, industry groups and governance experts, to enhance our cybersecurity oversight. We also consider the internal risk oversight programs of third-party service providers before engaging them to help protect us from any related vulnerabilities.

To help manage our material risks from cybersecurity threats and to help protect against, detect, and prepare to respond to cybersecurity incidents, we conduct periodic training for employees involved in our systems and processes that handle sensitive data. We also conduct on-boarding cybersecurity awareness assessments, cybersecurity training for all employees, and regular phishing email simulations for all employees. In addition, we use technology-based tools to help mitigate cybersecurity risks and to bolster our employee-based cybersecurity programs.

The Audit Committee of our Board of Directors provides oversight of our cybersecurity risk and provides regular updates to the Board of Directors regarding such oversight. The Audit Committee receives periodic updates from management regarding cybersecurity matters, and is notified between such updates regarding significant new cybersecurity threats or incidents.

Our Senior Director of Information Technology leads the operational oversight of company-wide cybersecurity strategy, policy, standards and processes and works across relevant departments to assess and help us prepare our employees to address cybersecurity risks. Our Senior Director of Information Technology has over seventeen years of experience in information technology, eleven years of experience in information technology for life sciences companies, and a relevant bachelor's degree in information technology. We do not believe that there are currently any known risks from cybersecurity threats that are reasonably likely to materially affect us or our business strategy, results of operations or financial condition.

Item 2. Properties.

We lease our corporate headquarters, which consists of approximately 49,869 square feet of office, laboratory, research and development and manufacturing space in Charlestown, Massachusetts. The lease for our corporate headquarters has an initial term of approximately ten years that expires in 2032 with an option to extend the lease for an additional five years. In addition, we lease smaller laboratory and office space in North Carolina.

Item 3. Legal Proceedings.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

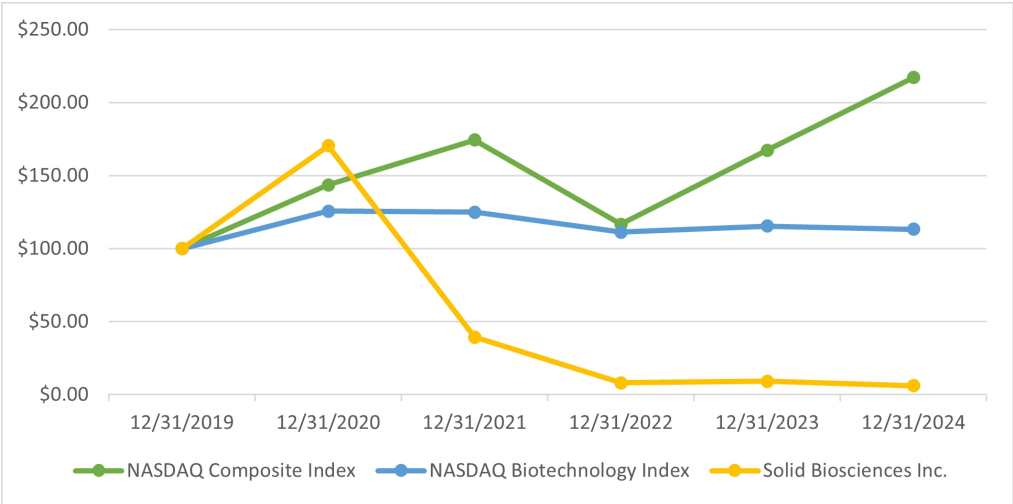
Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock has been publicly traded on the Nasdaq Global Select Market under the symbol "SLDB" since January 26, 2018 in connection with our initial public offering. Prior to that date, there was no established public trading market for our common stock.

The following graph compares the performance of our common stock to The Nasdaq Composite Index and to The Nasdaq Biotechnology Index from December 31, 2019 through December 31, 2024. The comparison assumes $100 was invested after the market closed on December 31, 2019 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any. The stock price performance included in this graph is not necessarily indicative of future stock price performance.

COMPARISON OF CUMULATIVE TOTAL RETURN
Among The Nasdaq Composite Index, The Nasdaq Biotechnology Index and Solid Biosciences Inc.



The performance graph in this Item 5 is not deemed to be "soliciting material" or to be "filed" with the SEC for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities under that Section, and shall not be deemed incorporated by reference into any of our filings under the Securities Act or the Exchange Act, except to the extent we specifically incorporate it by reference into such a filing.

Holders

As of March 3, 2025, we had approximately 38 holders of record of our common stock. This number does not include beneficial owners whose shares were held in street name. The actual number of holders of our common stock is greater than this number of record holders and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Recent Sales of Unregistered Securities

Except as set forth below, we did not sell any securities, during the year ended December 31, 2024 that were not registered under the Securities Act of 1933, as amended, or the Securities Act, and that have not otherwise been described in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K.

On December 12, 2024, we issued 364,990 shares of common stock to the Mayo Foundation for Medical Education and Research as partial consideration for the entry into the Collaboration, Patent and Know-How License Agreement between us and Mayo Foundation for Medical Education and Research. For more information about this agreement, see "Strategic Partnerships and Collaborations/License Agreements" in "Business". The shares were issued in reliance on the exemption from registration contained in Section 4(a)(2) of the Securities Act of 1933, as amended, or the Securities Act, as a privately negotiated transaction not involving any public offer or solicitation. The shares may not be offered, sold or otherwise transferred, assigned, pledged or hypothecated unless registered under the Securities Act or unless an exemption from the registration requirements of the Securities Act is available. None of the shares were sold through an underwriter and accordingly, there were no underwriting discounts or commissions involved.

Item 6. Reserved.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in or implied by these forward-looking statements.

Overview

We are a life sciences company focused on advancing a portfolio of current and future gene therapy candidates, which we refer to collectively as our Candidates, including SGT-003 for the treatment of Duchenne muscular dystrophy, or Duchenne, SGT-212 for the treatment of Friedreich's ataxia, or FA, SGT-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia, or CPVT, SGT-601 for the treatment of TNNT2-mediated dilated cardiomyopathy, or TNNT2 DCM, and additional assets for the treatment of genetic cardiac and other diseases, at different stages of development, with varying levels of investment. We are advancing our diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, our mission is to improve the daily lives of patients living with these devastating diseases.

Solid was purpose-built to advance the best science and accelerate the discovery and development of treatments that may benefit all patients with Duchenne. As we expand to bring meaningful treatments to patients living with other neuromuscular and cardiac diseases, the values and guiding principles that drive us continue. Our corporate vision is to build an innovation platform enabling the discovery and development of high-value genetic medicines for neuromuscular and cardiac diseases by integrating internal capabilities, including a vector core, use of validated animal models, optimized expression cassettes, novel capsids and regulatory expertise, and collaborations with leaders in related clinical and research fields. Our mission, which guides our operations, is to treat and change the course of neuromuscular and cardiac diseases at all stages. Underscoring this mission, our disease-focused business model is founded on the following fundamental principles:

- identify and develop meaningful therapies for underserved patients with sometimes fatal neuromuscular and cardiac diseases;
- build innovative libraries of delivery capsids and other enabling technologies with the potential to have broad impact on the gene therapy field at large:
- bring together the leading experts in neuromuscular and cardiac diseases, science, technology, disease management and care; and
- be guided by the needs of these patients.

We are continuing to advance our pipeline of Candidates. The U.S. Food and Drug Administration, or the FDA, has granted orphan drug designation, Rare Pediatric Disease designation and Fast Track designation for SGT-003 for Duchenne. The FDA has granted Fast Track Designation for SGT-212 for the treatment of FA. The FDA and the European Medicines Agency, or EMA, have granted orphan drug designation to SGT-501 for the treatment of CPVT and the FDA has granted Rare Pediatric Disease Designation for SGT-501.

SGT-003

Patient dosing in the Phase 1/2 INSPIRE DUCHENNE trial of SGT-003 began in the second quarter of 2024. The INSPIRE DUCHENNE trial is a Phase 1/2 first-in-human, open-label, single-dose, multicenter trial designed to evaluate the safety, tolerability and efficacy of SGT-003 in pediatric patients with Duchenne at a dose of 1E14vg/kg. SGT-003 is administered as a one-time intravenous infusion. In September 2024, we amended the INSPIRE DUCHENNE clinical trial protocol to increase the anticipated participant enrollment size, expand the participant cohort age groups, and extend the timepoints of certain secondary objective measurements. In connection with the expanded clinical trial, we have initiated work for additional GMP batches of SGT-003.

On February 18, 2025, we announced positive initial data from the Phase 1/2 INSPIRE DUCHENNE trial as of the data cutoff date of February 11, 2025. Interim biopsy data reported in the first three participants showed an average microdystrophin expression of 110%, as measured by western blot, and improvements in multiple biomarkers that are indicators of muscle integrity, health and resilience.

SGT-003 was well-tolerated in the first six participants dosed as of February 11, 2025. For a full description of the initial results from the INSPIRE DUCHENNE trial, see Part I, Item I, "Business" appearing in this Annual Report on Form 10-K.

Enrollment in the INSPIRE DUCHENNE trial is ongoing, with at least 10 total participants in the trial anticipated to be dosed by early in the second quarter of 2025 and approximately 20 total participants anticipated to be dosed by the fourth quarter of 2025.

INSPIRE DUCHENNE currently has a total of six active clinical sites in the United States and Canada and approved clinical trial applications in the United Kingdom and Italy. We expect to activate additional trial sites by the end of 2025. In mid-2025, we plan to request a meeting with the U.S. Food and Drug Administration, or the FDA, to discuss the potential for accelerated approval regulatory pathways for SGT-003.

SGT-212

In January 2025, we announced that the FDA cleared our investigational new drug, or IND, for SGT-212 for the treatment of FA. We anticipate initiating an open-label, multi-center Phase 1b clinical trial of SGT-212 in non-ambulatory and ambulatory adult patients living with FA in the second half of 2025. The FDA has granted Fast Track designation to SGT-212 for the treatment of FA.

SGT-501

We have conducted preclinical studies of SGT-501, including three-and six-month good laboratory practices, or GLP, toxicology studies. IND-enabling GLP toxicology studies of SGT-501 in non-human primates, including the in-life portion of the six-month toxicology, were completed in the first quarter of 2025. We expect to submit an IND to the FDA for SGT-501 for the treatment of patients with RYR2-mediated CPVT in the first half of 2025.

Other Cardiac Programs

We are currently developing a preclinical stage product candidate, SGT-601, for the treatment of TNNT2 DCM. Efficacy studies in mice suggest that SGT-601 treatment resulted in a restoration of ejection fraction function and a stabilization in cardiac function over time. We anticipate submitting an IND to the FDA for SGT-601 for the treatment of TNNT2 DCM in the second half of 2026.

Capsid

Solid's proprietary capsid used in SGT-003, AAV-SLB101, was well tolerated in the first patients dosed in the INSPIRE DUCHENNE trial as well as in NHP and mouse studies.

We are focused on developing transformative treatments to improve the lives of patients with rare neuromuscular and cardiac diseases. The majority of our current programs are designed to treat these diseases with gene transfer products. Gene transfer, a type of gene therapy, is designed to address diseases caused by mutated genes through the delivery of functional versions of those genes, called transgenes. The transgenes are then utilized by the body to produce proteins that act therapeutically to treat the condition. In addition to a transgene, our gene transfer Candidates include a viral capsid or vector (a protein shell utilized as a vehicle to deliver a transgene to cells in the body) and a promoter (a specialized DNA sequence that directs cells to produce the protein in specific tissues). The capsid is modified to no longer self-replicate yet still retain its ability to introduce new genetic material directly into patients' cells. Adeno-associated virus, or AAV, capsids have been approved for use to deliver transgenes to patients, including via systemic delivery as well as stereotactic neurosurgical administration to the brain. The use of AAV capsids to deliver gene therapies has also been extensively studied by third parties in human clinical trials for multiple disease indications, and in certain of these trials AAV was delivered systemically to the patient.

Our Operations

Due to our significant research and development expenditure, licensing and patent investment, and general and administrative costs associated with our operations, we have generated substantial operating losses in each period since our inception. Our net losses were $124.7 million and $96.0 million for the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, we had an accumulated deficit of $783.5 million. We expect to incur significant expenses and operating losses for the foreseeable future.

As we seek to develop and commercialize our Candidates, we anticipate that our expenses will increase significantly and that we will need substantial additional funding to support our continuing operations. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private equity financings, debt financings or other sources, which may include licensing agreements or strategic collaborations. We may be unable to raise additional funds or enter into such agreements or arrangements when needed on favorable terms, if at

all. If we fail to raise capital or enter into such agreements as and when needed, we may have to significantly delay, scale back or discontinue the development or commercialization of our Candidates.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or determine when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenue from product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of December 31, 2024, we had cash, cash equivalents, and available-for-sale securities of $148.9 million, excluding restricted cash of $2.0 million. In February 2025, we issued and sold in an underwritten offering, or the February 2025 Offering, 35,739,810 shares of our common stock at a price per share of $4.03, and, to certain investors in lieu of shares of common stock, pre-funded warrants to purchase 13,888,340 shares of our common stock at a price per warrant of $4.029. We received net proceeds of approximately $187.5 million, after deducting underwriting discounts and commissions and estimated offering costs. We believe that our cash, cash equivalents, and available-for-sale securities as of December 31, 2024, together with the net proceeds from the February 2025 Offering, will enable us to fund our operating expenses and capital expenditure requirements into the first half of 2027. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate.

Financial Operations Overview

Revenue

We have not generated any commercial product revenue to date and do not expect to generate any product revenue from the sale of our products for the foreseeable future, if ever. If our development efforts for our Candidates are successful and result in marketing approval, we may generate commercial product revenue in the future from product sales.

Operating Expenses

We classify our operating expenses into two categories: research and development and general and administrative expenses. Personnel costs, including salaries, benefits, bonuses, and equity-based compensation expense comprise a significant component of both expense categories. We allocate expenses associated with personnel costs based on the nature of work associated with these resources.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and clinical and preclinical development activities for our Candidates and include:

- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct research, preclinical and clinical activities on our behalf, as well as contract manufacturing organizations, or CMOs, that manufacture SGT-003, SGT-501, and other Candidates for use in our preclinical studies and clinical trials;
- salaries, benefits and other related costs, including equity-based compensation expense, for personnel engaged in research and development functions;
- costs of outside consultants, engaged to assist in our research and development activities, including their fees, equity-based compensation and related travel expenses;
- costs of laboratory supplies and acquiring, developing and manufacturing preclinical study and clinical trial materials;
- costs incurred in seeking regulatory approval of our Candidates;
- expenses incurred under our intellectual property licenses; and
- facility-related research and development expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of facilities and other operating costs.

Research and development activities are central to our business model. We are still in the early stages of development of our Candidates. Candidates in later stages of clinical development generally have higher development costs than those in preclinical development or in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will continue to increase for the foreseeable

future if and as we conduct clinical trials for SGT-003, initiate clinical trials for our other Candidates, including our planned Phase 1b clinical trial of SGT-212, and continue to identify and develop additional Candidates.

We typically use our employee and infrastructure resources across our Candidates. We track outsourced development costs and milestone payments made under our licensing arrangements by Candidate, but we do not allocate personnel costs, license payments made under our licensing arrangements or other internal costs to Candidates on a program-specific basis. These costs are included in unallocated research and development expenses in the table below.

The following table summarizes our research and development expenses by Candidate for the respective periods (in thousands):

	Year Ended December 31,	
	2024	2023
Allocated research and development expenses:		
SGT-001	$ 923	$ 3,432
SGT-003	15,197	20,856
SGT-501	17,223	3,200
SGT-212	5,435	794
Other development programs	13,344	6,645
Total allocated research and development expenses	52,122	34,927
Unallocated research and development expenses:		
Personnel related expenses	25,100	24,968
External expenses	19,209	16,668
Total unallocated research and development expenses	44,309	41,636
Total research and development expenses	$ 96,431	$ 76,563

We cannot determine with certainty the duration, costs, and timing of clinical trials of ongoing and planned SGT-003, SGT-212, SGT-501, or our other Candidates, or if, when, or to what extent we will generate revenue from the commercialization and sale of any of our Candidates for which we obtain marketing approval or our other research and development expenses. We may never succeed in obtaining marketing approval for any of our Candidates. The duration, costs, and timing of clinical trials and development of our Candidates will depend on a variety of factors, including:

- the scope, rate of progress, expense, and results of any clinical trials of our Candidates and other research and development activities that we may conduct;
- the imposition of regulatory restrictions on clinical trials, including full and partial clinical holds and the time and activities required to lift any such holds;
- uncertainties in clinical trial design and patient enrollment or drop out or discontinuation rates;
- significant and changing government regulation and regulatory guidance;
- potential additional studies or clinical trials requested by regulatory agencies;
- the timing and receipt of any marketing approvals; and
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including equity-based compensation, for personnel in our executive, finance, business development and administrative functions. General and administrative expenses also include legal fees relating to patent and corporate matters, professional fees for accounting, auditing, tax and consulting services, insurance costs, travel expenses, and facility-related expenses.

We expect that our general and administrative expenses will increase in the future as we support our research and development activities and activities related to our INSPIRE DUCHENNE trial, our planned Phase 1b clinical trial of SGT-212, and any other planned or future clinical trials for and potential commercialization of our Candidates.

Other Income, Net

Other income, net consists primarily of interest income. Interest income consists of income earned on our cash and cash equivalents, available-for-sale securities, and restricted cash.

Income Taxes

We account for income taxes using an asset and liability approach, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements but have not been reflected in taxable income. A valuation allowance is established to reduce deferred tax assets to their estimated realizable value.

We account for uncertainty in income taxes recognized in the financial statements by applying a two-step process to determine the amount of tax benefit to be recognized. First, the tax position must be evaluated to determine the likelihood that it will be sustained upon external examination by the taxing authorities. If the tax position is deemed more-likely-than-not to be sustained, the tax position is then assessed to determine the amount of benefit to recognize in the financial statements. The amount of the benefit that may be recognized is the largest amount that has a greater than 50% likelihood of being realized upon ultimate settlement. The provision for income taxes includes the effects of any resulting tax reserves, or unrecognized tax benefits, that are considered appropriate as well as the related net interest and penalties.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of our consolidated financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ materially from these estimates.

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements and that involve a significant level of estimation uncertainty.

Accrued Research and Development Expenses

As part of the process of preparing our consolidated financial statements, we are required to estimate our accrued research and development expenses. This process involves reviewing open contracts and purchase orders, communicating with our personnel to identify services that have been performed on our behalf and estimating the level of service performed and the associated costs incurred for the services when we have not yet been invoiced or otherwise notified of the actual costs. The majority of our service providers invoice us in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. We make estimates of our accrued expenses as of each balance sheet date in our consolidated financial statements based on facts and circumstances known to us at that time. Examples of estimated accrued research and development expenses include fees paid to:

- CROs in connection with performing research activities, clinical trials and preclinical studies on our behalf;
- vendors in connection with preclinical development and clinical activities;
- vendors related to product manufacturing and development and distribution of clinical and preclinical supplies; and
- third parties under our intellectual property licenses.

We base our expenses related to preclinical studies and clinical trials on our estimates of the services rendered and efforts expended pursuant to quotes and contracts with multiple CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of

services provided and result in a prepayment of the expense. In accruing fees, we estimate the time period over which services will be performed, and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and could result in us reporting amounts that are too high or too low in any particular period.

Equity-Based Compensation

We have equity plans under which we make equity awards to employees, directors and non-employees. We measure all stock options and other stock-based awards granted to employees, directors and non-employees based on the fair value on the date of the grant and recognize compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. We apply the straight-line method of expense recognition to all awards with only service-based vesting conditions. For performance-based restricted stock unit awards, which are subject to the achievement of performance milestones, the fair value is recognized as expense over the requisite service periods when the achievement of such performance milestones is determined to be probable. If a performance milestone is not determined to be probable or is not met, no equity-based compensation expense is recognized, and any previously recognized expense is reversed. For stock-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. In the first quarter of 2024, we determined that we have adequate historical stock price data to utilize solely our stock for historical volatility purposes. The expected term of stock options with service-based vesting conditions and options granted to non-employees has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. We use a 0% expected dividend yield in our determination of fair value based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future, if ever.

Derivative Liabilities

In connection with the asset purchase agreement with FA212 LLC, or FA212, certain development milestone payments to FA212 are payable in either cash, equity, or a combination of both, at our discretion. Such contingent payments were determined to be derivative liabilities.

Derivative liabilities are recorded at fair value based on the probability weighted present value of the estimated cash flows pursuant to the contractual terms of each agreement. The derivative liabilities are remeasured quarterly with changes in fair value recorded in change in fair value of derivative liabilities in the consolidated statements of operations.

Results of Operations

Comparison of the Years Ended December 31, 2024 and 2023

The following table summarizes our results of operations for the years ended December 31, 2024 and 2023 (in thousands, except percentages):

	Year Ended December 31,			
	2024	2023	Change	Change
Operating expenses:				
Research and development	$ 96,431	$ 76,563	$ 19,868	25.9%
General and administrative	33,297	27,752	5,545	20.0%
Restructuring expense	—	(63)	63	100.0%
Total operating expenses	129,728	104,252	25,476	24.4%
Loss from operations	(129,728)	(104,252)	(25,476)	24.4%
Other income, net:				
Interest income	9,469	7,582	1,887	24.9%
Interest expense	(340)	(440)	100	(22.7)%
Change in fair value of derivative liabilities	(4,750)	—	(4,750)	(100.0)%
Other income, net	652	1,095	(443)	(40.5)%
Total other income, net	5,031	8,237	(3,206)	(38.9)%
Net loss	$ (124,697)	$ (96,015)	$ (28,682)	29.9%

Research and Development Expenses

The following table summarizes our research and development expenses by Candidate for the respective periods (in thousands, except percentages):

	Year Ended December 31,			
	2024	2023	Change	Change
Allocated research and development expenses:				
SGT-001	$ 923	$ 3,432	$ (2,509)	(73.1)%
SGT-003	15,197	20,856	(5,659)	(27.1)%
SGT-501	17,223	3,200	14,023	438.2%
SGT-212	5,435	794	4,641	584.5%
Other development programs	13,344	6,645	6,699	100.8%
Total allocated research and development expenses	52,122	34,927	17,195	49.2%
Unallocated research and development expenses:				
Personnel related expenses	25,100	24,968	132	0.5%
External expenses	19,209	16,668	2,541	15.2%
Total unallocated research and development expenses	44,309	41,636	2,673	6.4%
Total research and development expenses	$ 96,431	$ 76,563	$ 19,868	25.9%

Research and development expenses for the year ended December 31, 2024 were $96.4 million, compared to $76.6 million for the year ended December 31, 2023. The increase of $19.9 million in research and development expenses was primarily related to a $14.0 million increase in costs for SGT-501 from increased manufacturing and study related costs, a $4.6 million increase in costs for SGT-212 related to the entry into the asset purchase agreement with FA212, and a $6.7 million increase in license fees and research and consulting costs for other development programs, partially offset by a $5.7 million decrease in costs for SGT-003 related to manufacturing and study related costs.

General and Administrative Expenses

General and administrative expenses were $33.3 million for the year ended December 31, 2024, compared to $27.8 million for the year ended December 31, 2023. The increase of $5.5 million was primarily related to a $4.1 million increase in personnel costs and a $1.4 million increase in legal fees.

Restructuring Expense

There was no restructuring expense during the year ended December 31, 2024, compared to $(0.1) million of restructuring expense for the year ended December 31, 2023. The restructuring expense was related to severance and other employee-related costs we recorded in the year ended December 31, 2022, in connection with a restructuring that occurred in December 2022. We paid $0.3 million and $3.7 million in severance and other employee-related costs during the years ended December 31, 2024 and 2023, respectively. See Note 16 to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K.

Other Income, Net

Other income, net was $5.0 million and $8.2 million for the years ended December 31, 2024 and 2023, respectively. The decrease of $3.2 million was primarily related to a $4.8 million expense for a change in fair value of derivative liabilities, partially offset by a $1.9 million increase in interest income primarily related to available-for-sale securities included within our portfolio.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have financed our operations primarily through the sale of redeemable preferred units and member units, the sale of securities in private placements and follow-on offerings, the sale of common stock in our initial public offering, and sales of common stock under our "at-the-market offering" sales agreement with Jefferies LLC, or Jefferies, or the ATM Sales Agreement. Through December 31, 2024, we raised an aggregate of $144.6 million of gross proceeds from our sales of preferred units prior to the completion of our initial public offering, and an aggregate of $668.9 million of net proceeds from the sale of our common stock through public offerings, including our IPO and follow-on public offering, private placements, the ATM Sales Agreement, and pursuant to the stock purchase agreements.

On March 13, 2019, we entered into the ATM Sales Agreement, which was amended and restated in March 2024, under which we may offer and sell, from time to time, shares of our common stock through Jefferies as sales agent. Any such sales being made by any method that is deemed an "at-the-market offering" as defined in Rule 415 promulgated under the Securities Act. We will pay Jefferies a commission of up to 3% of the gross proceeds of any sales of common stock pursuant to the ATM Sales Agreement. During the years ended December 31, 2024 and 2023, we sold 2,212,937 and 602,030 shares of common stock, respectively, pursuant to the ATM Sales Agreement resulting in net proceeds of $18.4 million and $3.0 million, respectively. During the three months ended December 31, 2024, we sold 1,004,650 shares pursuant to the ATM Sales Agreement resulting in net proceeds of $6.9 million.

On January 11, 2024, we issued and sold 16,973,103 shares of our common stock at a price per share of $5.53 and, to one investor in lieu of shares of common stock, pre-funded warrants to purchase 2,712,478 shares of common stock at a price of $5.529 per pre-funded warrant, in a private placement, which we refer to as the January 2024 Private Placement. We received $103.7 million of net proceeds from the January 2024 Private Placement after deducting offering costs.

As of December 31, 2024, we had cash, cash equivalents and available-for-sale securities of $148.9 million, excluding restricted cash of $2.0 million, and had no debt outstanding.

On February 19, 2025, we issued and sold in the February 2025 Offering 35,739,810 shares of our common stock at a price of $4.03 per share, and, to certain investors in lieu of shares of common stock, pre-funded warrants to purchase 13,888,340 shares of our common stock at a price of $4.029 per pre-funded warrant. We received approximately $187.5 million of net proceeds from the February 2025 Offering, after deducting underwriting discounts and commissions and estimated offering costs.

Summary of Cash Flows

The following table summarizes our sources and uses of cash for each of the periods presented (in thousands):

	Year Ended December 31,	
	2024	2023
Net cash used in operating activities	$ (100,012)	$ (94,180)
Net cash (used in) provided by investing activities	(16,086)	9,689
Net cash provided by financing activities	122,437	3,122
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 6,339	$ (81,369)

Operating Activities

During the year ended December 31, 2024, operating activities used $100.0 million of cash, primarily resulting from our net loss of $124.7 million offset by non-cash charges of $21.8 million due primarily to equity-based compensation of $10.5 million, an increase in the fair value of the derivative liabilities of $4.8 million, non-cash acquired in-process research and development of $3.4 million, depreciation expense of $2.5 million, non-cash lease expense of $2.4 million, and a non-cash up-front equity payment for a collaboration and license agreement of $2.0 million, partially offset by amortization of discount on available-for sale-securities of $3.6 million. Net cash provided by changes in our operating assets and liabilities was $2.9 million which included an increase of $4.8 million in accrued expenses and other current and non-current liabilities, and an increase in accounts payable of $2.2 million, partially offset by an increase in prepaid expenses and other current and non-current assets of $2.4 million and a decrease in operating lease liability of $1.7 million.

During the year ended December 31, 2023, operating activities used $94.2 million of cash, primarily resulting from our net loss of $96.0 million offset by non-cash charges of $10.7 million due primarily to equity-based compensation of $7.6 million and depreciation and impairment expense of $2.6 million, non-cash lease expense of $2.5 million, partially offset by amortization of discount on available-for sale-securities of $2.4 million. Net cash used by changes in our operating assets and liabilities was $8.9 million which included a decrease of $6.9 million in accrued expenses and other current and non-current liabilities, a decrease in operating lease liability of $1.7 million, and a decrease in accounts payable of $0.8 million, partially offset by a decrease in prepaid expenses and other current and non-current assets of $0.6 million.

Investing Activities

During the year ended December 31, 2024, investing activities used $16.1 million of cash, resulting from the purchases of available-for sale securities of $204.3 million and the purchases of property and equipment of $0.7 million, partially offset by the maturity of available-for sale securities of $188.9 million.

During the year ended December 31, 2023, investing activities provided cash of $9.7 million, consisting primarily of the sale of available-for-sale securities of $128.6 million, partially offset by net purchases of available-for-sale securities of $117.4 million and the purchase of property and equipment of $1.5 million primarily related to the corporate headquarters lease.

Financing Activities

During the year ended December 31, 2024, financing activities provided $122.4 million of cash, primarily resulting from the net proceeds from the sale of common stock and pre-funded warrants to purchase shares of common stock in the January 2024 Private Placement of $103.7 million and net proceeds from the sale of common stock under the ATM Sales Agreement of $18.4 million, proceeds from the exercise of common stock options of $0.5 million, and proceeds from the issuance of shares under the Company's Amended and Restated 2021 Employee Stock Purchase Plan, or ESPP, of $0.3 million, partially offset by payments of the principal portion of finance lease obligations of $0.5 million.

During the year ended December 31, 2023, financing activities provided $3.1 million of cash as a result of proceeds from the issuance of common stock of $3.0 million under the ATM Sales Agreement and from the issuance of shares of common stock of $0.1 million under the ESPP.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing development activities related to our Candidates. In addition, we have incurred and expect to continue to incur costs associated with operating as a public company. We expect that our expenses will increase substantially if and as we:

- continue to enroll patients in our INSPIRE DUCHENNE trial and advance clinical development of SGT-003;
- advance our other Candidates into clinical trials, including our planned Phase 1b clinical trial of SGT-212;
- continue research and preclinical development of our Candidates and adjacent technologies such as assays;
- seek to identify additional candidates;
- engage in regulatory interactions with the FDA and other regulatory authorities;
- submit regulatory filings relating to the development of our Candidates and seek marketing approvals for our Candidates that successfully complete clinical trials, if any;

- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- scale up our manufacturing processes and arrange manufacturing for larger quantities of our product candidates for preclinical and clinical development and potential commercialization;
- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control and scientific personnel;
- build out new facilities or expand existing facilities to support our activities;
- acquire or in-license other drugs, drug candidates, technologies and intellectual property; and
- add operational, financial and management information systems and personnel.

As of December 31, 2024, we had cash, cash equivalents and available-for-sale securities of $148.9 million, excluding restricted cash of $2.0 million. Based on our current operating plan, we believe that our cash, cash equivalents and available-for-sale securities as of December 31, 2024, together with the net proceeds from our February 2025 Offering, will be sufficient to fund our operating expenses and capital requirements into the first half of 2027. As a result, in order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate.

Due to the numerous risks and uncertainties associated with the development of our Candidates and because the extent to which we may enter collaborations with third parties for development of our Candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our Candidates. Our future capital requirements will depend on many factors, including:

- the progress, costs and results of the INSPIRE DUCHENNE trial, our planned clinical trial of SGT-212, and any future clinical trials of our Candidates;
- the costs, timing and outcome of regulatory review of our Candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for our Candidates;
- the costs associated with manufacturing and use of third-party manufacturers;
- the revenue, if any, received from commercial sale of our Candidates, should any of our Candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining, defending and enforcing our intellectual property rights and defending intellectual property-related claims;
- the outcome of any lawsuits filed against us;
- the terms of our current and any future license agreements and collaborations;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to public health emergencies or pandemics and collateral consequences related thereto.

We are supplying, and expect to continue to supply, our ongoing and future pre-clinical and clinical development programs with drug produced at a cGMP compliant facility located at one of our CMOs. We intend to establish the capability and capacity to supply Candidates at commercial scale from multiple sources.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval for any Candidates or generate revenue from the sale of any products for which we

may obtain marketing approval. In addition, our Candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if ever. Accordingly, we will need to obtain substantial additional funds to achieve our business objectives.

Adequate additional funds may not be available to us on acceptable terms, or at all. We do not currently have any committed external source of funds. To the extent that we raise additional capital through the sale of equity securities, our existing stockholders' ownership interest may be diluted. Any debt or preferred equity financing, if available, may involve agreements that include restrictive covenants that may limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, which could adversely impact our ability to conduct our business, and may require the issuance of warrants, which could potentially dilute existing stockholders' ownership interests.

If we raise additional funds through licensing agreements and strategic collaborations with third parties, we may have to relinquish valuable rights to our technology, future revenue streams, research programs, or Candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds, we may be required to delay, limit, reduce and/or terminate development of our Candidates or any future commercialization efforts or grant rights to develop and market Candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

We lease certain office space, lab space and lab equipment in Massachusetts and North Carolina. These leases are used for our continuing operations. For a description of our lease obligations, refer to Note 10 to our consolidated financial statements appearing in this Annual Report on Form 10-K.

Under various agreements with third-party licensors, we have agreed to make milestone payments and pay royalties to third parties based on specified milestones. For a description of our license agreements, see Part I, Item 1, "Business—Strategic Partnerships and Collaborations/Licenses" and see Note 12 of our consolidated financial statements appearing in this Annual Report on Form 10-K.

We enter into contracts in the normal course of business with CROs and CMOs for clinical trials, preclinical research studies and testing, manufacturing and other services and products for operating purposes. These contracts are cancelable by us upon prior notice of 30 days.

Recently Issued Accounting Pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K, such standards will not have a material impact on our consolidated financial statements or do not otherwise apply to our operations.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2024, our cash equivalents consisted of money market accounts and treasury bills that have contractual maturities of less than 90 days from the date of acquisition. As of December 31, 2024, our investments consisted of treasury bills and government bonds that have contractual maturities of less than one year. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

Inflation Risk

Inflation generally affects us by increasing our cost of labor and research, manufacturing and development costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations in the last two years. However, our operations may be adversely affected by the inflation in the future.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 is included at the end of this Annual Report on Form 10-K beginning on page F-1.

Item 9. Changes in and Disagreements With Accountants on Accounting and Financial Disclosure.

Not applicable.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer (our principal executive officer and principal financial and accounting officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2024. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to a company's management, including its principal executive and principal financial and accounting officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Based on the evaluation of our disclosure controls and procedures, our Chief Executive Officer and Chief Financial Officer concluded that, as of December 31, 2024, our disclosure controls and procedures were designed and operating effectively.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2024 based on the framework in *Internal Control-Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. Based on that evaluation, management has concluded that the Company's internal control over financial reporting was effective as of December 31, 2024.

As a non-accelerated filer and a “smaller reporting company”, as defined in Rule 12b-2 under the Exchange Act, our independent registered public accounting firm is not required to issue an attestation report on our internal control over financial reporting.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the three months ended December 31, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

(b) Director and Officer Trading Arrangements

None of our directors or officers adopted or terminated a Rule 10b5-1 trading arrangement or a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K) during the fourth quarter of 2024.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Set forth below are the names, ages and positions of our current executive officers and directors as of March 6, 2025.

Name	Age	Position(s) held
Executive Officers		
Alexander Cumbo	54	President, Chief Executive Officer and Director
Gabriel Brooks, M.D.	48	Chief Medical Officer
Jessie Hanrahan, Ph.D.	49	Chief Regulatory Officer
Paul Herzich	47	Chief Technology Officer
David Tyronne Howton	53	Chief Operating Officer and Secretary
Kevin Tan	47	Chief Financial Officer and Treasurer
Non-Employee Directors		
Ian F. Smith	59	Executive Chairman of the Board
Martin Freed, M.D.	64	Director
Ilan Ganot	51	Director
Clare Kahn, Ph.D.	73	Director
Georgia Keresty, Ph.D.	63	Director
Sukumar Nagendran, M.D.	59	Director
Adam Stone	45	Director
Lynne Sullivan	58	Director

Executive Officers

Alexander Cumbo has served as our President and Chief Executive Officer and as a director since December 2022. Prior to that, Mr. Cumbo served as the President and Chief Executive Officer and as a director of AavantiBio, a gene therapy company, from October 2020 to December 2022, when we acquired AavantiBio. From January 2013 to October 2020, Mr. Cumbo held positions of increasing responsibility at Sarepta Therapeutics, Inc., or Sarepta, a precision genetic medicine company, ultimately serving as Executive Vice President, Chief Commercial Officer. From 2011 to 2013, Mr. Cumbo served as Vice President of Sales and Treatment Education for Vertex Pharmaceuticals Incorporated, or Vertex, a biotechnology company, launching Incivek, a treatment for hepatitis C, and from 2010 to 2011, he served as area director for Vertex. Prior to Vertex, Mr. Cumbo served in multiple commercial roles supporting the HIV, HBV and cardiovascular franchises at Gilead Sciences, Inc., a biopharmaceutical company. Mr. Cumbo has served on the Board of Directors of Verve Therapeutics, Inc. since June 2022. Mr. Cumbo previously served on the Board of Directors of RA Pharmaceuticals, Inc., a clinical stage biopharmaceutical company acquired by UCB, Brussels, from November 2018 to April 2020. Mr. Cumbo received a Bachelor of Science in Laboratory Technology from Auburn University. Mr. Cumbo is qualified to serve on our Board of Directors based on his extensive and broad range of experience in the biopharmaceutical industry and his deep knowledge of our company as our Chief Executive Officer.

Gabriel Brooks, M.D. has served as our Chief Medical Officer since October 2023. Prior to that, Dr. Brooks served as Executive Director of Pfizer Inc., or Pfizer, a pharmaceutical company, from September 2020 to October 2023. Prior to joining Pfizer, Dr. Brooks served as Vice President of Research and Development for 4D Molecular Therapeutics, Inc., a biopharmaceutical company, from November 2018 to September 2020. Prior to joining 4D Molecular Therapeutics, Inc., Dr. Brooks served as Medical Director for GE HealthCare Technologies Inc., or GE Healthcare, a medical technology company. Prior to joining GE HealthCare, Dr. Brooks served as Associate Director of Clinical Research at Gilead Life Sciences, Inc., a biopharmaceutical company, from August 2015 to April 2017. Dr. Brooks received a Bachelor of Science in Biology from Carnegie Mellon University, a Doctor of Medicine from Cornell University, and a Master of Applied Sciences of Biostatistics and Epidemiology from the University of California, San Francisco. He completed his training in Internal Medicine at Johns Hopkins Hospital, Baltimore, and fellowships in general Cardiology and Advanced Imaging at the University of California, San Francisco.

Jessie Hanrahan, Ph.D. has served as our Chief Regulatory Officer since December 2022. Prior to that, Dr. Hanrahan served as Chief Regulatory Officer of AavantiBio from May 2021 to December 2022. Prior to joining AavantiBio, Dr. Hanrahan served as the Vice President of Regulatory Science at bluebird bio, Inc., or bluebird, a biotechnology company, from February 2020 to May 2021 and Senior Director of Regulatory Science at bluebird from August 2016 to February 2020. Prior to joining bluebird, Dr. Hanrahan worked at Sanofi Genzyme, a biotechnology company, as Senior Manager of Regulatory Affairs from October 2009 to July 2016 and as Principal Medical Writer from July 2007 to October 2009. Prior to joining Sanofi Genzyme, Dr. Hanrahan served as a Medical Writer at Boston Scientific, a biomedical engineering firm, from May

2006 to July 2007. Dr. Hanrahan holds a Ph.D., M.S. and M.Ph. in Molecular, Cellular and Developmental Biology from Yale University. Dr. Hanrahan received a Bachelor of Arts in Biology and History from Mount Holyoke College.

Paul Herzich has served as our Chief Technology Officer since December 2022. Prior to that, Mr. Herzich served as Chief Technology Officer of AavantiBio from April 2021 to December 2022. Before joining AavantiBio, Mr. Herzich served as Vice President of CMC at BridgeBio Pharma, Inc., or BridgeBio, a biopharmaceutical company, from August 2020 to April 2021. Previously, Mr. Herzich served as Head of Manufacturing Operations at BridgeBio from July 2019 to July 2020. Before joining BridgeBio, Mr. Herzich served as Senior Director of Manufacturing at LogicBio Therapeutics, Inc., a genetic medicine company, from January 2018 to July 2019. Additionally, Mr. Herzich served as Senior Manager and Director of cGMP Gene Therapy Manufacturing at Pfizer from August 2016 to January 2018. From July 2015 to August 2016, Mr. Herzich served as the head of TD Manufacturing at CSL Seqirus, a pharmaceutical company, after its acquisition of Novartis Vaccines and Diagnostics, Inc., where Mr. Herzich served in various roles of increasing responsibility from December 2007 to July 2015. Mr. Herzich holds a Master of Business Administration from North Carolina State University Poole College of Management and a Bachelor of Science in Biology from Rutgers University.

David Tyronne Howton has served as our Chief Operating Officer since September 2023 and as our Secretary since December 2022. Mr. Howton previously served as our Chief Administrative Officer from December 2022 to September 2023 and as our General Counsel from December 2022 to July 2024. Prior to that, Mr. Howton served as the Chief Operating Officer and General Counsel of AavantiBio from March 2021 to December 2022. From November 2012 to December 2020, Mr. Howton served as Executive Vice President, General Counsel and Corporate Secretary at Sarepta. Prior to joining Sarepta, Mr. Howton served as the Senior Vice President and Chief Legal Officer and Chief Compliance Officer at Vertex. Prior to Vertex, Mr. Howton served in multiple legal roles at Genentech, Inc., a biotechnology company. Mr. Howton has served on the board of Make-A-Wish® Massachusetts and Rhode Island since March 2021. Mr. Howton received a Bachelor of Arts in Political Science from Yale University and a Juris Doctor from Northwestern University School of Law.

Kevin Tan has served as our Chief Financial Officer and Treasurer since January 2023. Prior to that, Mr. Tan served as the Chief Financial Officer at Selecta Biosciences, Inc., or Selecta, a biopharmaceutical company, from September 2021 to November 2022. Prior to joining Selecta, Mr. Tan served as Treasurer at Sarepta from July 2020 to September 2021. Prior to becoming Treasurer at Sarepta, he served as Assistant Treasurer from May 2018 to June 2020. Before joining Sarepta, Mr. Tan worked as a freelance consultant from February 2017 to April 2018, providing independent financial advice and advisory services to individuals and private companies. From June 2012 to November 2016, Mr. Tan served as Senior Portfolio Manager — Public Market Investments at CPP Investments (f/k/a the Canada Pension Plan Investment Board). He has also served in various positions at Macquarie Capital (USA) Inc., Arrowhawk Capital Partners LLC, Lehman Brothers Inc. (subsequently acquired by Barclays Capital Inc.) and UBS Investment Bank. Mr. Tan holds a Bachelor of Commerce degree from Queen's University at Kingston, a Master of Engineering degree from The Graduate School at Princeton University, and a Master of Business Administration degree from the University of Chicago Booth School of Business. Mr. Tan is a Chartered Financial Analyst (CFA) charterholder.

Non-Employee Directors

Ian F. Smith is Executive Chairman of our Board of Directors and has served as a member of our Board of Directors since April 2020 and served as a consultant to us from February 2020 to December 2021. Mr. Smith is also the chairman of the Board of Directors of Rivus Pharmaceuticals, Inc. and iVexSol, Inc, and a member of the Board of Directors of Stoke Therapeutics, Inc., Foghorn Therapeutics Inc., Odyssey Therapeutics, Inc. and Alkeus Pharmaceuticals, Inc., which are all biotechnology companies. He is also a Senior Advisor to Bain Capital Life Sciences. Between 2001 and 2019, Mr. Smith served as Chief Financial Officer and then Chief Operating Officer at Vertex. He received a B.A. with honors in accounting and finance from Manchester Metropolitan University (UK). Mr. Smith is qualified to serve on our Board of Directors because of his more than 25 years of finance and broad operating experience for public companies in the biopharmaceutical industry.

Martin Freed, M.D., F.A.C.P. has served as a member of our Board of Directors since June 2018. Dr. Freed has served as an independent consultant to several private pharmaceutical, biotechnology, and healthcare companies, specializing in clinical and general pharmaceutical development and clinical and regulatory strategy since February 2015. He co-founded and served as Chief Medical Officer of Civitas Therapeutics, Inc., a biopharmaceutical company acquired by Acorda Therapeutics, Inc., from December 2010 to October 2014, and as Senior Vice President, Clinical Development of Acorda Therapeutics, Inc. from October 2014 to January 2015. He has also served as Chief Medical Officer at Adnexus Therapeutics, Inc. (acquired by Bristol-Myers Squibb) and Vitae Pharmaceuticals, Inc. Dr. Freed spent nearly 14 years at GlaxoSmithKline and its predecessor, SmithKline Beecham Pharmaceuticals or SmithKline Beecham, where he served numerous roles including vice president, clinical development and medical affairs in the metabolism therapeutic area. Dr. Freed currently serves on the Board of Directors for Avilar Therapeutics, Inc. He previously served on the Board of Directors for Sojournix, Inc., Dicerna

Pharmaceuticals and Intekrin Therapeutics. Dr. Freed has been Board Certified in Internal Medicine, Nephrology and Clinical Pharmacology. He performed his internal medicine residency at Temple University Hospital and nephrology fellowship at Yale-New Haven Hospital. A Fellow of the American College of Physicians, Dr. Freed received a B.S. with distinction in biology from the University of Delaware and an M.D. from Pennsylvania State University's College of Medicine. Dr. Freed is qualified to serve on our Board of Directors because of his extensive leadership experience, his public company board experience and his experience working in the healthcare sector.

Ilan Ganot is one of our founders and has served as a member of our Board of Directors since our inception in 2013. He also served as our Chief Executive Officer from 2013 to December 2022 and as our President from June 2018 to December 2022. From December 2022 to December 2023, Mr. Ganot assisted with the transition of his duties to Mr. Cumbo and provided other consulting and advisory services, as requested from time to time by us. Mr. Ganot is currently the Chief Executive Officer and a member of the Board of Alesta Therapeutics, a biotechnology company focused on the development of innovative therapies for rare diseases. Mr. Ganot is also a member of the Board of Directors of Minovia Therapeutics, a biotechnology company. Previously, Mr. Ganot served as an investment banker at JPMorgan Chase & Co., Nomura Securities Co., Ltd., and Lehman Brothers. Mr. Ganot received his MBA from London Business School and holds law and business degrees from Reichman University in Herzliya, Israel. Mr. Ganot also practiced corporate law in Israel and was a Captain in the Israeli Defense Forces. He is qualified to serve on our Board of Directors because of his personal dedication to improving treatments available for Duchenne patients, his extensive leadership experience and his extensive knowledge of our company based on his previous role as our Chief Executive Officer.

Clare Kahn, Ph.D. has served as a member of our Board of Directors since March 2021 and has served as a consultant to us since June 2022. Dr. Kahn has served as R&D Strategy Officer at X-VAX Technology Inc., or X-VAX, a biotechnology company developing vaccines against pathogens acquired by mucosal infection such as herpes, since September 2019. She served as Chief Regulatory and Preclinical Development Officer at X-VAX from September 2018 to September 2019. Dr. Kahn has also been the president of Clare Kahn Pharma Consulting LLC, through which she provides consulting services on regulatory strategy since July 2016. Dr. Kahn was previously Vice President, Worldwide Regulatory Strategy, Global Innovative Pharma at Pfizer from January 2014 to June 2016 and Vice President, Worldwide Regulatory Strategy, Specialty Care Business at Pfizer from June 2010 to December 2013. Prior to Pfizer, she was Vice President of Regulatory Affairs for a variety of therapeutic areas including cardiovascular, metabolic, urology, oncology and vaccines at GlaxoSmithKline from 1999 to 2010. Dr. Kahn has a Ph.D. in Biochemical Pharmacology from The Royal Postgraduate Medical School, London and served as Assistant Professor of Pharmacology and of Pathology and Laboratory Medicine at The University of Pennsylvania from 1981 to 1985. Dr. Kahn is qualified to serve on our Board of Directors because of her extensive leadership experience and her experience working in the healthcare sector.

Georgia Keresty, Ph.D., M.PH. has served as a member of our Board of Directors since March 2021. Dr. Keresty served as the Chief Operating Officer at Volnay Therapeutics, a biotechnology start-up company, from March 2023 to November 2024. Prior to this role, Dr. Keresty served as a senior advisor to Takeda R&D in 2021 and was their R&D Chief Operating Officer from 2017 to 2020. From 2003 to 2017 and from 1997 to 1999, she was an executive at Johnson & Johnson. From 1999 to 2003 and from 1983 to 1997, she held roles at Bristol-Myers Squibb Company and Novartis Pharmaceuticals Corporation or Novartis, respectively. Dr. Keresty holds BSc degrees in Chemical Engineering and Computer Science from Clarkson University and Ramapo College of New Jersey, an M.S. degree in Information Systems from Pace University, an MBA in Operations Management from Rutgers University, a Ph.D. in Operations Management from Rutgers University, and an MPH in Global Health Leadership from the University of Southern California. Dr. Keresty currently serves on the Board of Directors of Intellia Therapeutics, Inc. Dr. Keresty previously served on the Board of Directors of Aspen Technology, Inc. and Commissioning Agents, Inc. Dr. Keresty is also an NACD Board Leadership Fellow and Directorship Certified® since September 2020 and earned an NACD CERT Certificate in Cyber-Risk Oversight in January 2024. Dr. Keresty is qualified to serve on our Board of Directors because of her extensive leadership and governance experience, and her experience working in the healthcare sector.

Sukumar Nagendran, M.D. has served as a member of our Board of Directors since September 2018. Since December 2022, Dr. Nagendran has served as President, Head of Research and Development at Taysha Gene Therapies, Inc., a gene therapy company, and he has served as a member of their Board of Directors since July 2020. Prior to that, he was the Chief Medical Officer and President of Research and Development at Jaguar Gene Therapy, or Jaguar, a biotechnology company, from February 2020 to December 2022. Prior to Jaguar, Dr. Nagendran was Chief Medical Officer and Senior Vice President of AveXis Inc., or AveXis, a clinical-stage gene therapy company, from September 2015 to July 2018, prior to the company's acquisition by Novartis. Prior to AveXis, Dr. Nagendran was Vice President of Medical Affairs of Quest Diagnostics, a provider of diagnostic information services, from March 2013 to September 2015. Prior to Quest Diagnostics, Dr. Nagendran held key leadership positions at Pfizer, Novartis, Daiichi Sankyo, and Reata Pharmaceuticals. Prior to moving to the biotech industry, Dr. Nagendran practiced internal medicine, with a focus on diabetes and cardiovascular disease. He is a Mayo Alumni Laureate and founding member of the Robert Wood Johnson Legacy Society. He is also the sponsor for the

Fonseca-Nagendran Scholar award at the American Diabetes Association to enhance research in minority populations. Dr. Nagendran currently serves on the Board of Directors of SalioGen Therapeutics. Dr. Nagendran received his undergraduate degree in Biochemistry from Rutgers University and his M.D. from Rutgers Medical School and trained in Internal Medicine at Mayo Clinic, Rochester. Dr. Nagendran is qualified to serve on our Board of Directors because of his extensive leadership experience and his experience working in the healthcare sector

Adam Stone has served as a member of our Board of Directors since November 2015. Mr. Stone is currently the Chief Investment Officer of Perceptive Advisors, a life sciences focused investing firm, where he has worked since 2006. Since July 2021 and February 2021, respectively, Mr. Stone has served as a director and Chief Executive Officer of ARYA Sciences Acquisition Corp V and ARYA Sciences Acquisition Corp IV. Mr. Stone is currently a member of the Board of Directors of Immatics N.V., a public biopharmaceutical company, and LianBio and Xontogency LLC, both private companies, and he previously served as a member of the Board of Directors of Renovia Inc. and Prometheus Biosciences, Inc. Mr. Stone received a B.A. in molecular biology from Princeton University. Mr. Stone is qualified to serve on our Board of Directors because of his extensive experience developing early-stage biotech and healthcare companies.

Lynne Sullivan has served as a member of our Board of Directors since November 2015. Ms. Sullivan has served as the Chief Financial Officer for UNITY Biotechnology, Inc., a biotechnology company, since August 2020. Prior to that, Ms. Sullivan served as the Chief Financial Officer for Compass Therapeutics, LLC, or Compass, a biotechnology company, from December 2018 to August 2019. Prior to Compass, Ms. Sullivan served as Biogen Inc.'s senior vice president of Finance from 2016 to December 2018, where she also served as vice president of Tax and Corporate Finance from February 2015 to March 2016 and vice president of Tax from April 2008 to February 2015. Ms. Sullivan is currently a member of the Board of Directors of Inozyme Pharma, Inc., a public biopharmaceutical company. She previously served as a member of the Board of Directors of BiomX Inc. She received an M.S. in Taxation from Bentley University and a B.S.B.A. from Suffolk University. Ms. Sullivan was a Certified Public Accountant for over 20 years. Ms. Sullivan is qualified to serve on our Board of Directors because of her extensive experience in public accounting and financial expertise and her experience working in the healthcare sector.

Composition of the Board of Directors

Our board of directors currently consists of nine members divided into three classes, with members of each class holding office for staggered three-year terms. There are currently two Class I directors (Alexander Cumbo and Sukumar Nagendran), whose terms expire at the 2025 annual meeting of stockholders; three Class II directors (Clare Kahn, Adam Stone and Lynne Sullivan), whose terms expire at the 2026 annual meeting of stockholders; and four Class III directors (Martin Freed, Ilan Ganot, Georgia Keresty and Ian Smith) whose terms expire at the 2027 annual meeting of stockholders (in all cases subject to the election and qualification of their successors or to their earlier death, resignation or removal).

Delinquent Section 16(a) Reports

Section 16(a) of the Exchange Act requires our directors and executive officers and holders of more than 10% of our common stock to file with the SEC initial reports of ownership and reports of changes in ownership of our common stock and other equity securities.

Based solely on a review of our records and representations made by the persons required to file these reports, we believe all directors, executive officers, and 10% owners timely filed all reports regarding transactions in our securities required to be filed by Section 16(a) under the Exchange Act for the fiscal year ended December 31, 2024, with the exception of a Form 4 filed by Adam Stone on November 19, 2024 to report the grant of stock options on June 11, 2024.

Audit Committee

The members of our audit committee are Ms. Sullivan, Dr. Keresty and Mr. Stone, with Ms. Sullivan serving as chair of the audit committee. Our Board of Directors has determined that each of these individuals meets the independence requirements of the Sarbanes-Oxley Act, Rule 10A-3 under the Exchange Act, and the applicable listing standards of Nasdaq. Each member of our audit committee can read and understand fundamental financial statements in accordance with Nasdaq audit committee requirements. In arriving at this determination, the Board of Directors has examined each audit committee member’s employment and other experience. Our Board of Directors has determined that Ms. Sullivan qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the Nasdaq listing rules. In making this determination, our Board of Directors has considered Ms. Sullivan’s formal education and previous and current experience in financial roles. Both our independent registered public accounting firm and management periodically meet privately with our audit committee. Our audit committee met four times during fiscal year 2024.

Our audit committee's responsibilities include, among other things:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from that firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and Code of Conduct;
- overseeing our risk assessment and risk management policies;
- overseeing cybersecurity risks;
- establishing procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management;
- reviewing and approving or ratifying any related person transactions;
- reviewing on a periodic basis our investment policy; and
- preparing the audit committee report required by SEC rules.

We believe that the composition and functioning of our audit committee complies with all applicable requirements of the Sarbanes-Oxley Act and all applicable SEC and Nasdaq rules and regulations.

Code of Conduct

We have adopted a Code of Business Conduct and Ethics, or the Code of Conduct, that applies to our directors, executive officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. The Code of Conduct is available on the "Governance & Financials" section of the "Investors & Media" page of our website www.solidbio.com. Our Board of Directors and the audit committee of our Board of Directors are responsible for overseeing the Code of Conduct. We intend to post on our website all disclosures that are required by law or the listing standards of Nasdaq concerning any amendments to, or waivers of, any provision of the Code of Conduct.

Insider Trading Policy

We have adopted an Insider Trading Policy governing the purchase, sale and/or other dispositions of our securities by our directors, officers, employees and other covered persons. We believe the Insider Trading Policy is reasonably designed to promote compliance with insider trading laws, rules and regulations, and Nasdaq listing standards. A copy of our Insider Trading Policy is filed as Exhibit 19.1 to this Annual Report on Form 10-K for the fiscal year ended December 31, 2024. We do not engage in transactions in our securities while in possession of material nonpublic information concerning the company or our securities.

Item 11. Executive Compensation.

Compensation of our Named Executive Officers

The following information describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers, or the Named Executive Officers. Our Named Executive Officers for the year ended December 31, 2024 are:

- Alexander Cumbo, our President and Chief Executive Officer;
- Gabriel Brooks, our Chief Medical Officer; and
- David Tyronne Howton, our Chief Operating Officer and Secretary.

2024 Summary Compensation Table

The following table contains information about the compensation paid to or earned by each of our Named Executive Officers for the years ended December 31, 2024 and 2023.

Name and Principal Position	Year	Salary ($)	Bonus ($) (1)	Stock Awards ($) (2)	Option Awards ($) (3)	All Other Compensation ($) (4)	Total ($)
Alexander Cumbo, *President and Chief Executive Officer*	2024	608,400	334,620	2,215,183	1,699,286	12,420	4,869,909
	2023	585,000	321,750	—	—	11,880	918,630
Gabriel Brooks, M.D., *Chief Medical Officer*	2024	468,000	187,200	785,378	618,887	11,907	2,071,372
David Tyronne Howton, *Chief Operating Officer and Secretary*	2024	473,200	189,280	1,057,038	831,076	12,420	2,563,014
	2023	455,000	182,000	—	—	11,880	648,880

(1) Except where noted otherwise, represents annual bonuses earned in the calendar year and paid to the Named Executive Officers after the completion of each calendar year at the discretion of our Board of Directors.

(2) The amounts in this column represent the aggregate grant date fair value of the performance-based restricted stock unit awards, or PSUs, and restricted stock units, or RSUs, granted to the Named Executive Officers in the respective calendar years as computed in accordance with the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 718. The assumptions used in calculating the grant date fair value of the PSUs and RSUs are set forth in Note 9 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K. The grant date fair value of the PSUs granted in 2024 included in this column reflects the value of the award based on the probable outcomes of performance conditions. The grant date fair value assuming the highest level of performance conditions being achieved for these PSUs is $5,088,807, $1,767,764, and $2,383,403 for Mr. Cumbo, Dr. Brooks, and Mr. Howton, respectively.

(3) The amounts in this column represent the aggregate grant date fair value of the options granted to the Named Executive Officers in the respective calendars years as computed in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value of the options are set forth in Note 9 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

(4) Reflects a matching contribution under our 401(k) plan.

Narrative Disclosure to Summary Compensation Table

Base Salary. We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our Named Executive Officers. None of our Named Executive Officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary.

In 2024, the annualized base salaries of our Named Executive Officers were as follows: $608,400 for Mr. Cumbo, $468,000 for Dr. Brooks and $473,200 for Mr. Howton. For 2025, our Board of Directors increased the base salary amount for Mr. Cumbo to $650,900, for Dr. Brooks to $502,100 and for Mr. Howton to $513,400.

Annual Bonus. Our Board of Directors may, in its discretion, award bonuses to our Named Executive Officers from time to time. Our employment agreements with our Named Executive Officers provide that they will be eligible for annual performance-based bonuses up to a specified percentage of their salary, subject to approval by our Board of Directors. Performance-based bonuses, which are calculated as a percentage of base salary, are designed to motivate our employees to achieve annual goals based on our strategic, financial and operating performance objectives. From time to time, our Board of Directors has approved discretionary annual cash bonuses to our Named Executive Officers with respect to their prior year performance.

With respect to 2024, our Board of Directors awarded a discretionary bonus of $334,620 to Mr. Cumbo, $187,200 to Dr. Brooks and $189,280 to Mr. Howton, which in each case equaled 100% of their respective target bonuses for 2024.

With respect to fiscal year 2025 performance, Mr. Cumbo, Dr. Brooks and Mr. Howton are eligible for annual discretionary bonuses of up to 60%, 45% and 45% of their 2025 annual base salary, respectively.

Equity Incentives. Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incentivizes our executive officers to remain in our employment during the vesting period. Accordingly, our Board of Directors and compensation committee periodically review the equity incentive compensation of our Named Executive Officers and from time to time may grant equity incentive awards to them in the form of stock options or RSUs with time-based and/or performance-based vesting conditions.

In February 2024, we granted options to purchase 240,250, 87,500 and 117,500 shares of our common stock to Mr. Cumbo, Dr. Brooks and Mr. Howton, respectively, and RSUs with respect to 120,125, 43,750 and 58,750 shares of our common stock to Mr. Cumbo, Dr. Brooks and Mr. Howton, respectively. The options vest with respect to 25% of the shares underlying the options on the first anniversary of the grant date and as to an additional 2.0833% of the original number of shares underlying the option monthly thereafter through the fourth anniversary of the grant date. The RSUs vest in equal annual installments over a term of four years from the date of grant.

In June 2024, we granted PSUs with respect to 677,604, 235,389, and 317,365 shares of our common stock to Mr. Cumbo, Dr. Brooks and Mr. Howton, respectively. The PSUs provide for the vesting of 25% of the target number of underlying units granted upon the achievement of each of four independent performance milestones predetermined by the Board of Directors, or the Performance Milestones, subject to continued service with us, or the Approval Conditions.

The Performance Milestones are tied to the achievement of certain business objectives and are non-market and non-financial in nature. The Board of Directors will determine that all Approval Conditions have been satisfied and the number of units that will ultimately vest on the 2026 Evaluation Date, which will occur in the first quarter of 2026, and the 2027 Evaluation Date, which will occur in the first quarter of 2027. A maximum of 25% of the target number of PSUs may vest at the 2026 Evaluation Date and the percentage of the target number of PSUs allocable to any Performance Milestone that has not been achieved on or prior to the 2027 Evaluation Date shall be cancelled.

In January 2025, we granted RSUs with respect to 401,600, 112,900, and 169,100 shares of our common stock to Mr. Cumbo, Mr. Brooks, and Mr. Howton respectively. The RSUs vest in equal annual installments over four years from the date of grant.

Our employees and executives are eligible to receive stock options and other stock-based awards pursuant to our Amended and Restated 2020 Equity Incentive Plan, or the 2020 Plan. We use equity awards to compensate our executive officers in the form of initial grants in connection with the commencement of employment and also at various times, often but not necessarily annually. None of our executive officers is currently party to an employment agreement that provides for an automatic award of stock options, RSUs or PSUs. We have granted stock options and RSUs to our executive officers with time-based vesting. The options that we have granted to our executive officers typically become exercisable as to 25% of the shares underlying the option on the first anniversary of the grant date and as to an additional 2.0833% of the original number of shares underlying the option monthly thereafter through the fourth anniversary of the grant date. The RSUs that we have granted to our executive officers typically become exercisable as to 25% of the shares underlying the RSU on the first anniversary of the grant date and as to an additional 25% of the original number of shares underlying the RSU annually thereafter. In 2024, we also granted to our executive officers the PSUs as described above. Vesting rights cease upon termination of employment and exercise rights cease shortly after termination, except that vesting of certain equity awards is fully accelerated upon certain terminations in connection with a change of control and exercisability is extended in the case of death or disability. Prior to the exercise of an option or the vesting of an RSU or PSU, the holder has no rights as a stockholder with respect to the shares subject to such option or with respect to the RSUs or PSUs, including no voting rights and no right to receive dividends or dividend equivalents.

The exercise price of all stock options granted after the closing of our initial public offering is equal to the fair market value of shares of our common stock on the date of grant, which is determined by reference to the closing market price of our common stock on The Nasdaq Global Select Market on the date of grant.

Employment and Other Agreements

We have entered into employment agreements with each of our executive officers. The employment agreements set forth the terms of the executive officers' compensation, including their base salary and annual performance bonus opportunity. In addition, the employment agreements provide that, subject to eligibility requirements under the plan documents governing such programs and our policies, the executive officers are entitled, on the same basis as our other employees, to participate in

and receive benefits under, any medical, vision and dental insurance policy maintained by us and we will pay, consistent with our then-current employee benefit policy, a portion of the cost of the premiums for any such insurance policy in which the executive officers elect to participate. Each executive officer is also eligible to receive paid vacation time, sick time, and Company holidays consistent with our policies as then in effect from time to time and equity awards at such times and on such terms and conditions as the Board of Directors may determine. Each executive officer's employment is at will. The employment agreements with Mr. Cumbo, Dr. Brooks and Mr. Howton are summarized below.

On September 29, 2022, we entered into an employment agreement with each of Mr. Cumbo and Mr. Howton pursuant to which, effective as of December 2, 2022, Mr. Cumbo commenced employment as our President and Chief Executive Officer and Mr. Howton commenced employment as our Chief Administrative Officer, General Counsel and Secretary. Mr. Howton now serves as our Chief Operating Officer, and Secretary. On October 2, 2023, we entered into an employment agreement with Dr. Brooks, pursuant to which Dr. Brooks commenced employment as our Chief Medical Officer effective as of October 2, 2023.

Pursuant to their respective employment agreements, Mr. Cumbo was initially entitled to an annual base salary of $585,000, Dr. Brooks was initially entitled to an annual base salary of $450,000 and Mr. Howton was initially entitled to an annual base salary of $455,000. Each of their base salaries for 2024 and 2025 is set forth above under "—Narrative Disclosure to Summary Compensation Table". Each of their base salaries will be reviewed by our Board of Directors from time to time and is subject to change in the discretion of our Board of Directors.

Mr. Cumbo, Dr. Brooks and Mr. Howton are also eligible to earn an annual performance bonus, with a target bonus amount equal to a specified percentage of their annual base salary, based upon our Board of Directors' assessment of their performance and our attainment of targeted goals as set by our Board of Directors in their sole discretion. The bonus may be in the form of cash, equity award(s), or a combination of cash and equity award(s). Pursuant to their employment agreements, Mr. Cumbo, Dr. Brooks and Mr. Howton are eligible for annual discretionary bonuses of up to 55%, 40% and 40% of their annual base salary, respectively. Mr. Cumbo, Dr. Brooks and Mr. Howton must be employed on the date that bonuses are paid in order to receive their respective bonus, provided that if such executive is terminated by us without cause (as "cause" is defined in their respective employment agreements) between January 1 of the year following the performance year and the date of payment, such executive will be entitled to the same bonus that he would have received had he remained employed through the payment date.

In accordance with Mr. Cumbo's employment agreement, on December 2, 2022, we granted Mr. Cumbo an option, or the Cumbo Option**,** to purchase 228,900 shares of our common stock, at an exercise price per share equal to $6.77, which vested as to 25% of the shares underlying the Cumbo Option on the first anniversary of the grant date and vests as to an additional 1/48th of the total shares underlying the Cumbo Option upon his completion of each additional month of service over the 36-month period measured from the first anniversary of the grant date. We also granted Mr. Cumbo RSUs with respect to 114,449 shares of our common stock, or the Cumbo RSU, which vests as to 25% of the shares underlying the Cumbo RSU on each anniversary of December 2, 2022, subject to continued service. The Cumbo Option and the Cumbo RSU were granted as an inducement material to Mr. Cumbo's acceptance of employment with us in accordance with Nasdaq Listing Rule 5635(c)(4).

In accordance with Dr. Howton's employment agreement, on December 2, 2022, we granted Mr. Howton an option, or the Howton Option, to purchase 104,410 shares of our common stock, at an exercise price per share equal to $6.77, which vested as to 25% of the shares underlying the Howton Option on the first anniversary of the grant date and vests as to an additional 1/48th of the total shares underlying the Howton Option upon his completion of each additional month of service over the 36-month period measured from the first anniversary of the grant date. We also granted Mr. Howton RSUs with respect to 52,205 shares of our common stock, or the Howton RSU, which vests as to 25% of the shares underlying the Howton RSU on each anniversary of December 2, 2022, subject to continued service. The Howton Option and the Howton RSU were granted as an inducement material to Mr. Howton's acceptance of employment with us in accordance with Nasdaq Listing Rule 5635(c)(4).

In accordance with Dr. Brook's employment agreement, on October 18, 2023, we granted Dr. Brooks an option, or the Brooks Option, to purchase 78,000 shares of our common stock, at an exercise price per share equal to $2.15, which vested as to 25% of the shares underlying the Brooks Option on the first anniversary of the grant date and vests as to an additional 1/48th of the total shares underlying the Brooks Option upon his completion of each additional month of service over the 36-month period measured from the first anniversary of the grant date. We also granted Dr. Brooks RSUs with respect to 39,000 shares of our common stock, or the Brooks RSU, which vests as to 25% of the shares underlying the Brooks RSU on each anniversary of October 18, 2023, subject to continued service.

Mr. Cumbo, Dr. Brooks and Mr. Howton are bound by proprietary rights, non-disclosure, developments, non-competition and non-solicitation obligations pursuant to the restrictive covenants provided for in their respective employment agreements.

Under these restrictive covenants, each executive agrees not to compete with us during his employment and for a period of one year after the termination of his employment, not to solicit our employees, consultants, or actual or prospective customers or business relations during his employment and for a period of one year after the termination of his employment, and to protect our confidential and proprietary information indefinitely. In addition, under these restrictive covenants, each executive agrees that we own all inventions that are developed by him during a specified period of time with respect to any inventions made by him that are related to his activities while employed by us.

The employment of each of Mr. Cumbo, Dr. Brooks and Mr. Howton may be terminated as follows: (1) upon the death of the executive or at our election due to the executive's "disability" (as disability is defined in the applicable employment agreement); (2) at our election, with or without "cause" (as cause is defined in the applicable employment agreement); and (3) at such executive's election, with or without "good reason" (as good reason is defined in the applicable employment agreement).

In the event of the termination of employment of Mr. Cumbo, Dr. Brooks or Mr. Howton by us without cause, or by such executive for good reason, prior to, or more than 12 months, in the case of Mr. Cumbo and Mr. Howton, and 18 months, in the case of Dr. Brooks, following a "change in control" (as change in control is defined in the applicable employment agreement), the executive is entitled to receive his base salary that has accrued and to which he is entitled as of the termination date, to the extent consistent with our policy, accrued but unused paid time off through and including the termination date, unreimbursed business expenses for which expenses the executive has timely submitted appropriate documentation, and other amounts or benefits to which the executive is entitled in accordance with the terms of the benefit plans then-sponsored by us, which we refer to collectively as the Accrued Obligations. In addition, subject to the executive's execution and nonrevocation of a release of claims in our favor, the executive is entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 12 months, (2) in the case of Dr. Brooks, a lump sum payment equal to 100% of his target bonus for the year in which his employment is terminated, or, if higher, his target bonus immediately prior to the change in control, prorated through his date of termination and (3) provided he is eligible for and timely elects to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 12 months following his date of termination.

In the event of the termination of employment of Mr. Cumbo, Dr. Brooks or Mr. Howton by us without cause, or by such executive for good reason, within 12 months, in the case of Mr. Cumbo and Mr. Howton, and 18 months, in the case of Dr. Brooks, following a change in control, the executive is entitled to receive the Accrued Obligations. In addition, subject to the executive’s execution and nonrevocation of a release of claims in our favor, the executive is entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 18 months, in the case of Mr. Cumbo, and 12 months, in the case of Mr. Tan and Mr. Howton, (2) provided the executive is eligible for and timely elects to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 18 months, in the case of Mr. Cumbo, and 12 months, in the case of Dr. Brooks and Mr. Howton, following his date of termination, (3) a lump sum payment equal to 150%, in the case of Mr. Cumbo, and 100%, in the case of Dr. Brooks and Mr. Howton, of the executive's target bonus for the year in which his employment is terminated or, if higher, the executive's target bonus immediately prior to the change in control and (4) full vesting acceleration of any then-unvested equity awards that vest based solely based on the passage of time held by the executive, such that any such equity awards held by the executive become fully exercisable or non-forfeitable as of the termination date.

If the employment of Mr. Cumbo, Dr. Brooks or Mr. Howton is terminated for any other reason, including as a result of his death or disability, for cause, or voluntarily by him without good reason, our obligations under such employment agreement cease immediately, and the executive is only entitled to receive the Accrued Obligations.

Outstanding Equity Awards at 2024 Fiscal Year-End

The following table sets forth information regarding equity awards held by our Named Executive Officers as of December 31, 2024.

	Option Awards					Stock Awards						
Name	Number of securities underlying unexercised options (#) exercisable	Number of securities underlying unexercised options (#) unexercisable		Option exercise price ($)	Option expiration date	Number of shares of stock that have not vested (#)		Market value of shares of stock that have not vested ($) (1)		Equity incentive plan awards: number of unearned shares, units or other rights that have not vested (#)		Equity incentive plan awards: market or payout value of unearned shares, units or other rights that have not vested ($)(1)	
Alexander Cumbo	114,452	114,448	(2)	6.77	12/02/2032								
	-	240,250	(3)	7.85	2/13/2034								
						57,225	(4)	228,900	(4)				
						120,125	(5)	480,500	(5)				
										169,401	(6)	677,604	(6)
Gabriel Brooks	22,751	55,249	(7)	2.15	10/18/2033								
	-	87,500	(3)	7.85	2/13/2034								
						29,250	(8)	117,000	(8)				
						43,750	(5)	175,000	(5)				
										58,847	(6)	235,388	(6)
David Tyronne Howton	52,208	52,202	(2)	6.77	12/02/2032								
	-	117,500	(3)	7.85	2/13/2034								
						26,103	(4)	104,412	(4)				
						58,750	(5)	235,000	(5)				
										79,341	(6)	317,364	(6)

(1) Based on the $4.00 closing sale price of our common stock on December 31, 2024 as reported by the Nasdaq Global Select Market.

(2) This option was granted on December 2, 2022 as an inducement grant. The option is subject to vesting with respect to 25% of the shares underlying the option on the first anniversary of the grant date and as to an additional 2.0833% of the original number of shares underlying the option monthly thereafter through the fourth anniversary of the grant date.

(3) This option was granted on February 13, 2024 under the 2020 Plan and is subject to vesting with respect to 25% of the shares underlying the option on the first anniversary of the grant date and as to an additional 2.0833% of the original number of shares underlying the option monthly thereafter through the fourth anniversary of the grant date.

(4) These RSUs were granted on December 2, 2022 as inducement grants. The RSUs vest in equal annual installments over a term of four years from the date of grant.

(5) These RSUs were granted on February 13, 2024 under the 2020 Plan and vest in equal annual installments over a term of four years from the date of grant.

(6) These PSUs were granted on June 11, 2024 under the 2020 Plan. The Performance Milestones are tied to the achievement of certain business objectives and are non-market and non-financial in nature. The Board of Directors will determine the number of units that will ultimately vest on the 2026 Evaluation Date, which will occur in the first quarter of 2026, and the 2027 Evaluation Date, which will occur in the first quarter of 2027. A maximum of 25% of the target

number of PSUs may vest at the 2026 Evaluation Date. The number in the table assumes 25% achievement on the 2026 Evaluation Date. For more information about the PSUs, see Narrative Disclosure to Summary Compensation Table in Item 11, "Executive Compensation" of this Annual Report on Form 10-K.

(7) This option was granted on October 18, 2023 under the 2020 Plan and is subject to vesting with respect to 25% of the shares underlying the option on the first anniversary of the grant date and as to an additional 2.0833% of the original number of shares underlying the option monthly thereafter through the fourth anniversary of the grant date.

(8) These RSUs were granted on October 18, 2023 under the 2020 Plan and vest in equal annual installments over a term of four years from the date of grant.

Policies and Practices Related to the Grant of Equity Awards

We grant equity awards, including stock options and/or restricted stock units, to our employees on an annual basis. We grant stock options to our directors on an annual basis. We may also grant stock options and/or restricted stock units to individuals upon hire or promotion or for retention purposes. We currently do not grant stock appreciation rights or similar option-like instruments. During the year ended December 31, 2024, neither the Board of Directors nor the compensation committee took material nonpublic information into account when determining the timing or terms of equity awards, nor did we time the disclosure of material nonpublic information for the purpose of affecting the value of executive compensation.

While we do not have any policy or obligation that requires us to grant equity awards on specified dates, annual stock option and/or restricted stock unit grants to employees are typically approved in January or February of each year. During the year ended December 31, 2024, new hire equity grants were made by our Chief Executive Officer on the first business day of the month following such employee's hire pursuant to delegated authority under our 2024 Inducement Stock Incentive Plan. Our Chief Executive Officer does not take into account material nonpublic information when determining the timing or terms of such equity grants. Under our non-employee director compensation program, our annual stock option grants to directors are granted on the date of our annual meeting of stockholders.

During the year ended December 31, 2024, we did not grant stock options to any named executive officer during any period beginning four business days before and ending one business day after the filing of any Form 10-Q or Form 10-K, or the filing or furnishing of a Form 8-K that discloses material nonpublic information.

Non-Employee Director Compensation

Non-employee director compensation is set by our Board of Directors at the recommendation of our compensation committee. In 2024, the compensation committee retained Radford, an AON Hewitt company, to assist in assessing our non-employee director compensation program and provide recommendations with respect to the compensation program.

Under our current director compensation program, we pay our non-employee directors a cash retainer for their service on the Board of Directors and for their service on each committee of which the director is a member. The chairs of each committee receive higher retainers for such service. These fees are payable in arrears in equal semi-annual installments not later than the 15th business day following the end of the second and fourth calendar quarters, provided that the amount of such payment will be prorated for any portion of such semi-annual period that the director is not serving on the Board of Directors, on such committee or in such position. The fees paid to non-employee directors for their service on the Board of Directors and for their service on each committee of the Board of Directors of which the director is a member are as follows:

	Member Annual Fee ($)	Chairperson Incremental Annual Fee ($)
Board of Directors	40,000	35,000
Audit Committee	7,500	7,500
Clinical Committee	7,500	7,500
Compensation Committee	5,000	5,000
Nominating and Corporate Governance Committee	4,000	4,000

We also reimburse our non-employee directors for reasonable out-of-pocket business expenses incurred in connection with the performance of their duties as directors, including travel expenses in connection with their attendance in person at our Board of Directors and committee meetings.

In addition, under our current director compensation program, each new non-employee director elected to our Board of Directors will receive an option to purchase 68,800 shares of our common stock under the 2020 Plan. Each of these options vests in equal annual installments over a three-year period measured from the date of grant, subject to the director's continued service as a director. Further, on the date of our annual meeting of stockholders, each non-employee director that has served on our Board of Directors for at least six months prior to such annual meeting will receive an option to purchase 34,400 shares of our common stock under our 2020 Plan. Each of these options vests in full on the earlier to occur of the one-year anniversary of the grant date and immediately prior to our first annual meeting of stockholders occurring after the grant date, subject to the director's continued service as a director. All options granted to our non-employee directors under our director compensation program will be issued at exercise prices equal to the fair market value of our common stock on the date of grant and will become exercisable in full in the event of a change in control.

This program is intended to provide compensation for our non-employee directors in a manner that enables us to attract and retain outstanding director candidates and reflects the substantial time commitment necessary to oversee our affairs. We also seek to align the interests of our directors and our stockholders, and we have chosen to do so by compensating our non-employee directors with a mix of cash and equity-based compensation.

The table below shows the compensation paid to our non-employee directors during 2024.

Name	Fees Earned or Paid in Cash ($)	Option Awards ($) (1) (2)	Stock Awards ($)(3)	All Other Compensation ($)	Total ($)
Ian Smith	75,000	299,160 (4)	75,001 (5)	—	449,161
Martin Freed, M.D., F.A.C.P.	60,000	224,204 (6)	—	—	284,204
Ilan Ganot	40,000	224,204 (6)	—	241,145 (7)	505,349
Clare Kahn, Ph.D.	51,500	224,204 (6)	—	—	275,704
Georgia Keresty, Ph.D., M.PH.	49,247	224,204 (6)	—	—	273,451
Adam Koppel, M.D., Ph.D.(8)	17,901	46,803	—	—	64,704
Sukumar Nagendran, M.D.	52,500	224,204 (6)	—	—	276,704
Rajeev Shah(8)	22,376	54,551	—	—	76,927
Adam Stone	55,500	224,204 (6)	—	—	279,704
Lynne Sullivan	59,000	224,204 (6)	—	—	283,204

(1) The amounts in this column (i) represent the aggregate grant date fair value of the options granted to the non-employee directors as computed in accordance with FASB ASC Topic 718 and (ii) for Dr. Koppel and Mr. Shah, represents $46,803 and $54,551, respectively, of incremental fair value related to previously granted and vested options that were modified in August 2024, computed as of the modification date in accordance with FASB ASC Topic 718. The modification extended the exercise period for options that were vested as of the date of their resignation from our Board of Directors. The assumptions used in calculating the grant date fair value of the options are set forth in Note 9 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.

(2) As of December 31, 2024, our non-employee directors held options to purchase shares of our common stock as follows: Mr. Smith: 203,906 shares; Dr. Freed: 65,430 shares; Mr. Ganot: 150,314 shares; Dr. Kahn: 62,432 shares; Dr. Keresty: 59,766 shares; Dr. Koppel: 21,966 shares; Dr. Nagendran: 63,765 shares; Mr. Shah: 28,032 shares; Mr. Stone: 62,432 shares; and Ms. Sullivan: 62,432 shares

(3) The amount in this column represents the aggregate grant date fair value of the RSUs as computed in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value of the award are set forth in Note 9 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K. As of December 31, 2024, our non-employee directors held RSUs with respect to shares of our common stock as follows: Mr. Smith: 3,255 RSUs and Mr. Ganot: 7,767 RSUs.

(4) Consists of (i) an option to purchase 15,112 shares of our common stock granted on January 3, 2024 in respect of his services as Executive Chairman and (ii) an option to purchase 34,400 shares of our common stock granted on June 11, 2024 in accordance with our non-employee director compensation program.

(5) Consists of 13,021 RSUs granted on January 3, 2024 in respect of his services as Executive Chairman.

(6) Consists of an option to purchase 34,400 shares of our common stock granted on June 11, 2024 in accordance with

our non-employee director compensation program.

(7) Represents severance payments (representing continued payment of his former base salary) made to Mr. Ganot pursuant to the Ganot Transition Agreement (as defined below). For further information about the Ganot Consulting Agreement and Ganot Transition Agreement, see Item 13, "Certain Relationships and Related Transactions, and Director Independence" of this Annual Report on Form 10-K.

(8) Resigned from our Board of Directors effective June 11, 2024.

In respect of his services as Executive Chairman of our Board of Directors, on January 6, 2025, we granted Mr. Smith an option to purchase 22,148 shares of our common stock and 18,293 RSUs.

Annie Ganot, who is the wife of Ilan Ganot and one of our co-founders, serves as our Vice President, Patient Advocacy. The compensation she receives in connection with such service is set forth in Item 13, "Certain Relationships and Related Transactions, and Director Independence" of this Annual Report on Form 10-K.

During 2024, we did not provide any compensation to Mr. Cumbo, our President and Chief Executive Officer, for his service as a member of our Board of Directors. Mr. Cumbo's compensation for his service as an executive officer is set forth above under "Compensation of our Named Executive Officers — 2024 Summary Compensation Table."

Compensation Committee Interlocks and Insider Participation

None of the current members of our compensation committee has ever been an executive officer or employee of ours. None of our executive officers currently serves, or has served during the last completed fiscal year, on the compensation committee or board of directors of any other entity that has one or more executive officers serving as a member of our Board of Directors or compensation committee.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth information regarding the beneficial ownership of our common stock as of February 24, 2025 by (i) each person whom we know to beneficially own more than 5% of our outstanding common stock, which we refer to as a 5% stockholder, (ii) each director, (iii) each Named Executive Officer and (iv) all current directors and executive officers as a group. Unless otherwise indicated, the address of each executive officer and director is c/o Solid Biosciences Inc., 500 Rutherford Avenue, 3rd Floor, Charlestown, Massachusetts 02129.

The number of shares of common stock "beneficially owned" by each stockholder is determined under rules issued by the SEC regarding the beneficial ownership of securities. This information is not necessarily indicative of beneficial ownership for any other purpose. Under these rules, beneficial ownership of shares of our common stock includes (1) any shares as to which the person or entity has sole or shared voting power or investment power and (2) any shares as to which the person or entity has the right to acquire beneficial ownership within 60 days after February 24, 2025. The percentage of beneficial ownership in the table below is based on 76,516,805 shares of common stock deemed to be outstanding as of February 24, 2025.

Unless otherwise indicated below, and subject to community property laws where applicable, to our knowledge, all persons named in the table have sole voting and investment power with respect to their shares of common stock.

Name of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned
5% Stockholders:		
Perceptive Life Sciences Master Fund Ltd. and affiliated entities(1)	11,934,678	15.59 %
Entities affiliated with RA Capital Management, L.P.(2)	8,011,678	9.99 %
Entities affiliated with Bain Capital Life Sciences Investors, LLC(3)	7,933,582	9.99 %
Invus Public Equities, L.P. and affiliated entities (4)	7,415,905	9.69 %
Commodore Capital Master LP(5)	5,825,000	7.61 %
Entities managed by Vestal Point Capital, LP (6)	4,288,850	5.61 %
Adage Capital Management, L.P.(7)	4,248,084	5.55 %
Named Executive Officers and Directors:		
Alexander Cumbo(8)	264,314	*
Gabriel Brooks(9)	77,585	*
David Tyronne Howton(10)	119,969	*
Ian Smith(11)	292,589	*
Martin Freed(12)	34,614	*
Ilan Ganot(13)	257,781	*
Clare Kahn(14)	29,132	*
Georgia Keresty(15)	25,366	*
Sukumar Nagendran(16)	33,515	*
Adam Stone(17)	28,032	*
Lynne Sullivan(18)	28,032	*
All current directors and executive officers as a group (14 persons)(19)	1,461,139	1.88 %

* Less than 1 percent

(1) Consists of (a) 11,833,539 shares held by Perceptive Life Sciences Master Fund, Ltd., (b) 73,107 shares held by Perceptive Xontogeny Venture Fund, L.P. and (c) 28,032 shares underlying options held by Adam Stone, a member of our Board of Directors, that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date. Perceptive Advisors LLC is the investment manager to Perceptive Life Sciences Master Fund, Ltd. and may be deemed to beneficially own the securities directly held by Perceptive Life Sciences Master Fund, Ltd. Perceptive Xontogeny Venture GP, LLC is the investment manager to Perceptive Xontogeny Venture Fund, LP. Joseph Edelman is the managing member of Perceptive Advisors LLC and Perceptive Xontogeny Venture GP, LLC. Perceptive Advisors LLC, Perceptive Xontogeny Venture GP, LLC and Mr. Edelman may be deemed to beneficially own the shares held by Perceptive Life Sciences Master Fund, Ltd. and Perceptive Xontogeny Venture Fund, LP. The address of Perceptive is 51 Astor Place, 10th Floor, New York, New York 10003. Perceptive Life Sciences Master Fund, Ltd. reports that it holds shared voting and shared dispositive power with respect to 11,861,572 shares, Perceptive Xontogeny Venture Fund, L.P. reports that it holds shared voting and shared dispositive power with respect to 73,107 shares and Perceptive Advisors LLC reports that it holds shared voting and dispositive power with respect to 11,934,679 shares. Based on information set forth in a Schedule 13D/A filed with the SEC on February 21, 2025.

(2) Consists of (a) 4,192,216 shares held by RA Capital Healthcare Fund, L.P., or RACHF, (b) 109,661 shares held by RA Capital Nexus Fund, L.P., or RACNF, (c) 28,569 shares held by a separately managed account, which is referred to, together with RACHF and RACNF, as the RA Capital Entities, (d) 28,032 shares of common stock underlying vested options held by Rajeev Shah, a former member of our Board of Directors, for the benefit of RA Capital Management, L.P., or RACM, that are exercisable as of February 24, 2025 and (e) pre-funded warrants exercisable for up to 3,722,085 shares of common stock held by RACHF. The pre-funded warrants contain a provision which precludes the exercise of the pre-funded warrants to the extent that, following exercise, RACHF, together with its affiliates and other attribution parties, would own more than 9.99% of our common stock outstanding. RACHF, together with its affiliates and other attribution parties, is currently prohibited from exercising the pre-funded to the extent that such exercise would result in beneficial ownership of more than 8,011,678 shares of common stock. RA Capital Healthcare Fund GP, LLC is the general partner of RACHF and RA Capital Nexus Fund GP, LLC is the general partner of RACNF. RACM is the investment manager for RACHF, RACNF and the separately managed account. The general partner of RACM is RA Capital Management GP, LLC, or RACM GP, of which Dr. Peter Kolchinsky and Mr. Shah are the managing members. Mr. Shah is a former member of our Board of Directors. RACM, RACM GP, Dr. Kolchinsky and Mr. Shah may be deemed to have voting and investment power over the shares held of record by RACHF, RACNF and the separately managed account. RACM, RACM GP, Dr. Kolchinsky and Mr. Shah expressly disclaim beneficial ownership over all shares held by RACHF, RACNF, the separately managed account and Mr. Shah, except to the extent of their pecuniary interest therein. The address for each of RACHF, RACNF and RACM is 200 Berkeley Street,

18th Floor, Boston, Massachusetts 02116. RACM reports that it may be deemed to share voting power and dispositive power with respect to the shares held by the RA Capital Entities. Based on information set forth in a Schedule 13D/A filed with the SEC on February 21, 2025.

(3) Consists of (a) 528,660 shares held by BCLS SB Investco, LP., or BCLS SB, (b) an aggregate of 3,301,955 shares and pre-funded warrants exercisable for up to 6,444,170 shares of common stock held by BCLS II Investco, LP, or BCLS II Investco, (c) 267,257 shares held by Bain Capital Life Sciences Fund II, L.P., or BCLS Fund II, (d) 32,550 shares held by BCIP Life Sciences Associates, LP, or BCIPLS, and (e) 904,160 shares held by BCLS II Equity Opportunities, LP, or BCLS II EO, which is referred to, together with BCIPLS, BCLS SB, BCLS II Investco and BCLS Fund II, as the Bain Capital Life Sciences Entities. Bain Capital Life Sciences Investors, LLC (i) is the manager of Bain Capital Life Sciences Partners, LP, which is the general partner of BCLS SB, (ii) is the manager of Bain Capital Life Sciences Investors II, LLC, which is the general partner of BCLS Fund II, which is the managing member of BCLS II Investco (GP), LLC, which is the general partner of BCLS II Investco, and (iii) governs the investment strategy and decision-making process with respect to investments held by BCIPLS. As a result, Bain Capital Life Sciences Investors, LLC may be deemed to share voting and dispositive power with respect to the shares held by the Bain Capital Life Sciences Entities. BCLS II Investco is prohibited from exercising the pre-funded warrants to the extent that BCLS II Investco, together with its affiliates and other attribution parties, would, after such exercise, collectively beneficially own in excess of 9.99% of our outstanding common stock. BCLS II Investco, together with its affiliates and other attribution parties, is currently prohibited from exercising the pre-funded to the extent that such exercise would result in beneficial ownership of more than 7,933,582 shares of common stock. The address of the Bain Capital Life Sciences Entities is c/o Bain Capital Life Sciences, LP, 200 Clarendon Street, Boston, Massachusetts 02116. Based on information set forth in a Schedule 13D/A filed with the SEC on February 21, 2025.

(4) Consists of 6,402,653 shares held by Invus Public Equities L.P., or Invus Public Equities, and 1,013,252 shares held by Avicenna Life Sci Master Fund LP, or Avicenna Fund. Invus Public Equities Advisors, LLC, or Invus PE Advisors, as the general partner of Invus Public Equities, controls Invus Public Equities and, accordingly, may be deemed to beneficially own the shares directly held by Invus Public Equities. Invus Global Management, LLC, or Global Management, as the managing member of Invus PE Advisors, controls Invus PE Advisors and, accordingly, may be deemed to beneficially own the shares that Invus PE Advisors may be deemed to beneficially own. Siren, L.L.C., or Siren, as the managing member of Global Management, controls Global Management and, accordingly, may be deemed to beneficially own the shares that Global Management may be deemed to beneficially own. Avicenna Life Sci Master GP LLC, or Avicenna GP, as the general partner of Avicenna Fund, controls Avicenna Fund and, accordingly, may be deemed to beneficially own the Shares beneficially held by Avicenna Fund. Ulys, as the managing member of Avicenna GP, controls Avicenna GP and, accordingly, may be deemed to beneficially own the shares that Avicenna GP may be deemed to beneficially own. Mr. Raymond Debbane, as the managing member of Siren and Ulys L.L.C., or Ulys, controls Siren and Ulys and, accordingly, may be deemed to beneficially own the shares that Siren and Ulys may be deemed to beneficially own. The address of Invus Public Equities and its affiliates is 750 Lexington Avenue, 30th Floor, New York, NY 10022. Based, in part, on information set forth in a Schedule 13G filed on February 12, 2025 and the shares purchased in the underwritten offering on February 19, 2025.

(5) Consists of shares held by Commodore Capital Master LP, or Commodore. Commodore Capital LP is the investment manager of Commodore and may be deemed to beneficially own these shares. Michael Kramarz and Robert Egen Atkinson are the managing partners of Commodore Capital LP and exercise investment discretion with respect to these securities. The address of Commodore is 444 Madison Avenue, Floor 35, New York, New York 10022. Based on information set forth in a Schedule 13G filed with the SEC on February 25, 2025.

(6) Consists of shares held by Vestal Point Capital, LP. The sole general partner of Vestal Point Master Fund, LP is Vestal Point Partners GP, LLC. The managing member of Vestal Point Partners GP, LLC is Ryan Wilder. The sole general partner of Vestal Point Capital, LP is Vestal Point Capital, LLC. The managing member of Vestal Point Capital, LLC is Mr. Wilder. As a result, Mr. Wilder may be deemed to have voting and investment power over the securities held by Vestal Point Master Fund, LP and the account separately managed by Vestal Point Capital, LP. Mr. Wilder disclaims beneficial ownership of such securities, except to the extent of his pecuniary interest therein. The address of these entities and Mr. Wilder is c/o Vestal Point Capital, LP, 632 Broadway, Suite 602, New York, NY 10012. Based, in part, on information set forth in a Schedule 13G filed on November 14, 2024 and the shares purchased in the underwritten offering on February 19, 2025.

(7) Consists of shares held by Adage Capital Partners, L.P. Robert Atchinson and Phillip Gross are the managing members of Adage Capital Advisors, L.L.C., which is the managing member of Adage Capital Partners GP, L.L.C., which is the general partner of Adage Capital Partners, LP, and each such person or entity, as the case may be, has shared voting and/or dispositive power over the securities held by Adage Capital Partners, LP and may be deemed the beneficial owner of such shares, and each such person or entity, as the case may be, disclaims beneficial ownership of such securities except to the extent of their respective pecuniary interest therein. The address of Adage Capital Partners, LP, Adage Capital Advisors, L.L.C., Adage Capital Partners GP, L.L.C., Mr. Atchinson and Mr. Gross is c/o Adage

Capital Partners, L.P., 200 Clarendon St., 52nd Floor, Boston, MA 02116. Based on information set forth in a Form 4 filed with the SEC on February 19, 2025.

(8) Consists of (a) 60,717 shares of common stock owned by Mr. Cumbo and (b) 203,597 shares of common stock underlying options held by Mr. Cumbo that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(9) Consists of (a) 22,812 shares of common stock owned by Dr. Brooks and (b) 54,773 shares of common stock underlying options held by Dr. Brooks that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(10) Consists of (a) 24,789 shares of common stock owned by Mr. Howton and (b) 95,180 shares of common stock underlying options held by Mr. Howton that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(11) Consists of (a) 112,972 shares of common stock owned by Mr. Smith, (b) 175,043 shares of common stock underlying options held by Mr. Smith that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date and (c) 4,574 shares of common stock underlying RSUs held by Mr. Smith that will vest within 60 days after such date.

(12) Consists of (a) 3,584 shares of common stock owned by Dr. Freed and (b) 31,030 shares of common stock underlying options held by Dr. Freed that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(13) Consists of (a) 94,648 shares held by Mr. Ganot as an individual, (b) 4,042 shares held by Mr. Ganot and Ms. Ganot as joint tenants with right of survivorship, (c) 19,394 shares held by Mr. Adam Ganot and Ms. Ganot and their successors, as trustees for the Ilan Ganot 2017 Irrevocable Trust, (d) 108,147 shares of common stock underlying options held by Mr. Ganot that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date, (e) 14,839 shares held by Ms. Ganot, and (f) 16,711 shares of common stock underlying options held by Ms. Ganot that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date

(14) Consists of (a) 1,100 shares of common stock owned by Dr. Kahn, and (b) 28,032 shares of common stock underlying options held by Dr. Kahn that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(15) Consists of 25,366 shares of common stock underlying options held by Dr. Keresty that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(16) Consists of (a) 4,150 shares of common stock owned by Dr. Nagendran and (b) 29,365 shares of common stock underlying options held by Dr. Nagendran that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(17) Consists of 28,032 shares of common stock underlying options held by Mr. Stone that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date. Mr. Stone is Chief Investment Officer of Perceptive Advisors LLC. Mr. Stone disclaims beneficial ownership of the shares held by Perceptive. The address of Mr. Stone is 51 Astor Place, 10th Floor, New York, NY 10003.

(18) Consists of 28,032 shares of common stock underlying options held by Ms. Sullivan that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date.

(19) Includes (a) 1,023,599 shares of common stock underlying options that are exercisable as of February 24, 2025 or will become exercisable within 60 days after such date and (b) 4,574 shares of common stock underlying RSUs that will vest within 60 days after February 24, 2025.

Securities Authorized for Issuance under Equity Compensation Plans

The following table provides information about our equity compensation plans as of December 31, 2024. As of December 31, 2024, we had four equity compensation plans: our 2018 Omnibus Incentive Plan, our 2020 Plan, our Amended and Restated 2021 Employee Stock Purchase Plan, or the ESPP, each of which was approved by our stockholders, and our 2024 Inducement Stock Incentive Plan, which plan was not approved by our stockholders. We have also made inducement awards outside of our equity compensation plans to certain new hires, which awards were not approved by our stockholders.

Plan Category	(a) Number of securities to be issued upon the exercise of outstanding options, warrants and rights		(b) Weighted-average exercise price of outstanding options, warrants and rights (2)	(c) Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a))	
Equity compensation plans approved by security holders	5,227,045	(1)	$ 22.84	663,969	(3)(4)
Equity compensation plans not approved by security holders	1,346,489	(5)	6.91	599,933	
Total	6,573,534		$ 18.22	1,263,902	

(1) Reflects shares issuable upon exercise of options and settlement of RSUs and PSUs (assuming payout of PSUs at the maximum achievement level).

(2) The weighted-average exercise price does not include RSUs and PSUs, which have no exercise price.

(3) The number of shares of common stock reserved for issuance under the 2020 Plan will be increased on the first day of each fiscal year beginning with the fiscal year ended December 31, 2023 and ending in the fiscal year ending December 31, 2032, in an amount equal to the lesser of: (i) 5% of the outstanding shares of our common stock on the first day of the applicable fiscal year and (ii) an amount determined by our Board of Directors. On January 1, 2025, the shares of common stock reserved under the 2020 Plan were increased by 2,023,407 shares pursuant to the annual increase described above.

(4) The number of shares of common stock reserved for issuance under the ESPP will be increased on the first day of each fiscal year beginning with the fiscal year ending December 31, 2024 and ending in the fiscal year ending December 31, 2033, in an amount equal to the lesser of: (i) 293,597 shares of common stock, (ii) 1% of the outstanding shares of our common stock on the first day of the applicable fiscal year and (iii) an amount determined by our Board of Directors. On January 1, 2025, the shares reserved under the ESPP were increased by 293,597 shares pursuant to the annual increase described above.

(5) Represents inducement stock option and RSU awards granted to employees in accordance with Nasdaq Listing Rule 5635(c)(4), with an exercise price equal to the closing price of our common stock on the date of grant, for inducement stock option awards.
This aggregate amount includes the following inducement awards made to our officers (as defined in Exchange Act Rule 16a-1(f)):

Name	Options(1)	RSUs(2)	Grant Date
Alexander Cumbo, *President, Chief Executive Officer and Director*	228,900	114,449	12/2/2022
Kevin Tan, *Chief Financial Officer and Treasurer*	90,000	45,000	1/9/2023
David Tyronne Howton, *Chief Operating Officer and Secretary*	104,410	52,205	12/2/2022
Jessie Hanrahan, Ph.D., *Chief Regulatory Officer*	94,899	47,449	12/2/2022
Paul Herzich, *Chief Technology Officer*	57,900	28,955	12/2/2022

(1) Vests over a term of four years, with 25% of the original number of shares vesting on the first anniversary of the grant date and 2.0833% of the original number of shares monthly thereafter until the fourth such anniversary.

(2) Vests over a term of four years, with 25% of the original number of shares vesting on each anniversary of the grant date until the fourth such anniversary.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

Transactions with Related Persons

In addition to the executive officer and director compensation arrangements discussed in Item 11, "Executive Compensation" of this Annual Report on Form 10-K, the following describes transactions since January 1, 2023 to which we have been or will be a participant, in which the amount involved in the transaction exceeds the lesser of $120,000 or 1% of our total assets at year end for each of the last two completed fiscal years and in which any of our directors, executive officers or beneficial holders of more than 5% of any class of our capital stock, or any immediate family member of, or person sharing the household with, any of these individuals, had or will have a direct or indirect material interest.

Amended and Restated Registration Rights Agreement

We are party to an Amended and Restated Registration Rights Agreement, or the Registration Rights Agreement, dated March 29, 2017, with certain of our stockholders, or the Investors, which includes holders of more than 5% of our voting securities and entities affiliated with certain of our directors. The Registration Rights Agreement provides the Investors the right, subject to certain conditions, to demand that we file a registration statement or to request that their shares be covered by a registration statement that we are otherwise filing.

February 2025 Underwritten Registered Offering

On February 19, 2025, we closed an underwritten offering in which we issued and sold an aggregate of 35,739,810 shares of our common stock at a price per share of $4.03 and pre-funded warrants to purchase 13,888,340 shares of our common stock at a price per warrant of $4.029, before expenses and underwriting discounts and commissions. We received aggregate net proceeds of approximately $187.5 million, after deducting expenses and underwriting discounts and commissions. The number of shares that our holders of more than 5% of our voting securities purchased and the aggregate purchase price paid for such shares is set forth in the table below.

Purchaser (1)	Pre-Funded Warrants Purchased (#)	Aggregate Purchase Price for Pre-Funded Warrants ($)	Shares of Common Stock Purchased (#)	Aggregate Purchase Price for Shares ($)
BCLS II Investco, LP	6,444,170	25,963,561	1,000,000	4,030,000
Perceptive Life Sciences Master Fund Ltd.	—	—	5,000,000	20,150,000
RA Capital Healthcare Fund, LP	3,722,085	14,996,281	—	—
Adage Capital Partners, L.P.	—	—	2,481,400	10,000,042
Invus Public Equities, L.P.	—	—	3,722,100	15,000,063
Vestal Point Capital, LP	—	—	1,488,850	6,000,066

(1) See Item 12, "Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters" of this Annual Report on Form 10-K for more information about the shares held by the identified entities.

January 2024 Private Placement

On January 8, 2024, we entered into a securities purchase agreement with respect to the private placement of an aggregate of 16,973,103 shares of our common stock at a price of $5.53 per share, and, to one investor in lieu of shares, pre-funded warrants to purchase 2,712,478 shares of our common stock, at a price of $5.529 per pre-funded warrant. We received $103.7 million in aggregate net proceeds from the private placement, after deducting offering costs. The number of shares purchased by the holders of more than 5% of our voting securities at the time of the private placement and the aggregate purchase price paid for such shares is set forth in the table below.

Name (1)	Shares of Common Stock Purchased (#)	Purchase Price ($)
Perceptive Life Sciences Master Fund Ltd.	3,410,713	18,861,243
Adage Capital Partners, L.P.	2,712,478	15,000,003
Deerfield Partners, L.P.	2,260,398	12,500,001
Invus Public Equities, L.P.	1,808,319	10,000,004
RA Capital Healthcare Fund, L.P.	904,160	5,000,005
BCLS II Equity Opportunities, LP	904,160	5,000,005

(1) See Item 12, "Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters" of this Annual Report on Form 10-K for more information about the shares held by the identified entities.

Arrangements with Ian F. Smith

Mr. Smith became our Executive Chairman, effective January 1, 2022. The compensation that we have paid to Mr. Smith in connection with his services as Executive Chairman and as our non-employee director is discussed above in Item 11, "Executive Compensation" of this Annual Report on Form 10-K.

Arrangements with Ilan Ganot

Mr. Ganot is a member of our Board of Directors and served as our President and Chief Executive Officer until December 2022. On September 29, 2022, we entered into an Executive Transition and Separation Agreement with Mr. Ganot, or the Ganot Transition Agreement, which became effective as of December 2, 2022, or the Separation Date. Pursuant to the Ganot Transition Agreement, Mr. Ganot was entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 18 months from the Separation Date (an aggregate of $868,050) and (2) provided he was eligible for and timely elected to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 18 months following the Separation Date. Mr. Ganot also received $477,427, less applicable taxes and withholdings, which is a lump sum payment equal to 150% of his target bonus for 2022.

On September 29, 2022, we also entered into a Consulting Agreement with Mr. Ganot, or the Ganot Consulting Agreement, effective as of the Separation Date, pursuant to which Mr. Ganot assisted us with the transition of his duties to Mr. Cumbo and provided other consulting and advisory services, as requested from time to time by us. The Ganot Consulting Agreement expired by its terms in December 2023. Mr. Ganot was compensated at a rate of $20,833 per month for his services under the agreement for a total of $250,000.

We employ Ms. Ganot, one of our co-founders and the wife of Ilan Ganot, as our Vice President, Patient Advocacy. For 2023 and 2024, Ms. Ganot received annual salary and bonus payments of $360,380 and $374,795, respectively. For 2025 we expect Ms. Ganot will receive annual salary and bonus payments of less than $475,000 in the aggregate. In January 2023, February 2024 and February 2025, we granted (i) an option to purchase 23,950 shares of our common stock and RSUs with respect to 11,975 shares of our common stock, (ii) RSUs with respect to 19,444 shares of our common stock and (iii) RSUs with respect to 60,000 shares of our common stock, respectively, to Ms. Ganot relating to her service as our Vice President, Patient Advocacy. The options vest over a term of four years, with 25% of the original number of shares vesting on the first anniversary of the date of grant and 2.0833% of the original number of shares on the first day of each calendar month thereafter until the fourth such anniversary. The RSUs vest in equal annual installments over a term of four years from the date of grant.

For further information about the compensation received by Mr. Ganot in 2024, see "Non-Employee Director Compensation" in Item 11, "Executive Compensation" of this Annual Report on Form 10-K.

Arrangements with Carl Morris

Carl Morris served as our Chief Scientific Officer, Neuromuscular until July 14, 2023, or the Morris Separation Date. On May 22, 2023, we entered into an executive transition and separation agreement with Dr. Morris, or the Morris Transition and Separation Agreement. On the Morris Separation Date, we also entered into a consulting agreement, or the Morris Consulting Agreement, pursuant to which Dr. Morris assisted with the transition of his duties and provided other consulting and advisory services, as requested from time to time by us.

Pursuant to the Morris Transition Separation Agreement, Dr. Morris was entitled to receive all unpaid base salary earned through the Morris Separation Date, any amounts for accrued unused paid time off to which he was entitled through such

date in accordance with our policy, and reimbursement of any properly incurred unreimbursed business expenses incurred through such date. In addition, contingent on his providing a release of claims, Dr. Morris was entitled to (1) severance equal to a year's base salary, paid ratably over 12 months from the Morris Separation Date (an aggregate of $430,500), (2) provided he was eligible for and timely elected to continue receiving group health insurance under COBRA, payment by us of the portion of health coverage premiums that we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 12 months following the Morris Separation Date, and (3) $172,200, less applicable taxes and withholdings, which is a lump sum payment equal to 100% of his target bonus for 2023. In January 2023, we granted options to purchase 95,100 shares of our common stock and RSUs with respect to 47,555 shares of our common stock to Dr. Morris. The options vested with respect to 25% of the shares underlying the options on the first anniversary of the grant date and as to an additional 2.0833% of the original number of shares underlying the options monthly thereafter through the fourth anniversary of the grant date. The RSUs vest in equal annual installments over four years from the date of grant. In consideration for services under the Morris Consulting Agreement, Dr. Morris' then-outstanding equity awards continued to vest and remained exercisable in accordance with the applicable equity plans and award agreements.

Arrangements with Danforth Advisors, LLC

In November 2020, we entered into a consulting agreement with Danforth Advisors, LLC, or Danforth, an affiliate of Stephen DiPalma, our former interim chief financial officer who resigned in January 2023. Pursuant to the consulting agreement, Danforth provided us with the chief financial officer services of Mr. DiPalma, and continues to provide us other services, including financial planning, offering support and accounting services, in exchange for fees payable to Danforth based on hourly rates. We have paid Danforth approximately $2.8 million to date. The consulting agreement may be terminated by either party without cause upon 60 days' prior written notice to the other party and with cause upon 30 days' prior written notice to the other party.

Indemnification Agreements

We have entered into agreements to indemnify our directors and executive officers. These agreements require us, among other things, to indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such persons in any action or proceeding, including any action by or in our right, on account of any services undertaken by any such person on behalf of our company or that person's status as a member of our Board of Directors to the maximum extent allowed under Delaware law.

Policy for Approval of Related-Person Transactions

We have adopted a written related-person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification of any transaction, arrangement or relationship in which we are a participant, the amount involved exceeds $120,000 and one of our executive officers, directors, director nominees or 5% stockholders (or their immediate family members), each of whom we refer to as a "related person," has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a "related-person transaction," the related person must report the proposed related-person transaction to our general counsel. The policy calls for the proposed related-person transaction to be reviewed by and if deemed appropriate approved by, the audit committee of our Board of Directors. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the audit committee will review and, in its discretion, may ratify the related-person transaction. The policy also permits the chair of the audit committee to review, and if deemed appropriate approve, proposed related-person transactions that arise between audit committee meetings, subject to ratification by the audit committee at its next meeting. Any related-person transactions that are ongoing in nature will be reviewed annually.

A related-person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the audit committee after full disclosure of the related person's interest in the transaction. As appropriate for the circumstances, the committee will review and consider:

- the related person's interest in the related-person transaction;
- the approximate dollar amount involved in the related-person transaction;
- the approximate dollar amount of the related person's interest in the transaction without regard to the amount of any profit or loss;

- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the related-person transaction; and
- any other information regarding the related-person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The audit committee may approve or ratify the transaction only if the audit committee determines that, under all of the circumstances, the transaction is not inconsistent with our best interests. The audit committee may impose any conditions on the related-person transaction that it deems appropriate.

The policy provides that transactions involving compensation of executive officers will be reviewed and approved by the compensation committee of our Board of Directors in the manner specified in its charter.

Director Independence

Nasdaq rules require a majority of a listed company's board of directors to be comprised of independent directors. In addition, Nasdaq rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent. Audit committee members must also satisfy independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act, and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. The Nasdaq independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees and that neither the director nor any of his or her family members has engaged in various types of business dealings with us. In addition, under applicable Nasdaq rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.

Our Board of Directors has determined that all members of the Board of Directors, except Alexander Cumbo and Ilan Ganot, are independent directors, as defined under applicable Nasdaq rules. In March 2024, our Board of Directors also previously determined that Adam Koppel and Rajeev Shah, former directors who resigned from our board of directors, effective June 11, 2024, were independent directors, as defined under applicable Nasdaq rules. In making such determinations, our Board of Directors considered the relationships that each director has with our Company and all other facts and circumstances that our Board of Directors deemed relevant in determining his or her independence, including the beneficial ownership of our common stock by each director. Mr. Cumbo is not an independent director under applicable rules because he is our President and Chief Executive Officer. Mr. Ganot is not an independent director under applicable rules because he was our President and Chief Executive Officer until December 2, 2022.

Our Board of Directors has determined that the composition of our committees currently complies with all applicable independence requirements of Nasdaq and the rules and regulations of the SEC.

Item 14. Principal Accountant Fees and Services.

The following table represents aggregate fees billed to us by PricewaterhouseCoopers LLP, our independent registered public accounting firm, for the fiscal years ended December 31, 2024 and 2023:

	Year Ended December 31,	
	2024	2023
Audit fees	$ 752,500	$ 903,500
Audit-related fees	—	—
Tax fees	67,160	66,463
All other fees	2,125	2,125
Total	$ 821,785	$ 972,088

The services rendered by PricewaterhouseCoopers LLP in connection with the fees presented above were as follows:

Audit Fees

Audit fees consist of fees billed for professional services for the audit of our annual consolidated financial statements, the review of interim consolidated financial statements, and related services that are normally provided in connection with registration statements.

Tax Fees

Tax fees consist of fees for professional services related to tax compliance and consultations.

All Other Fees

All other fees include license fees for web-based accounting research tools.

Pre-Approval Policies

The audit committee has not adopted policies and procedures for the pre-approval of audit and non-audit services rendered by our independent registered public accounting firm and, consequently, all audit and non-audit services are pre-approved by the whole audit committee or the chair of the audit committee. All fees for the fiscal years ended December 31, 2024 and 2023 were so pre-approved.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(1) Financial Statements:

	Page
Report of Independent Registered Public Accounting Firm (PCAOB ID 238)	F-1
Consolidated Balance Sheets at December 31, 2024 and 2023	F-3
Consolidated Statements of Operations for the Years Ended December 31, 2024 and 2023	F-4
Consolidated Statements of Comprehensive Loss for the Years Ended December 31, 2024 and 2023	F-5
Consolidated Statements of Changes in Stockholders' Equity for the Years Ended December 31, 2024 and 2023	F-6
Consolidated Statements of Cash Flows for the Years Ended December 31, 2024 and 2023	F-7
Notes to Consolidated Financial Statements	F-9

(2) Financial Statement Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits. The following is a list of exhibits filed as part of this Annual Report on Form 10-K.

Exhibit Number	Description
2.1	Agreement and Plan of Merger, dated as of September 29, 2022, by and among Solid Biosciences Inc., Greenland Merger Sub LLC, AavantiBio, Inc. and, solely in his capacity as the Equityholder Representative, Doug Swirsky (incorporated by reference to Exhibit 2.1 to the Current Report on Form 8-K filed on September 30, 2022).
3.1	Certificate of Incorporation of Solid Biosciences Inc., as amended (incorporated herein by reference to Exhibit 4.1 to the Registrant's Registration Statement on Form S-8 (File No. 333-280116) on June 11, 2024).
3.2	Bylaws of Solid Biosciences Inc. (incorporated by reference to Exhibit 4.2 to the Registration Statement on Form S-8 filed on January 29, 2018).
4.1	Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registration Statement on Form S-1 filed on December 29, 2017).
4.2	Description of the Company's Securities Registered under Section 12 of the Exchange Act (incorporated by reference to Exhibit 4.2 to the Annual Report on Form 10-K filed on March 23, 2023).
10.1†	Amended and Restated Registration Rights Agreement dated March 29, 2017 by and among Solid Biosciences, LLC and certain investors (incorporated by reference to Exhibit 10.17 to the Registration Statement on Form S-1 filed on December 29, 2017).
10.2†	Solid Biosciences, LLC Amended and Restated Equity Incentive Plan and form of unit restriction agreement (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 29, 2018).
10.3†	Solid Biosciences Inc. 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 99.1 to the Registration Statement on Form S-8 filed on January 29, 2018).
10.4†	Form of Incentive Stock Option Agreement under 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 13, 2019).
10.5†	Form of Nonqualified Stock Option Agreement under 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 10.8 to the Annual Report on Form 10-K filed on March 13, 2019).
10.6†	Form of Restricted Stock Agreement under 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 10.9 to the Registration Statement on Form S-1 filed on December 29, 2017).
10.7#	Exclusive Patent License Agreement, dated as of October 16, 2015, by and between Solid GT, LLC and the University of Washington (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 13, 2024).
10.8#	License Agreement, dated as of October 15, 2015, by and between Solid GT, LLC and The Curators of the University of Missouri (incorporated by reference to Exhibit 10.8 to the Annual Report on Form 10-K filed on March 13, 2024).
10.9†	Form of Indemnification Agreement for Directors and Officers (incorporated by reference to Exhibit 10.16 to the Registration Statement on Form S-1 filed on December 29, 2017).
10.10*†	Summary of Non-Employee Director Compensation Program
10.11	Form of Pre-Funded Warrant to Purchase Common Stock (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on July 26, 2019).
10.12	Registration Rights Agreement, dated July 25, 2019, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed on July 26, 2019).
10.13†	Consulting Agreement, dated as of November 19, 2020, by and between Solid Biosciences Inc. and Danforth Advisors, LLC (incorporated by reference to Exhibit 10.24 to the Annual Report on Form 10-K filed on March 15, 2021).
10.14*†	Executive Chair Agreement, effective January 1, 2022, by and between Solid Biosciences Inc. and Ian F. Smith, as amended by the First Amendment to Executive Chair Agreement, effective September 30, 2022, Second Amendment to the Executive Chair Agreement, effective January 1, 2024, and Third Amendment to the Executive Chair Agreement, effective January 6, 2025.

10.15†	Amended and Restated 2020 Equity Incentive Plan, as amended (incorporated herein by reference to Exhibit 99.1 to the Registrant's Registration Statement on Form S-8 (File No. 333-280116) filed on June 11, 2024).
10.16†	Form of Stock Option Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on August 6, 2020).
10.17†	Form of Restricted Stock Unit Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.28 to the Annual Report on Form 10-K filed on March 14, 2022).
10.18	Stock Purchase Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.19#	Investor Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.3 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.20#	First Amendment, dated as of October 9, 2020, to the Exclusive Patent License by and between the Company and the University of Washington (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.21	Registration Rights Agreement, dated December 10, 2020, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on December 11, 2020).
10.22#	First Amendment, dated as of January 27, 2021, to the Exclusive Patent License by and between the Company and the Curators of the University of Missouri (incorporated by reference to Exhibit 10.36 to the Annual Report on Form 10-K filed on March 14, 2021).
10.23	Lease, dated June 15, 2021, between Solid Biosciences Inc. and Hood Park LLC (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on August 16, 2021).
10.24†	Amended and Restated 2021 Employee Stock Purchase Plan.(incorporated by reference to Exhibit 10.27 to the Annual Report on Form 10-K filed on March 13, 2024).
10.25†	Form of Nonstatutory Inducement Stock Option Agreement (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on August 16, 2021).
10.26†	Form of Inducement Restricted Stock Unit Award Agreement (incorporated by reference to Exhibit 99.5 to the Registration Statement on Form S-8 filed on August 16, 2021).
10.27	Amended and Restated Sales Agreement, dated March 13, 2024, by and between the Company and Jefferies LLC (incorporated by reference to Exhibit 10.30 to the Annual Report on Form 10-K filed on March 13, 2024).
10.28	Form of Parent Support Agreement (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on September 30, 2022).
10.29	Form of Support and Joinder Agreement (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on September 30, 2022).
10.30	Registration Rights Agreement, dated September 29, 2022, by and among Solid Biosciences Inc. and the persons party thereto (incorporated by reference to Exhibit 10.4 to the Current Report on Form 8-K filed on September 30, 2022).
10.31†	Employment Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Alexander Cumbo (incorporated by reference to Exhibit 10.5 to the Current Report on Form 8-K filed on September 30, 2022).
10.32†	Executive Transition and Separation Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Ilan Ganot (incorporated by reference to Exhibit 10.6 to the Current Report on Form 8-K filed on September 30, 2022).
10.33†	Consulting Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Ilan Ganot (incorporated by reference to Exhibit 10.7 to the Current Report on Form 8-K filed on September 30, 2022).
10.34†	Executive Transition and Separation Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Erin Powers Brennan (incorporated by reference to Exhibit 10.8 to the Current Report on Form 8-K filed on September 30, 2022).

10.35†	Consulting Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and Erin Powers Brennan (incorporated by reference to Exhibit 10.9 to the Current Report on Form 8-K filed on September 30, 2022).
10.36	Employment Agreement, dated September 29, 2022, by and between Solid Biosciences Inc. and David Tyronne Howton (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on May 11, 2023).
10.37	Employment Agreement, dated October 2, 2023, by and between Solid Biosciences Inc. and Gabriel Brooks (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on November, 8 2023).
10.38#	Standard Exclusive License Agreement With Know-How (Agreement No. A19110), dated as of March 17, 2020, by and between AavantiBio, Inc. and University of Florida Research Foundation, Incorporated, as amended on August 23, 2022 (incorporated by reference to Exhibit 10.42 to the Annual Report on Form 10-K filed as on March 23, 2023).
10.39#	Standard Exclusive License Agreement With Know-How (Agreement No. A19111), dated as of March 17, 2020, by and between AavantiBio, Inc. and University of Florida Research Foundation, Incorporated, as amended on May 25, 2021 and August 23, 2022 (incorporated by reference to Exhibit 10.43 to the Annual Report on Form 10-K filed on March 23, 2023).
10.40	Form on Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Current Report on Form 8-K filed on January 8, 2024).
10.41	Form of Registration Rights Agreement, dated January 8, 2024, by and among Solid Biosciences Inc. and the other parties thereto (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on January 8, 2024).
10.42†	2024 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 10.49 to the Annual Report on Form 10-K filed on March 13, 2024).
10.43†	Form of Non-Statutory Stock Option Agreement under the 2024 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 10.50 to the Annual Report on Form 10-K filed on March 13, 2024).
10.44†	Form of Restricted Stock Unit Agreement under the 2024 Inducement Stock Incentive Plan. Form of Restricted Stock Unit Agreement under the 2024 Inducement Stock Incentive Plan (incorporated by reference to Exhibit 10.51 to the Annual Report on Form 10-K filed on March 13, 2024).
10.45†	Form of Restricted Stock Unit Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.52 to the Annual Report on Form 10-K filed on March 13, 2024).
10.46##	Patent License Agreement, dated as of March 10, 2016, by and between Solid GT, LLC and the Regents of the University of Michigan.(incorporated by reference to Exhibit 10.53 to the Annual Report on Form 10-K filed on March 13, 2024).
10.47##	Life Technologies Cell Line License Agreement, dated as of November 20, 2016, by and between Solid Biosciences, LLC and Life Technologies Corporation (incorporated by reference to Exhibit 10.54 to the Annual Report on Form 10-K filed on March 13, 2024).
10.48##	License Agreement, dated as of June 23, 2016, by and between Solid GT, LLC and President and Fellows of Harvard College (incorporated by reference to Exhibit 10.55 to the Annual Report on Form 10-K filed on March 13, 2024).
10.49##	License Agreement, dated as of August 3, 2017, by and between Solid Biosciences, LLC and President and Fellows of Harvard College (incorporated by reference to Exhibit 10.56 to the Annual Report 10-K filed March 13, 2024).
10.50*#	Research, Collaboration & License Agreement, dated as of November 4, 2020, by and between the Trustees of the University of Pennsylvania and FA212 LLC, as amended
10.51*#	Asset Purchase Agreement, dated as of September 19, 2024, by and between Solid Biosciences Inc. and FA212 LLC
10.52	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Current Report on Form 8-K filed on February 18, 2025).
19.1*	Insider Trading Policy.
21.1*	Subsidiaries of Solid Biosciences Inc.

23.1*	Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm.
31.1*	Certification of Chief Executive Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Chief Financial Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Chief Executive Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2**	Certification of Chief Financial Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97.1	Dodd-Frank Compensation Recovery Policy (incorporated by reference to Exhibit 97.1 to the Annual Report on Form 10-K filed on March 13, 2024).
101.INS	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document With Embedded Linkbase Documents
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101).

* Filed herewith.
** Furnished herewith.
† Indicates management contract or compensatory plan.
Certain portions of this exhibit have been omitted because they are not material and contain information that the Registrant customarily and actually treats as private or confidential.
Filed with this Annual Report on Form 10-K solely for the purpose of transitioning this previously-filed exhibit, which is the subject of expiring confidential treatment orders, to the rules governing the filing of redacted exhibits under Regulation S-K Item 601(b)(10)(iv) pursuant to the SEC's CF Disclosure Guidance: Topic 7. Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

Item 16. Form 10-K Summary.

Not applicable.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended, the Registrant has duly caused this Report to be signed on its behalf by the undersigned, thereunto duly authorized**.**

SOLID BIOSCIENCES INC.

Date: March 6, 2025

By: /s/ Alexander Cumbo

Alexander Cumbo
President, Chief Executive Officer and Director
(Principal Executive Officer)

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, this Report has been signed below by the following persons on behalf of the Registrant in the capacities and on the dates indicated.

/s/ Alexander Cumbo Alexander Cumbo	President, Chief Executive Officer and Director *(Principal Executive Officer)*	March 6, 2025
/s/ Kevin Tan Kevin Tan	Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 6, 2025
/s/ Ian F. Smith Ian F. Smith	Executive Chairman of the Board	March 6, 2025
/s/ Martin Freed Martin Freed	Director	March 6, 2025
/s/ Ilan Ganot Ilan Ganot	Director	March 6, 2025
/s/ Clare Kahn Clare Kahn	Director	March 6, 2025
/s/ Georgia Keresty Georgia Keresty	Director	March 6, 2025
/s/ Sukumar Nagendran Sukumar Nagendran	Director	March 6, 2025
/s/ Adam Stone Adam Stone	Director	March 6, 2025
/s/ Lynne Sullivan Lynne Sullivan	Director	March 6, 2025

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Solid Biosciences, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Solid Biosciences, Inc. and its subsidiaries (the "Company") as of December 31, 2024 and 2023, and the related consolidated statements of operations, of comprehensive loss, of changes in stockholders' equity and of cash flows for the years then ended, including the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2024 and 2023, and the results of its operations and its cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audits to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matters

The critical audit matter communicated below is a matter arising from the current period audit of the consolidated financial statements that was communicated or required to be communicated to the audit committee and that relates to accounts or disclosures that are material to the consolidated financial communication of critical audit matters does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which it relates.

External Research and Development Costs

As described in Note 2 to the consolidated financial statements, research and development costs are expensed as incurred. Research and development expenses include salaries, equity-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company's research and development activities, including allocated facility-related expenses and external costs of outside vendors engaged to conduct both preclinical studies and clinical trials. The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancellable, and related payments are recorded as research and development expenses as incurred. Management records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, management analyzes progress of the studies, including the phase or completion of events, invoices received and

contracted costs. As disclosed by management, the majority of service providers invoice the Company in arrears for services performed, on a pre-determined schedule or when contractual milestones are met; however, some require advanced payments. The Company's research and development expense for the year ended December 31, 2024 was $96.4 million, a majority of which relates to external research and development costs.

The principal consideration for our determination that performing procedures relating to external research and development costs is a critical audit matter is a high degree of auditor effort in performing procedures related to the Company's external research and development costs.

Addressing the matter involved performing procedures and evaluating audit evidence in connection with forming our overall opinion on the consolidated financial statements. These procedures included, among others, testing external research and development costs on a sample basis by agreeing relevant information, including overall contract value, amounts incurred to date, and percentage of completion amounts to the (i) underlying agreements with outside vendors engaged to conduct preclinical studies and clinical trials, (ii) purchase orders, (iii) invoices received, (iv) underlying payments made for expenses incurred on the contracts, and (v) external confirmations or communications obtained by management from outside vendors.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
March 6, 2025

We have served as the Company's auditor since 2017.

SOLID BIOSCIENCES INC.
CONSOLIDATED BALANCE SHEETS
(In thousands, except share and per share data)

	December 31,	
	2024	**2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 80,235	$ 74,015
Available-for-sale securities	68,685	49,625
Prepaid expenses and other current assets	8,382	6,094
Total current assets	157,302	129,734
Non-current assets:		
Operating lease, right-of-use asset	24,295	26,539
Property and equipment, net	4,747	6,624
Other non-current assets	366	209
Restricted cash	1,952	1,833
Total non-current assets	31,360	35,205
Total assets	$ 188,662	$ 164,939
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,237	$ 2,032
Accrued expenses and other current liabilities	19,852	10,161
Operating lease liabilities	1,787	1,855
Finance lease liabilities	1,231	469
Derivative liabilities	3,150	—
Total current liabilities	30,257	14,517
Non-current liabilities:		
Operating lease liabilities, net of current portion	21,159	22,707
Finance lease obligations, net of current portion	—	1,234
Total non-current liabilities	21,159	23,941
Total liabilities	51,416	38,458
Commitments and Contingencies (Note 11)		
Stockholders' Equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized at December 31, 2024 and 2023; no shares issued and outstanding at December 31, 2024 and 2023	—	—
Common stock, $0.001 par value; 120,000,000 and 60,000,000 shares authorized at December 31, 2024 and 2023, respectively; 40,468,141 and 20,386,606 shares issued and outstanding at December 31, 2024 and 2023, respectively	40	20
Additional paid-in capital	920,609	785,199
Accumulated other comprehensive income	47	15
Accumulated deficit	(783,450)	(658,753)
Total stockholders' equity	137,246	126,481
Total liabilities and stockholders' equity	$ 188,662	$ 164,939

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except share and per share data)

	Year Ended December 31,	
	2024	**2023**
Operating expenses:		
Research and development	$ 96,431	$ 76,563
General and administrative	33,297	27,752
Restructuring expense	—	(63)
Total operating expenses	129,728	104,252
Loss from operations	(129,728)	(104,252)
Other income, net:		
Interest income	9,469	7,582
Interest expense	(340)	(440)
Change in fair value of derivative liabilities	(4,750)	—
Other income, net	652	1,095
Total other income, net	5,031	8,237
Net loss	$ (124,697)	$ (96,015)
Net loss per share, basic and diluted	$ (3.06)	$ (4.83)
Weighted average shares of common stock outstanding used to compute basic and diluted net loss per share	40,816,694	19,884,007

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands)

	Year Ended December 31,	
	2024	**2023**
Net loss	$ (124,697)	$ (96,015)
Other comprehensive income:		
Unrealized gain on available-for-sale securities	32	83
Comprehensive loss	$ (124,665)	$ (95,932)

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(In thousands, except share data)

	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at January 1, 2023	19,556,732	$ 20	$ 774,452	$ (68)	$ (562,738)	$ 211,666
Issuance of common stock in public offering, net of sales commissions of $76	602,030	—	2,974	—	—	2,974
Vesting of restricted stock units	183,951	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	43,893	—	148	—	—	148
Unrealized gain on available-for-sale securities	—	—	—	83	—	83
Equity-based compensation	—	—	7,625	—	—	7,625
Net loss	—	—	—	—	(96,015)	(96,015)
Balance at December 31, 2023	20,386,606	20	785,199	15	(658,753)	126,481
Issuance of common stock in private placement, net of issuance costs of $4,407	16,973,103	17	89,437	—	—	89,454
Issuance of pre-funded warrants in private placement, net of issuance costs of $704	—	—	14,293	—	—	14,293
Issuance of common stock in public offering, net of sales commissions of $471	2,212,937	2	18,367	—	—	18,369
Issuance of common stock in private placement for upfront consideration	364,990	—	2,000	—	—	2,000
Vesting of restricted stock units	335,981	1	—	—	—	1
Issuance of common stock under employee stock purchase plan	122,962	—	337	—	—	337
Exercise of common stock options	71,562	—	457	—	—	457
Unrealized gain on available-for-sale securities	—	—	—	32	—	32
Equity-based compensation	—	—	10,519	—	—	10,519
Net loss	—	—	—	—	(124,697)	(124,697)
Balance at December 31, 2024	40,468,141	$ 40	$ 920,609	$ 47	$ (783,450)	$ 137,246

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,	
	2024	**2023**
Operating activities:		
Net loss	$ (124,697)	$ (96,015)
Adjustments to reconcile net loss to net cash used in operating activities:		
Net amortization of discount on available-for-sale securities	(3,592)	(2,408)
Equity-based compensation expense	10,519	7,625
Depreciation and amortization expense	2,455	2,581
Non-cash lease expense	2,391	2,531
Non-cash acquired in-process research and development	3,400	—
Common stock issued as up-front payment for collaboration and license agreement	2,000	—
Change in fair value of derivative liabilities	4,750	—
Other	(88)	374
Changes in operating assets and liabilities:		
Prepaid expenses and other current and non-current assets	(2,445)	598
Accounts payable	2,205	(764)
Change in operating lease liability	(1,676)	(1,735)
Accrued expenses and other current and non-current liabilities	4,766	(6,967)
Net cash used in operating activities	(100,012)	(94,180)
Investing activities:		
Purchases of property and equipment	(654)	(1,515)
Proceeds from sale of property and equipment	5	—
Proceeds from sales and maturities of available-for-sale securities	188,900	128,632
Purchases of available-for-sale securities	(204,337)	(117,428)
Net cash (used in) provided by investing activities	(16,086)	9,689
Financing activities:		
Proceeds from issuance of common stock and pre-funded warrants in private placement	108,858	—
Payments of common stock and pre-funded warrants issuance costs in private placement	(5,111)	—
Proceeds from issuance of common stock in public offering, net of sales commissions	18,369	2,974
Proceeds from exercise of stock options	457	—
Employee stock purchase plan purchases	337	148
Payments of principal portion of finance lease obligations	(473)	
Net cash provided by financing activities	122,437	3,122
Net increase (decrease) in cash, cash equivalents and restricted cash	6,339	(81,369)
Cash, cash equivalents, and restricted cash at beginning of period	75,848	157,217
Cash, cash equivalents, and restricted cash at end of period	$ 82,187	$ 75,848

	Year Ended December 31,	
	2024	**2023**
Supplemental disclosure of cash flow information:		
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows for operating leases	$ 4,264	$ 4,501
Operating cash flows for finance leases	$ 469	$ 375
Financing cash flows for finance leases, cash paid for interest	$ 340	$ 435
Supplemental disclosure of non-cash investing and financing activities:		
Right-of-use assets obtained in exchange for operating lease liabilities	$ 407	$ 121
Decrease in right-of-use asset and lease liability due to lease termination	$ (261)	$ (252)
Decrease in property, plant and equipment due to asset exchange	$ —	$ (950)
Property and equipment purchases included in accounts payable and accruals	$ —	$ 76

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS (continued)
(In thousands)

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the consolidated balance sheets to the amounts reported in the consolidated statements of cash flows:

	December 31,	
	2024	2023
Cash and cash equivalents	$ 80,235	$ 74,015
Restricted cash, non-current	1,952	1,833
Total cash and cash equivalents, and restricted cash, as reported in the consolidated statements of cash flows	$ 82,187	$ 75,848

The accompanying notes are an integral part of these consolidated financial statements.

1. Nature of the Business and Basis of Presentation

Nature of Business

Solid Biosciences Inc. was organized in March 2013 under the name SOLID Ventures Management, LLC and operated as a Delaware limited liability company until immediately prior to the effectiveness of its registration statement on Form S-1 on January 25, 2018, at which time it completed a statutory corporate conversion into a Delaware corporation and changed its name to Solid Biosciences Inc. (the "Company"). On December 2, 2022, the Company completed its acquisition of AavantiBio, Inc. ("AavantiBio"), a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia ("FA") and rare cardiomyopathies (the "Acquisition"). Upon the consummation of the Acquisition, the Company acquired AavantiBio's gene therapy programs, AVB-202-TT for FA and AVB-401 for BAG3 mediated dilated cardiomyopathy, as well as additional assets for the treatment of other cardiac diseases, platform technologies and know-how related thereto. AavantiBio is a wholly owned subsidiary of the Company.

The Company is a life sciences company focused on advancing a portfolio of current and future gene therapy candidates (collectively, "Candidates"), including SGT-003 for the treatment of Duchenne muscular dystrophy ("Duchenne"), SGT-212 for the treatment of FA, SGT-501 for the treatment of catecholaminergic polymorphic ventricular tachycardia, SGT-601 for the treatment of TNNT2-mediated dilated cardiomyopathy, and additional assets for the treatment of genetic cardiac and other diseases, at different stages of development with varying levels of investment. The Company is advancing its diverse pipeline across rare neuromuscular and cardiac diseases, bringing together experts in science, technology, disease management and care. Patient-focused and founded by those directly impacted by Duchenne, the Company's mission is to improve the daily lives of patients living with these devastating diseases.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on licenses, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing to fund operations. Candidates currently under development will require significant additional research and development efforts, including extensive preclinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting capabilities.

The Company's Candidates are in development. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from, among others, other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, partners, and consultants.

Liquidity

The accompanying consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business. Through December 31, 2024, the Company has funded its operations primarily with the proceeds from the sale of redeemable preferred units and member units as well as the sale of common stock and pre-funded warrants to purchase shares of its common stock in private placements and the sale of common stock in its initial public offering and follow-on public offering in March 2021 and under its at-the-market sales agreement.

On January 11, 2024, the Company issued and sold in a private placement 16,973,103 shares of the Company's common stock at a price per share of $5.53 and, to one investor in lieu of shares of common stock, pre-funded warrants to purchase 2,712,478 shares of common stock at a price of $5.529 per pre-funded warrant (the "January 2024 Private Placement"). The Company received $103.7 million of net proceeds from the January 2024 Private Placement after deducting offering costs. No warrants were exercised during the year ended December 31, 2024.

During the year ended December 31, 2024, the Company issued 2,212,937 shares of its common stock, pursuant to the Company's "at-the-market offering" sales agreement, between the Company and Jefferies LLC (the "ATM Sales Agreement") for net proceeds of $18.4 million.

On February 19, 2025, the Company issued and sold 35,739,810 shares of its common stock at a price per share of $4.03 and, to certain investors in lieu of shares of common stock, pre-funded warrants to purchase 13,888,340 shares of common stock at a price of $4.029 per pre-funded warrant (the "February 2025 Offering"). The Company received approximately $187.5 million of net proceeds from the February 2025 Offering after deducting underwriting discounts and commissions and estimated offering costs.

In accordance with Accounting Standards Codification ("ASC") 205-40, *Going Concern*, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the financial statements are issued. As of December 31, 2024, the Company had an accumulated deficit of $783.5 million. During the years ended December 31, 2024 and 2023, the Company incurred net losses of $124.7 million and $96.0 million, respectively. The Company used $100.0 million of cash in operations for the year ended December 31, 2024. The Company expects to continue to generate operating losses in the foreseeable future. Based upon its current operating plan, the Company expects that its cash, cash equivalents and available-for-sale securities of $148.9 million, excluding restricted cash of $2.0 million, as of December 31, 2024, together with the net proceeds from the February 2025 Offering, will be sufficient to fund its operating expenses and capital expenditure requirements for at least twelve months from the date of issuance of these financial statements. However, the Company has based this estimate on assumptions that may prove to be wrong, and its operating plan may change as a result of many factors currently unknown to it. As a result, the Company could deplete its capital resources sooner than it currently expects. The Company expects to finance its future cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances or licensing arrangements. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its research and development programs, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects.

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). The accompanying consolidated financial statements include the accounts of the Company and its wholly owned or controlled subsidiaries. All intercompany accounts and transactions have been eliminated.

Reclassifications

Certain prior period amounts within cash flows from operating activities in the consolidated statement of cash flows have been reclassified to conform to the current period presentation. These reclassifications are not material and had no effect on the previously reported net cash used in operating activities.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of the Company's consolidated financial statements in conformity with GAAP requires management to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, estimates related to recognition of research and development expenses, equity-based compensation and derivative liabilities. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from the Company's estimates.

Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of 90 days or less at acquisition date to be cash equivalents.

Available-for-Sale Securities

Available-for-sale securities consist of investments with original maturities greater than 90 days at acquisition date. The Company has classified its investments with maturities beyond one year as short term, based on their highly liquid nature and

because such available-for-sale securities represent the investment of cash that is available for current operations.

The Company classifies all of its investments as available-for-sale securities. The Company's investments are measured and reported at fair value using quoted prices in active markets for similar securities. Unrealized gains and losses on available-for-sale debt securities are reported as a separate component of stockholders' equity. The cost of debt securities sold is determined on a specific identification basis, and realized gains and losses are included in other income, net within the consolidated statement of operations. Impairment of available-for-sale securities is evaluated considering numerous factors, and their relative significance varies depending on the situation. Factors considered include whether a decline in fair value below the amortized cost basis is due to credit-related factors or non-credit-related factors, the financial condition and near-term prospects of the issuer, and our intent and ability to hold the investment to allow for an anticipated recovery in fair value. A credit-related impairment is recognized as an allowance on the balance sheet with a corresponding adjustment to earnings. Any impairment that is not credit-related is recognized in other comprehensive income. No such adjustments were necessary during the periods presented.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash, cash equivalents and available-for-sale securities. Periodically, the Company maintains deposits in accredited financial institutions in excess of federally insured limits. The Company maintains each of its cash, cash equivalents and available-for-sale securities balances with high-quality and accredited financial institutions and accordingly, such funds are not exposed to significant credit risk. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities of its programs, including clinical and preclinical testing. These programs could be adversely affected by a significant interruption in the supply of such drug substance products.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and available-for-sale securities are carried at fair value, determined according to the fair value hierarchy described above. Derivative liabilities are recorded at fair value based on the probability weighted present value of the estimated cash flows pursuant to the contractual terms of each agreement. The derivative liabilities are remeasured quarterly with changes in fair value recorded in change in fair value of derivative liabilities in the consolidated statements of operations. See Note 4, *Fair Value of Financial Assets and Liabilities*, for additional information. The carrying values of the Company's accounts payable and accrued expenses and other current liabilities approximate their fair value due to the short-term nature of these liabilities.

Leases

At the inception of a contract, the Company determines if a contract meets the definition of a lease. A lease is a contract, or part of a contract, that conveys the right to control the use of identified property, plant, or equipment (an identified asset) for a period of time in exchange for consideration. The Company determines if the contract conveys the right to control the use of an identified asset for a period of time. The Company assesses throughout the period of use whether the Company has both of the following: (1) the right to obtain substantially all of the economic benefits from use of the identified asset and (2) the

right to direct the use of the identified asset. This determination is reassessed if the terms of the contract are changed. Leases are classified as operating or finance leases based on the terms of the lease agreement and certain characteristics of the identified asset. Right-of-use assets and lease liabilities are recognized at the lease commencement date based on the present value of the minimum future lease payments. Adjustments to the right-of-use asset may be required for items such as lease prepayments or incentives received. The Company's policy is to not record leases with an original term of twelve months or less on the consolidated balance sheets. The Company recognizes lease expense for these short-term leases on a straight-line basis over the lease term. Certain lease agreements include rental payments that are adjusted periodically for inflation or other variables. In addition to rent, the leases may require the Company to pay additional amounts for taxes, insurance, maintenance and other expenses, which are generally referred to as non-lease components. Such adjustments to rental payments and variable non-lease components are treated as variable lease payments and recognized in the period in which the obligation for these payments was incurred. Variable lease components and variable non-lease components are not measured as part of the right-of-use asset and liability. Only when lease components and their associated non-lease components are fixed are they accounted for as a single lease component and recognized as part of a right-of-use asset and liability. Total contract consideration is allocated to the combined fixed lease and non-lease components.

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful life of the asset. Laboratory equipment is depreciated over five years. Computer equipment is depreciated over three years. Computer software is depreciated over two years. Furniture and office equipment are depreciated over five years. Leasehold improvements are amortized over the shorter of the lease term or the estimated useful life of the related asset. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the net book value is written off and any resulting gain or loss is included in the consolidated statements of operations. Equipment under a finance lease is stated at fair value at the inception of the lease less accumulated depreciation and is depreciated over the remaining lease term or the estimated useful life of the equipment.

Impairment of Long-Lived Assets

Long-lived assets, comprised of property and equipment, to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows.

Exclusive Licenses

If a license granted in an arrangement is determined to be distinct from the other promises or performance obligations identified in the arrangement, which generally include research and development services, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license In assessing whether a license is distinct from the other promises, the Company considers relevant facts and circumstances of each arrangement, including the research and development capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from the license for its intended purpose without the receipt of the remaining promise, whether the value of the license is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the arrangement.

Research and Development Services

The promises under the Company's collaboration and license agreements generally include research and development services to be performed by the Company on behalf of the collaboration partner. For performance obligations that include research and development services, the Company generally recognizes revenue allocated to such performance obligations based on an appropriate measure of progress. The Company utilizes judgment to determine the appropriate method of measuring progress for purposes of recognizing revenue, which is generally an input measure, such as costs incurred.

Milestone Payments

At the inception of each arrangement that includes milestone payments based on certain events, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company, such as regulatory approvals, are not considered probable of being achieved until those approvals are received or the specified event occurs. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment. If a milestone or other variable consideration relates specifically to the Company's efforts to satisfy a single performance obligation or to a specific outcome from satisfying the performance obligation, the Company generally allocates the milestone amount entirely to that performance obligation once it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur.

Royalties

For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

Collaboration Revenue

The Company analyzes its collaboration arrangements to assess whether they are within the scope of ASC 808, *Collaborative Arrangements* ("ASC 808") to determine whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company first determines which elements of the collaboration are deemed to be within the scope of ASC 808 and those that are more reflective of a vendor-customer relationship and therefore within the scope of ASC 606, *Revenue from Contracts with Customers* ("ASC 606"). For elements of collaboration arrangements that are accounted for pursuant to ASC 808, an appropriate recognition method is determined and applied consistently, generally by analogy to ASC 606.

Costs Associated with License and Collaborative Arrangements

All costs associated with license and collaborative arrangements are expensed as incurred and recorded in research and development expense in the consolidated statements of operations.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses include salaries, equity-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company's research and development activities, including allocated facility-related expenses and external costs of outside vendors engaged to conduct both preclinical studies and clinical trials. Non-refundable pre-payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as

expense as the goods or services are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

The Company may in-license the rights to develop and commercialize product candidates. For each in-license transaction the Company evaluates whether it has acquired processes or activities along with inputs that would be sufficient to constitute a “business” as defined under GAAP. A “business” as defined under GAAP consists of inputs and processes applied to those inputs that have the ability to create outputs. Although businesses usually have outputs, outputs are not required for an integrated set of activities to qualify as a business. When the Company determines that it has not acquired sufficient processes or activities to constitute a business, any upfront payments, as well as milestone payments, are immediately expensed as acquired research and development in the period in which they are incurred.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Estimates based on available information are made in determining the accrual balances at the end of any reporting period. Actual results could differ from the Company's estimates; however, the Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred for filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Equity-Based Compensation

The Company measures all stock options and other stock-based awards granted to employees, directors and non-employees based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company applies the straight-line method of expense recognition to all awards with only service-based vesting conditions.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. In the first quarter of 2024, the Company determined that it has adequate historical stock price data to utilize solely its own stock for historical volatility purposes. The expected term of stock options with service-based vesting conditions and options granted to non-employees has been determined utilizing the “simplified” method for awards that qualify as “plain-vanilla” options. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. The Company utilized a 0% expected dividend yield in its determination of grant fair value based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future, if ever.

The fair values of restricted stock units and performance-based restricted stock units are measured at the grant date based on the closing price of the Company’s common stock on the date of grant. For restricted stock units, the fair value of the award is recognized on a straight-line basis over the requisite service periods. For performance-based restricted stock unit awards, which are subject to the achievement of performance milestones, the fair value is recognized as expense over the requisite service periods when the achievement of such performance milestones is determined to be probable. If a performance milestone is not determined to be probable or is not met, no equity-based compensation expense is recognized, and any previously recognized expense is reversed.

Forfeitures are recognized as a reduction of equity-based compensation expense as they occur.

The Company classifies stock-based compensation expense in its consolidated statements of operations in the same manner in which the award recipient’s payroll costs are classified or in which the award recipient’s service payments are classified.

The Company does not currently hold any treasury shares. Upon the exercise of stock options and the vesting of restricted stock units and performance stock units, the Company issues new shares of common stock and delivers them to the participant.

Income Taxes

Income taxes are accounted for under the asset and liability method. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are

expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. The Company records valuation allowances to reduce deferred income tax assets to the amount that is more likely than not to be realized. The Company determines whether it is more likely than not that a tax position will be sustained upon examination. If it is not more likely than not that a position will be sustained, no amount of benefit attributable to the position is recognized. The tax benefit to be recognized of any tax position that meets the more likely than not recognition threshold is calculated as the largest amount that is more than 50% likely of being realized upon resolution of the contingency.

Prior to January 25, 2018, the Company had not been subject to U.S. federal income taxes as the Company was organized as a limited liability company. As such, the taxable income or loss was passed through to and included in the tax returns of the members. Since January 25, 2018, the Company's income has since been subject to U.S. federal, state, local, and foreign income taxes and taxed at the prevailing corporate tax rates.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on developing treatments through gene therapy and other means for patients with neuromuscular and cardiac diseases. All of the Company's tangible assets are held in the United States. Please refer to Note 17, *Segment Reporting* for further information.

Comprehensive Loss

Comprehensive loss includes net loss, as well as other changes in stockholders' equity that result from transactions and economic events other than those with members. The Company's only element of other comprehensive income in all periods presented was unrealized gains from available-for-sale securities.

Net Loss per Share

The Company follows the two-class method when computing net loss per share, as the Company has issued shares that meet the definition of participating securities. The two-class method determines net loss per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net loss per share is computed by dividing the net loss by the weighted average number of shares of common stock and pre-funded warrants outstanding for the period. Diluted net loss is computed by adjusting net loss to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share is computed by dividing the diluted net loss by the weighted average number of shares of common stock and pre-funded warrants outstanding for the period, including potential dilutive shares of common stock assuming the dilutive effect of common stock equivalents.

Any preferred stock that the Company may issue in the future could entitle the holders of such shares to participate in dividends and not require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss, diluted net loss per share is the same as basic net loss per share, since dilutive shares of common stock are not assumed to have been issued if their effect is anti-dilutive. As of December 31, 2024 and 2023, there was no preferred stock issued or outstanding with any contractual rights.

Contingencies

Loss contingency provisions are recorded if the potential loss from any claim, asserted or unasserted, or legal proceeding, is considered probable and the amount can be reasonably estimated, or a range of loss can be determined. These accruals represent the Company's best estimate of probable loss. Disclosure also is provided when it is reasonably possible that a loss will be incurred or when it is reasonably possible that the amount of a loss will exceed the recorded provision. The Company reviews the status of each significant matter and assesses its potential financial exposure. Significant judgment is required in both the determination of probability and the determination as to whether an exposure is reasonably estimable. Because of uncertainties related to these matters, accruals are based only on the best information available at the time. As additional information becomes available, the Company reassesses the potential liability related to pending claims and may change its estimates. These changes in the estimates of the potential liabilities could have a material impact on the Company's consolidated results of operations and financial position.

Business Combinations

The Company's consolidated financial statements include the operations of acquired businesses after the completion of the acquisitions. The Company accounts for acquired businesses using the acquisition method of accounting. Application of this method of accounting requires that (i) identifiable assets acquired (including identifiable intangible assets) and liabilities assumed be measured and recognized at fair value as of the acquisition date, and (ii) the excess of the purchase price over the net fair value of identifiable assets acquired and liabilities assumed be recorded as goodwill. Acquired in-process research and development ("IPR&D") is recognized at fair value and initially characterized as an indefinite-lived intangible asset, irrespective of whether the acquired IPR&D has an alternative future use. Transaction costs are expensed as incurred. Amounts assigned to goodwill and other identifiable intangible assets are based on independent appraisals or internal estimates.

Asset Acquisitions

Acquisitions of assets or a group of assets that do not meet the definition of a business are accounted for as asset acquisitions using the cost accumulation method, whereby the cost of the acquisition, including certain transaction costs, is allocated to the assets acquired on the basis of relative fair values. No goodwill is recognized in an asset acquisition. Intangible assets that are acquired in an asset acquisition for use in research and development activities which have an alternative future use are capitalized as IPR&D. Acquired IPR&D which has no alternative future use is recognized as research and development expense at acquisition. Contingent milestone payments associated with asset acquisitions are recognized when probable and estimable. These amounts are expensed to research and development if there is no alternative future use associated with the asset or capitalized as an intangible asset if an alternative future use of the asset exists.

Recently Issued Accounting Pronouncements

In December 2023, the Financial Accounting Standards Board (the "FASB") issued Accounting Standards Update ("ASU") No. 2023-09, "Income Taxes (Topic 740): Improvements to Income Tax Disclosures." This ASU updates income tax disclosure requirements primarily by requiring specific categories and greater disaggregation within the rate reconciliation and disaggregation of income taxes paid by jurisdiction. This ASU is effective for annual periods beginning after December 15, 2024 and is applicable to the Company's fiscal year beginning January 1, 2025, with early application permitted. The Company is currently evaluating the impact of adopting this ASU on its consolidated financial statements and disclosures.

In November 2024, the FASB issued ASU No. 2024-03, "Income Statement - Reporting Comprehensive Income - Expense Disaggregation Disclosures (Sub-Topic 220-40)*:* Disaggregation of Income Statement Expenses," which requires additional disclosure of the nature of expenses included in the income statement. The standard requires disclosures about specific types of expenses included in the expense captions presented in the income statement. This ASU is effective for fiscal years beginning after December 15, 2026, and interim periods beginning after December 15, 2027, with early adoption permitted. The requirements should be applied on a prospective basis while retrospective application is permitted. The Company is currently evaluating the impact of adopting this ASU on its consolidated financial statements and disclosures.

Recently Adopted Accounting Pronouncements

On November 27, 2023, the FASB issued ASU No. 2023-07, "Segment Reporting (Topic 280): Improvements to Reportable Segment Disclosures.″ Among other new disclosure requirements, ASU 2023-07 requires companies to disclose significant segment expenses that are regularly provided to the chief operating decision maker. We adopted the standard effective December 31, 2024 and applied the disclosure requirements retrospectively to all prior periods presented in the financial statements. Adoption of ASU 2023-07 did not have an impact on the Company's consolidated financial position, results of operations, or cash flows.

3. FA212 Asset Acquisition

On September 19, 2024, the Company entered into an asset purchase agreement with FA212 LLC ("FA212") for the purchase of certain intellectual property, including patents and assigned licenses related to a pre-clinical drug candidate, which the Company now refers to as SGT-212, assigned manufacturing contracts as well as research and development materials such as manufactured materials and samples.

The Company paid FA212 an upfront payment of $1.0 million. Additionally, the Company agreed to pay FA212 development milestone payments of up to $34.0 million, cumulative sales milestone payments of up to $21.0 million, and

tiered royalties on net sales in the low-single-digits. The Company also assumed from FA212 contingent development milestone payments of up to $4.2 million, regulatory milestone payments of up to $13.0 million, cumulative sales milestone payments of up to $27.5 million, and tiered royalties on worldwide net sales in the mid-single digits payable to the University of Pennsylvania.

Certain development milestone payments to FA212 are payable in either cash, equity, or a combination of both, at the Company's discretion. Such contingent payments were determined to be derivative liabilities and were initially recorded at their fair value of $3.4 million, see Note 4, *Fair Value of Financial Assets and Liabilities*.

The Company determined that the FA212 agreement represented an asset acquisition of IPR&D assets with no alternative future use and recognized the aggregate acquisition cost of $5.1 million as research and development expense in the consolidated statement of operations for the year ended December 31, 2024, which included the upfront payment, initial recognition of the derivative liability and $0.7 million in transaction costs.

The Company submitted an investigational new drug application ("IND") for SGT-212 for the treatment of FA to the U.S. Food and Drug Administration ("FDA"), which was cleared in December 2024. Upon clearance of the IND, a $5.0 million development milestone became payable to FA212. The $5.0 million milestone liability payment is included in Accrued expenses and other current liabilities in the accompanying consolidated balance sheet as of December 31, 2024. The derivative liabilities for the remaining contingent payments were recorded at a fair value of $3.2 million as of December 31, 2024.

4. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company’s assets and liabilities that are measured at fair value on a recurring basis:

	December 31, 2024			
	Level 1	Level 2	Level 3	Total
Financial assets				
Cash equivalents:				
Money market funds	$ —	$ 18,088	$ —	$ 18,088
Treasury bills	—	17,434	—	17,434
Total cash equivalents	—	35,522	—	35,522
Available-for-sale securities (Note 5):				
Treasury bills	—	27,895	—	27,895
Government bonds	—	40,790	—	40,790
Total available-for-sale securities	—	68,685	—	68,685
Total financial assets	$ —	$ 104,207	$ —	$ 104,207
Financial liabilities				
Derivative liabilities	$ —	$ —	$ 3,150	$ 3,150
Total financial liabilities	$ —	$ —	$ 3,150	$ 3,150

	December 31, 2023			
	Level 1	Level 2	Level 3	Total
Financial assets				
Cash equivalents:				
Money market funds	$ —	$ 62,141	$ —	$ 62,141
Certificates of deposit	—	257	—	257
Total cash equivalents	—	62,398	—	62,398
Available-for-sale securities (Note 5):				
Treasury bills	—	49,625	—	49,625
Total available-for-sale securities	—	49,625	—	49,625
Total financial assets	$ —	$ 112,023	$ —	$ 112,023

As of December 31, 2024 and 2023, the fair values of the Company’s cash equivalents and available-for-sale securities were determined using Level 2 inputs.

The Company's accounts payable, accrued expenses, and other current liabilities approximated their estimated fair values due to the short-term nature of these financial instruments.

The Company estimated the fair value of the derivative liabilities by using a Monte Carlo simulation forecasting the timing and likelihood of certain development milestone events being achieved and discounting the probability adjusted payments using an appropriate discount rate based on market interest rates. The main assumptions when determining the fair value of the derivative liabilities are the timing of and probability of achieving certain milestones, the estimated volatility of the Company's common stock, and the discount rate. The estimated fair value presented is not necessarily indicative of an amount that could be realized in a current market exchange. The use of alternative inputs and estimation methodologies could have a material effect on these estimates of fair value.

Significant unobservable inputs for the derivative liabilities as of December 31, 2024 are as follows:

Derivative Liabilities	Valuation Technique	Unobservable Input	Range	Average
Development Milestones	Monte Carlo Simulation	Probability of achieving certain development milestones	28.0% - 70.0%	49.0%
		Volatility	85.0%	85.0%
		Discount Rate	4.2% - 4.3%	4.3%
		Timing of achieving certain development milestones	1.0 - 3.7 years	2.3 years

Significant unobservable inputs for the derivative liabilities as of September 19, 2024 (the date of entry into the agreement with FA212) are as follows:

Derivative Liabilities	Valuation Technique	Unobservable Input	Range	Average
Development Milestones	Monte Carlo Simulation	Probability of achieving certain development milestones	2.0% - 80.0%	28.6%
		Volatility	114.0%	114.0%
		Discount Rate	3.5% - 4.8%	3.8%
		Timing of achieving certain development milestones	0.3 to 5.5 years	2.4 years

The following table reflects the fair value of the Company's Level 3 derivative liabilities for the year ended December 31, 2024:

Fair value of derivative liabilities as of December 31, 2023	$ —
Fair value of derivative liabilities as of September 19, 2024	3,400
Change in fair value of derivative liabilities	4,750
Reclassification of accrued milestone payment to accrued expenses and other current liabilities	(5,000)
Fair value of derivative liabilities as of December 31, 2024	$ 3,150

During the years ended December 31, 2024 and 2023, there were no transfers between Level 1, Level 2 and Level 3.

5. Available-for-Sale Securities

A summary of the Company's available-for-sale securities is presented below:

	December 31, 2024			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Treasury bills maturing in one year or less	$ 27,879	$ 16	$ —	$ 27,895
Government bonds maturing in one year or less	40,759	31	—	$ 40,790
Total available-for-sale securities	$ 68,638	$ 47	$ —	$ 68,685

	December 31, 2023			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Treasury bills maturing in one year or less	$ 49,610	$ 15	$ —	$ 49,625
Total available-for-sale securities	$ 49,610	$ 15	$ —	$ 49,625

The weighted average contractual maturity of the Company's available-for-sale securities was approximately 0.5 years and 0.4 years as of December 31, 2024 and 2023, respectively.

6. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31,	
	2024	2023
Prepaid research and development expenses	$ 6,731	$ 3,980
Prepaid other and other current assets	1,651	2,114
Total	$ 8,382	$ 6,094

7. Property and Equipment

Property and equipment consisted of the following:

	December 31,	
	2024	2023
Furniture and fixtures	$ 936	$ 936
Laboratory equipment	14,404	16,137
Leasehold improvements	470	481
Computer equipment	764	842
Computer software	320	553
Construction in process	879	410
	17,773	19,359
Less accumulated depreciation	(13,026)	(12,735)
Total	$ 4,747	$ 6,624

Depreciation and amortization expense was $2.5 million and $2.6 million for the years ended December 31, 2024 and 2023, respectively. The Company recognized an impairment loss $0.4 million for the year ended December 31, 2023. No impairment loss was recognized during the year ended December 31, 2024.

8. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consisted of the following:

	December 31,	
	2024	2023
Accrued research and development expense	$ 7,268	$ 2,614
Accrued compensation	6,221	5,948
Accrued milestone payment (Note 3)	5,000	—
Accrued other and other current liabilities	1,363	1,599
Total	$ 19,852	$ 10,161

9. Equity-Based Compensation

Equity-Based Compensation Expense

The Company has classified equity-based compensation in its consolidated statements of operations as follows:

	Year Ended December 31,	
	2024	2023
Research and development expenses	$ 3,537	$ 3,094
General and administrative expenses	6,982	4,531
Total	$ 10,519	$ 7,625

Equity Incentive Plans

As of December 31, 2024, the Company's approved equity incentive plans include: the 2018 Omnibus Incentive Plan (the "2018 Plan"); Amended and Restated 2020 Equity Incentive Plan (the "2020 Plan"); the Amended and Restated 2021 Employee Stock Purchase Plan (the "ESPP"); and the 2024 Inducement Stock Incentive Plan (the "2024 Inducement Plan"). These plans are administered by the Board of Directors (the "Board") and permit the granting of stock options, stock appreciation rights, restricted stock, restricted stock units ("RSUs"), performance awards, and/or other stock-based or cash-based awards. Upon the adoption of the 2020 Plan, the Company no longer grants new equity awards under its 2018 Plan.

Amended and Restated 2020 Equity Incentive Plan

On June 11, 2024, the Company's stockholders approved an amendment to the 2020 Plan to increase the number of shares of common stock reserved for issuance under the plan by 2,000,000 shares. As of December 31, 2024, there were 2,000,796 stock options outstanding, 981,849 RSUs outstanding, 2,165,325 performance stock units outstanding, and 185,519 shares remained available for future issuance under the 2020 Plan.

Amended and Restated 2021 Employee Stock Purchase Plan

The ESPP was adopted by the Company's Board of Directors on April 14, 2021, approved by the stockholders on June 16, 2021, and became effective on June 16, 2021. The first offering period under the ESPP commenced on September 1, 2021.

On June 6, 2023, the Company's stockholders approved an amendment and restatement of the ESPP to (i) increase the number of shares of common stock reserved for issuance under the ESPP from 73,525 to 473,525 and (ii) provide for an annual increase to be added on the first day of each fiscal year, beginning with the fiscal year ending December 31, 2024 and ending with the fiscal year ending December 31, 2033, equal to the least of (a) 293,597 shares of common stock, (b) one percent (1%) of the outstanding shares of common stock on such date and (c) the number of shares of common stock determined by the Board of Directors. The Company's Board of Directors amended and restated the ESPP on November 12, 2023 to provide for 24-month offering periods.

The number of shares of the Company's common stock reserved for issuance under the ESPP is 677,391 shares. At December 31, 2024, 478,450 shares remained available for future issuance under the ESPP.

2024 Inducement Stock Incentive Plan

In March 2024, the Board approved the 2024 Inducement Plan, which provides for the reservation of 1,000,000 shares of common stock for equity granted as an inducement material to the individual's entering into employment with the Company and in accordance with the requirements of Nasdaq Stock Market Rule 5635(c)(4). As of December 31, 2024, there were 100,000 stock options outstanding, 300,067 RSUs outstanding, and 599,933 shares remained available for future issuance under the 2024 Inducement Plan.

Stock Options

The table below summarizes the activity with respect to stock options for the year ended December 31, 2024:

	Number of Options	Weighted Average Exercise Price	Weighted Average Remaining Contractual Life (in years)	Aggregate Intrinsic Value
Outstanding at January 1, 2024	2,259,672	$ 21.93		$ 863
Granted	1,219,522	7.81		
Exercised	(71,562)	6.39		
Expired	(44,521)	31.11		
Forfeitures	(435,174)	8.93		
Outstanding at December 31, 2024	2,927,937	$ 18.22	8.01	$ 193
Vested and expected to vest as of December 31, 2024	2,927,937	$ 18.22	8.01	$ 193
Exercisable at December 31, 2024	1,117,768	$ 35.27	6.79	$ 541

The assumptions used in the Black-Scholes option-pricing model for all stock options granted during each period presented are as follows:

	Year Ended December 31,	
	2024	2023
Common stock price	$5.76-$8.64	$2.15-$7.33
Expected volatility	119.7% - 128.7%	121.8% - 129.60%
Expected dividends	0.0%	0.0%
Expected term (in years)	5.31 - 6.25	5.31 - 6.25
Risk-free rate	3.7% - 4.4%	3.5% - 4.9%

The weighted average grant date fair values of stock options granted during the years ended December 31, 2024 and 2023 were $6.96 and $4.76, respectively.

The Company recognized $5.1 million and $5.3 million of equity-based compensation expense in connection with stock options during the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, there was $9.0 million of total unrecognized equity-based compensation cost related to unvested stock options. This cost is expected to be recognized over a weighted average period of 2.3 years. The intrinsic value of stock options exercised during the years ended December 31, 2024 and 2023 was $0.3 million and $0, respectively.

Restricted Stock Units

The following table summarizes activity with respect to RSUs during the year ended December 31, 2024:

	Units	Weighted-Average Grant Date Fair Value
Nonvested RSUs at January 1, 2024	877,181	$ 6.37
Granted	1,196,954	$ 7.94
Vested	(335,981)	$ 6.55
Forfeitures	(257,882)	$ 6.91
Nonvested RSUs at December 31, 2024	1,480,272	$ 7.50

The Company recognized $3.0 million and $2.2 million of equity-based compensation expense in connection with RSUs during the years ended December 31, 2024 and 2023, respectively. As of December 31, 2024, there was $9.0 million of total unrecognized equity-based compensation cost related to nonvested RSUs. This cost is expected to be recognized over a weighted average period of 2.9 years. The aggregate grant date fair value of RSUs vested during the years ended December 31, 2024 and 2023 was $2.2 million and $1.4 million, respectively.

Performance Stock Units

In June 2024, the Board approved a grant of performance-based restricted stock unit awards (“PSUs" or “Performance Awards”) to the Company’s executive team. Each PSU represents the contingent right to receive one share of the Company’s common stock. These Performance Awards provide for the vesting of 25% of the target number of underlying RSUs granted upon the achievement of each of four independent performance milestones predetermined by the Board (“Performance Milestones”), subject to the grantee’s continued service with the Company (the “Approval Conditions”).

The Performance Milestones are tied to the achievement of certain business objectives and are non-market and non-financial in nature. The Board will determine that all Approval Conditions have been satisfied and the number of units that will ultimately vest on the 2026 Evaluation Date, which will occur in the first quarter of 2026, and the 2027 Evaluation Date, which will occur in the first quarter of 2027. A maximum of 25% of the target number of RSUs may vest at the 2026 Evaluation Date and the percentage of the target number of RSUs allocable to any Performance Milestone that has not been achieved on or prior to the 2027 Evaluation Date shall be cancelled.

The Company granted 2,165,325 PSUs during the year ended December 31, 2024 with a weighted average grant date fair value of $7.51 per unit. During the year ended December 31, 2024, the Company recognized $2.2 million in expense for these grants.

As of December 31, 2024, there was $5.9 million of unrecognized equity-based compensation cost related to nonvested PSUs based on the achievement of all Performance Milestones. The Company expects to recognize this cost over a weighted average period of 1.9 years.

10. Leases

The Company has operating leases for laboratory and office space in Massachusetts and North Carolina.

In June 2021, the Company entered into a lease with Hood Park LLC (“Landlord”), pursuant to which the Company leases approximately 49,869 square feet of office, laboratory, research and development and manufacturing space located in Charlestown, Massachusetts (“Premises”). The Company relocated its corporate headquarters to the Premises in June 2022. The initial term of the lease commenced in June 2022 when the construction of the lessor assets was substantially completed and continues for a ten-year period, unless earlier terminated. The lease provides the Company with an option to extend the lease for an additional five-year term. The Company and the Landlord were each obligated to undertake certain improvements prior to the commencement of the lease, and significant improvements were completed as of June 2022. The monthly lease payment is approximately $0.3 million with annual escalation of approximately 3%. The lease includes a $10.2 million construction allowance which is considered a lease incentive and included within the right-of-use asset. The Company was required to post a customary letter of credit in the amount of $1.8 million, subject to decrease on a set schedule, as a security deposit pursuant to the lease.

On December 22, 2022, the Company entered into a sub-lease agreement (the "Sub-Lease") with Arkea Bio Corp ("Arkea"). The Sub-Lease permits use by Arkea of a portion of the space leased by the Company at 500 Rutherford Avenue in Charlestown, Massachusetts. The Company subleased approximately 12,461 square feet of the 49,869 square foot building interior space. The Sub-Lease term originally ended on February 28, 2025. The Sub-Lease was subsequently amended by the Sub-Lease Amendment, by and between Arkea and the Company, dated May 10, 2024, to increase the subleased area to approximately 13,714 square feet and extend the term to February 29, 2028.

As of December 31, 2024, minimum future lease payments for these operating and finance leases were as follows:

	Finance Leases	Operating Leases
2025	$ 651	$ 4,176
2026	—	4,284
2027	—	4,280
2028	—	4,364
Thereafter	—	17,118
Total	651	34,222
Less: Imputed Interest	(580)	11,276
Total Lease Liabilities	$ 1,231	$ 22,946

The following table shows the components of lease costs:

	Year Ended December 31,	
Lease cost	**2024**	**2023**
Operating lease cost	$ 4,961	$ 5,250
Finance lease cost:		
Amortization of right-of-use assets	441	476
Interest on lease liabilities	340	440
Sub-lease income	(963)	(1,094)
Total lease cost	$ 4,779	$ 5,072

Short-term lease and variable lease costs were not material for the years ended December 31, 2024 and 2023.

The following table shows the weighted average remaining lease term and weighted average discount rate of the Company's leases are as follows:

	Year Ended December 31,	
	2024	**2023**
Weighted-average remaining lease term (in years)		
Operating lease	7.6	8.5
Finance lease	0.8	1.8
Weighted-average discount rate		
Operating lease	10.8%	10.7%
Finance lease	22.8%	22.8%

11. Commitments and Contingencies

Letter of Credit

The Company had an outstanding letter of credit in the amount of $2.0 million and $1.8 million at December 31, 2024 and 2023, respectively, which was required as a condition of the Company's office and laboratory leases.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters, including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with its executive officers and members of its Board of Directors that require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as executive officers or directors. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnification arrangements.

The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2024 and 2023.

Legal Proceedings

The Company may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which the Company is focused. The Company is not aware of any material legal proceedings or claims as of December 31, 2024.

12. License Agreements

University of Washington License Agreement

In 2015, the Company entered into a license agreement with the University of Washington, acting through UW CoMotion, under which the Company obtained an exclusive, royalty-bearing, sublicensable, worldwide license under a patent application owned by the University of Washington relating to novel micro-dystrophins and all patents claiming priority to such patent to develop, manufacture, and commercialize products for use in the treatment of Duchenne and related disease indications caused by a lack of functional dystrophin. The Company has the right to grant sublicenses to third parties contingent upon written approval by the University of Washington prior to executing such sublicense, which approval may not be unreasonably withheld.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately $1.0 million upon the achievement of certain milestones. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017. In October 2020, the license agreement was amended such that the Company was required to pay the University of Washington $0.4 million in connection with the execution of the Collaboration Agreement. This payment was recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that the Company is required to pay an aggregate of approximately $3.4 million upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2024 and 2023. The Company must also pay royalties of a low single digit percentage of future sales by the Company and its sublicensees of products developed under the licensed patent rights. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to the Company and its sublicensees.

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. The Company may terminate the agreement at any time upon providing sixty days' written notice to the University of Washington. The University of Washington may terminate the agreement upon the Company's uncured, material breach of the agreement or if the Company enters into an insolvency-related event.

The Company recorded research and development expense in the amount of $0.1 million and $0 for the years ended December 31, 2024 and 2023, respectively, under the agreement.

The University of Missouri License Agreement

In 2015, the Company entered into a license agreement with the Curators of the University of Missouri (the "University of Missouri"), a public corporation of Missouri, under which the Company obtained an exclusive, royalty-bearing, sublicensable, worldwide license under certain patent and patent applications owned by the University of Missouri relating to a novel synthetic microdystrophin gene to make, sell and distribute products for use in the treatment of Duchenne and related disease indications resulting from a lack of functional dystrophin.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately $1.0 million upon the achievement of certain milestones for each product developed based on the licensed patents. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017.

Under the agreement, in the event the Company grants a sublicenses to another party, the Company is required to pay the University of Missouri a percentage of the consideration received. The license agreement was amended such that the Company was required to pay the University of Missouri $0.8 million in 2021 and $1.3 million in 2022. These amounts were recorded as a research and development expense in the fourth quarter of 2020. The Company paid $0.8 million in February 2021 and $1.3 million in February 2022. The license agreement was also amended such that the Company is required to pay an aggregate of approximately $1.9 million upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2024 and 2023. The Company must pay a royalty of a low single digit percentage of future sales or by its sublicensees of products developed using the licensed patents. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

Under the agreement, the Company granted the University of Missouri a non-exclusive, royalty-free, irrevocable, paid-up license, with the right to grant sublicenses to non-profit, academic, educational or governmental institutions, to practice and use improvements made by the Company using the licensed patent rights, solely for non-commercial research purposes.

The license agreement remains in effect until the expiration of the last-to-expire patent or the abandonment of the last to be abandoned patent application licensed under the agreement. The University of Missouri may terminate the agreement, or render the license granted thereunder non-exclusive, in individual countries if the Company's sublicensees fail to achieve

certain milestones. The Company may terminate the license agreement at any time upon providing six months' written notice to the University of Missouri and paying a termination fee. Each of the University of Missouri and the Company may also terminate the agreement for an uncured default or breach of the agreement by the other party. The Company's ability to cure such breach only applies to the first two notices of such breach provided by the University of Missouri, and thereafter, the University of Missouri may terminate the agreement for the Company's default or breach of the agreement upon thirty days' written notice without an opportunity to cure such default or breach.

The Company recorded research and development expense in the amount of $0.1 million and $0.1 million for the years ended December 31, 2024 and 2023, respectively, under the agreement.

University of Florida License Agreements

In 2020, AavantiBio entered into license agreements with the University of Florida Research Foundation, Inc. ("UFRF"). Broadly, the agreements relate to FA. The Company acquired the agreements in connection with the Acquisition. In 2023, the Company entered into an additional license agreement with UFRF. Under each agreement the Company obtained an exclusive, royalty-bearing, sublicensable, world-wide license to certain patents and patent applications and a royalty-bearing non-exclusive license under the know-how, to make, have made, use, see, have sold, import and export licensed products. UFRF retains the right to practice the patent rights and know-how for internal non-commercial research, including research sponsored by commercial entities, and educational purposes.

In consideration for the rights granted under each agreement, AavantiBio paid a one-time non-refundable license fee. In connection with each agreement, the Company is required to pay an annual license maintenance fee until the first commercial sale of a licensed product after which time a minimum annual royalty will replace such maintenance fees. Under each agreement, the Company is required to reimburse UFRF for costs incurred in applying for, prosecuting and maintaining patents, pay up to an aggregate of approximately $2.9 million upon the achievement of certain intellectual property, clinical and regulatory milestones for each licensed product under the agreement, and pay a low, single digit royalty on annual net sales by us and our sublicensees of licensed products on a licensed-product-by-licensed product basis. For any licensed product covered by both of these agreements, the Company is only obligated to make one payment for each milestone achieved and royalty payment due. Prior to the Acquisition, AavantiBio paid a single milestone fee related to the agreements of $0.1 million. Under each agreement, in the event the Company grants a sublicense to another party, the Company is required to pay UFRF a percentage of the consideration received.

Under each agreement, the Company has the right to grant sublicenses to third parties through multiple tiers, to the extent we are in compliance with our diligence obligations under the agreement and that sublicensee is subject to the terms of such agreement.

Under each agreement, the Company is obligated to use commercially reasonable efforts to develop and commercialize products covered by the licensed patent rights or know-how and to achieve certain regulatory and commercialization milestones within estimated time periods. Under the agreements entered in 2023 and 2024, the Company agreed to pay to UFRF cumulative sales milestones of up to $8.5 million and $27.0 million, respectively, upon achievement of specified commercial milestone events and tiered royalties on worldwide net sales in the low-to-mid-single digits.

Under each agreement, UFRF controls the prosecution and maintenance of the licensed patents in consultation with the Company and at the Company's expense. In countries in which the Company has not requested prosecution or maintenance of licensed patents in a particular country or jurisdiction, the license granted to such patent rights will terminate in such country or jurisdiction. The Company has the first right to enforce such licensed patents at our expense.

Each of the agreements terminates on a licensed product-by-licensed product basis on the later of: (i) expiration of the patent rights covering such licensed product or (ii) ten years from the first commercial sale of such licensed product. After five years, the Company may terminate an agreement for any reason giving advance written notice and reason for termination. UFRF may terminate an agreement for our uncured default or breach of the agreement. UFRF may immediately terminate an agreement if we bring or assist others in bringing a patent challenge against of the licensed patent rights. If UFRF sends the Company a written demand to terminate a sublicense agreement due to such sublicensee bringing or assisting a patent challenge, UFRF may terminate such agreement if we do not terminate the license with such sublicensee.

Maugeri License Agreement

On June 29, 2023, the Company entered into a license agreement (the "Maugeri License Agreement"), with ICS Maugeri S.p.A. SB ("Maugeri"), to focus on the development and commercialization of cardiac-related products by the Company based on Maugeri's inventions. Pursuant to the Maugeri License Agreement, Maugeri granted us an exclusive worldwide sublicensable license in certain Maugeri patent rights, including existing patent rights, and those in any improvements or know-how made in performance of the Maugeri License Agreement, and a non-exclusive worldwide sublicensable license in

certain Maugeri know-how, including existing know-how, and on any improvement thereto, in each case, subject to certain conditions, that is necessary or reasonably useful to develop the licensed products under the terms of the Maugeri License Agreement. The Company will conduct certain activities agreed to by the parties with respect to the research and development of licensed products. A condition precedent to the effectiveness of the Maugeri License Agreement was regulatory review in Italy, which was completed in the third quarter of 2023 and, upon the completion of the condition precedent, the Maugeri License Agreement became effective.

The Company paid Maugeri an upfront license fee of €1.5 million, which was recorded as research and development expense during the second quarter of 2023. Additionally, the Company agreed to cumulative developmental, regulatory, and commercial milestone payments of up to €15.0 million, cumulative sales milestone payments of up to €15.0 million, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits. There were no milestones achieved during the years ended December 31, 2024 and December 31, 2023.

The Maugeri License Agreement continues until the latest expiry of (i) the last valid claim (as defined in the Maugeri License Agreement), (ii) regulatory exclusivity, and (iii) all payment obligations. Either party may terminate the Maugeri License Agreement for the other party's uncured material breach. The Company may also terminate the Maugeri License Agreement in its sole discretion upon 60 days' prior written notice to Maugeri and payment of a fee.

Mayo Clinic Collaboration and License Agreement

In December 2024, the Company entered into a collaboration, patent and know-how license agreement with the Mayo Foundation for Medical Education and Research (the "Mayo") to further advance the Company's research and development efforts in the field of genetic therapies, particularly for rare and debilitating cardiac diseases.

As part of the collaboration, the Company will be providing manufactured viral materials and CMC know-how while the Mayo will be responsible for supporting all preclinical research through IND-enabling studies for six programs. The Company will then be responsible for the clinical development of programs chosen by it to develop. Under the terms of the collaboration, the Company and Mayo agreed to share intellectual property rights arising from the collaboration, with the Company retaining exclusive rights to commercialize any resulting therapies.

In connection with the agreement, the Company paid a one-time upfront payment of $0.6 million in cash and $2.0 million of shares of its common stock to Mayo, which were recorded to research and development expense in the fourth quarter of 2024 having no alternative future use. Additionally, Mayo is eligible for cumulative developmental milestones of $5.0 million, cumulative regulatory milestones of $2.0 million, and cumulative sales milestone payments of up to $18.0 million, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-high-single-digits for certain licensed products developed by the Company. The Company also agreed to pay an annual $0.6 million know-how access fee to Mayo. There were no milestones achieved during the year ended December 31, 2024.

The agreement also includes provisions for the potential sublicensing of certain intellectual property rights. The Company will also be responsible for reimbursing Mayo for costs incurred in the prosecution and maintenance of any patents resulting from the collaboration.

The collaboration remains in effect for the duration of the intellectual property rights associated with any therapies developed under the agreement. Either party may terminate the agreement with proper notice should specific terms be breached or should an insolvency-related event occur.

Other Agreements

The Company has committed to make potential future milestone payments and pay legal fees to third parties as part of licensing and development programs. The agreements generally required an upfront license fee and, under each agreement, the Company may be required to pay annual maintenance fees, royalties, milestone payments and sublicensing fees. Each license agreement is generally cancelable by the Company, given appropriate prior written notice. At December 31, 2024, potential future milestone payments under these agreements totaled an aggregate of $18.1 million. None of these milestones were assessed to be probable as of December 31, 2024.

13. Net Loss per Share

The following table sets forth the computation of the Company's basic and diluted net loss per share:

	Year Ended December 31,	
	2024	2023
Numerator:		
Net loss	$ (124,697)	$ (96,015)
Denominator:		
Shares used to compute net loss per share, basic and diluted		
Weighted average shares of common stock outstanding	38,178,327	19,884,007
Weighted average pre-funded warrants to purchase shares of common stock	2,638,367	—
Weighted average shares of common stock used to compute basic and diluted net loss per share	40,816,694	19,884,007
Net loss per share, basic and diluted	$ (3.06)	$ (4.83)

Included within weighted average shares of common stock outstanding for the year ended December 31, 2024 are 2,712,478 shares of common stock issuable upon the exercise of the pre-funded warrants as the pre-funded warrants are exercisable at any time for nominal consideration, and as such, the shares are considered outstanding for the purpose of calculating basic and diluted net loss per share. There were no pre-funded warrants issued and outstanding during the year ended December 31, 2024.

The outstanding securities presented below were excluded from the calculation of net loss per share because the inclusion of such securities would have been anti-dilutive due to the Company’s net loss per share during the periods presented.

	Year Ended December 31,	
	2024	2023
Options to purchase common stock	2,927,937	2,259,672
Nonvested restricted stock units	1,480,272	877,181
Nonvested performance stock units	2,165,325	—
Shares subject to employee stock purchase plan	281,640	—
Warrants	9,230	—
Total	6,864,404	3,136,853

14. Income Taxes

The Company recorded no tax benefit for the years ended December 31, 2024 and 2023 for the net operating losses incurred due to its uncertainty of realizing a benefit from those items.

A reconciliation of income taxes computed using the U.S. federal statutory rate to that reflected in operations as of December 31, 2024 and 2023 is as follows:

	December 31,	
	2024	2023
Income tax computed at federal statutory tax rate	21.0%	21.0%
State taxes, net of federal benefit	6.6%	6.6%
Permanent differences	(0.1)%	(0.5)%
Tax credits	6.3%	7.0%
Change in deferred tax rate	(2.5)%	0.2%
Stock compensation cancelations	(0.2)%	(1.0)%
Impact of ownership change	—	(108.5)%
Other items	0.2%	0.4%
Valuation allowance	(31.3)%	74.8%
Total	—	—

The Company established deferred tax assets and liabilities on identified book to tax temporary differences as of the date of conversion to a C-corporation. Deferred income taxes reflect the net tax effects of these temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's net deferred tax assets as of December 31, 2024 and 2023 are as follows:

	December 31,	
	2024	2023
Deferred tax assets:		
Tax loss carryforwards	$ 37,183	$ 16,622
Tax credit carryforwards	15,324	7,537
Deferred expenses	6,067	6,755
Accrued expenses	1,536	1,506
Stock compensation	9,986	8,496
Intangible assets	2,535	203
Capitalized R&D	63,087	57,133
Depreciation	—	672
Derivative liabilities	833	—
Other	53	80
Total deferred tax assets	136,604	99,004
Valuation allowance	(130,690)	(91,705)
Deferred tax liabilities:		
Right-of-use asset	(5,570)	(7,299)
Depreciation	(344)	—
Total deferred tax liabilities	(5,914)	(7,299)
Net deferred taxes	$ —	$ —

As of December 31, 2024, the Company had federal net operating loss carryforwards of $133.3 million which may be available to offset future taxable income and do not expire but are limited in their usage to an annual deduction equal to 80% of annual taxable income. In addition, as of December 31, 2024, the Company had state net operating loss carryforwards of approximately $146.5 million which may be available to offset future taxable income, of which $143.3 million begins to expire in 2032 and $3.2 million has unlimited carryforward. The Company also had federal and state tax credits of $13.1 million and $2.8 million, respectively, which may be used to offset future tax liability and each of which begin to expire in 2037.

The Company's ability to utilize these federal and state carryforwards may be limited in the future if the Company experiences an ownership change pursuant to Section 382 of the Internal Revenue Code of 1986, as amended (the "Internal Revenue Code"). Ownership changes, as defined in the Internal Revenue Code, including those resulting from the issuance of common stock in connection with the Company's public offerings, may limit the amount of net operating loss and tax credit carryforwards that can be utilized to offset future taxable income or tax liability. The Company completed a study in 2023 to assess whether a change of control had occurred under Section 382, and it was determined that all net operating loss carryforwards and credits generated before December 2, 2022 are limited. As a result, the carryforwards before the ownership change date of December 2, 2022 are not available for utilization and have been written off. The carryforwards as of December 31, 2024 were generated after the ownership change of December 2, 2022.

A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has evaluated the positive and negative evidence bearing upon the realizability of the deferred tax assets. The Company concluded, in accordance with the applicable accounting standards, that it is more likely than not that the Company will be unable to realize the benefit of its deferred tax assets. Accordingly, the Company has recorded a full valuation allowance against its deferred tax assets.

The following table presents the changes in the balance of the Company's deferred income tax asset valuation allowance:

	December 31,	
	2024	2023
Valuation allowance at beginning of year	$ 91,705	$ 163,566
Increases (decreases) recorded to income tax provision	38,985	(71,861)
Valuation allowance at end of year	$ 130,690	$ 91,705

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's C-Corporation tax years beginning with the year ended December 31, 2020 are open under statute. Any tax credit or net operating loss carryforward can be adjusted in future periods after the respective year of generation's statute of limitation has closed.

As of December 31, 2024 and 2023, the Company did not have unrecognized tax benefits. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of December 31, 2024 and 2023, no interest and penalties have been recorded.

15. Defined Contribution Plan

The Company has a defined contribution savings plan under Section 401(k) of the Internal Revenue Code. The plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis. Company contributions to the plan may be made at the discretion of the Company's Board of Directors. The Company made $0.6 million of contributions during each of the years ended December 31, 2024 and 2023.

16. Restructuring

In November 2022, the Company's Board of Directors approved a plan to reduce the Company's workforce by approximately 18 percent. These reductions were completed by December 5, 2022. This plan was designed to streamline the Company's operating structure following the Acquisition. The Company recorded a restructuring charge in the fourth quarter of 2022 of $5.7 million related to the reduction in force, consisting of severance and other employee termination benefits. The Company paid the remaining $0.3 million of this amount during the year ended December 31, 2024.

The following table shows the balance of accrued restructuring charges and the changes in the accrued amounts in connection with the associated restructuring plans for the years ended December 31, 2024 and 2023:

One-Time Employee Termination Benefits	November 2022
Accrued restructuring charges as of December 31, 2022	$ 3,921
Accrual recorded as a result of restructuring charges	—
Amounts paid during the period	(3,669)
Accrued restructuring charges as of December 31, 2023	$ 252
Accrual recorded as a result of restructuring charges	—
Amounts paid during the period	(252)
Accrued restructuring charges as of December 31, 2024	$ —

17. Segment Reporting

The Company has one reportable and one operating segment and manages its business activities primarily in North America and on a consolidated basis. The Company's singular focus is on developing treatments through gene therapy and other means for patients with neuromuscular and cardiac diseases. All of the Company's tangible assets are held in the United States.

The accounting policies of the Company are the same as those described in the summary of significant accounting policies.

The Company's chief operating decision maker ("CODM") is its chief executive officer. The CODM assesses performance for the Company and decides how to allocate resources based on net loss as reported on the consolidated statements of operations. The annual budgeting process is the primary mechanism used to make these decisions. The financial information also helps in making performance assessments using budgeted versus actual results.

The following table presents segment expenses, other segment items, and segment net loss for the periods presented:

	Year Ended December 31,	
	2024	2023
Segment Expenses:		
SGT-003	15,197	20,856
SGT-501	17,223	3,200
External R&D other	10,580	7,907
Internal R&D expense[1]	21,705	21,798
External G&A expense	20,122	19,635
Internal G&A expense[1]	12,223	10,911
Other segment items[2]	32,678	19,945
Other income, net	(5,031)	(8,237)
Consolidated net loss	(124,697)	(96,015)

[1] Internal expenses consisted primarily of payroll and related costs, temporary services, and travel and entertainment.
[2] Other segment items primarily included equity-based compensation expense, other program costs, and depreciation and amortization expense

The measure of segment assets is reported on the balance sheet as total consolidated assets.

18. Subsequent Events

On February 19, 2025, the Company issued and sold 35,739,810 shares of its common stock at a price of $4.03 per share, and, to certain investors in lieu of shares of common stock, pre-funded warrants to purchase 13,888,340 shares of its common stock at a price of $4.029 per pre-funded warrant, in the February 2025 Offering. The Company received approximately $187.5 million of aggregate net proceeds from the February 2025 Offering, after deducting underwriting discounts and commissions and estimated offering costs.

Following the FDA's clearance of the Company's IND application for SGT-212 for the treatment of FA, a $5.0 million development milestone became payable to FA212. On February 28, 2025, the Company made the first milestone payment in the form of 975,496 shares of its common stock.

Exhibit 10.10

SOLID BIOSCIENCES INC.

NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

The non-employee directors of Solid Biosciences Inc. (the "Company") shall receive the following compensation for their service as members of the Board of Directors of the Company (the "Board").

Director Compensation

Our goal is to provide compensation for our non-employee directors in a manner that enables us to attract and retain outstanding director candidates and reflects the substantial time commitment necessary to oversee the Company's affairs. We also seek to align the interests of our directors and our stockholders, and we have chosen to do so by compensating our non-employee directors with a mix of cash and equity-based compensation.

Cash Compensation

The fees that will be paid to our non-employee directors for service on the Board, and for service on each committee of the Board on which the director is then a member, and the fees that will be paid to the chairperson of each committee of the Board will be as follows:

	Member Annual Fee	**Chairperson Incremental Annual Fee**
Board of Directors	$40,000	$35,000
Audit Committee	$7,500	$7,500
Clinical Committee	$7,500	$7,500
Compensation Committee	$5,000	$5,000
Nominating and Corporate Governance Committee	$4,000	$4,000

The foregoing fees will be payable in arrears in equal semi-annual installments not later than the 15th business day following the end of the second and fourth calendar quarters, provided that the amount of such payment will be prorated for any portion of such semi-annual period that the director is not serving on the Board, on such committee or in such position, and no fee shall be payable in respect of any period prior to the completion of our initial public offering.

Equity Compensation

Initial Stock Option Grants. Upon initial election to our Board, each non-employee director will be granted, automatically and without the need for any further action by the Board, an initial equity award of an option to purchase 68,800 shares of our common stock. The option shall have a term of ten years from the grant date and shall vest and become exercisable as to 1/3 of the shares underlying such option on each anniversary of the grant date until the third anniversary of the grant date, subject to the director's continued service as a director through each applicable vesting date. The vesting shall accelerate as to 100% of the shares upon a change in control of the Company. The exercise price shall be the closing price of our common stock on the date of grant.

Annual Stock Option Grants. Each non-employee director who has served as a member of our Board for at least six months prior to the date of our annual meeting of stockholders for a particular year will be granted, automatically and without the need for any further action by the Board, an equity award on the date of such annual meeting of an option to purchase 34,400 shares of our common stock. The option shall have a term of ten years from the grant date and shall vest and become exercisable in full on the earlier to occur of the one-year anniversary of the grant date and immediately prior to our first annual meeting of stockholders occurring after the grant date, subject, in each case, to the director's continued service as a director through the applicable vesting date. The vesting shall accelerate as to 100% of the shares upon a change in control of the Company. The exercise price shall be the closing price of our common stock on the date of grant.

The foregoing share amounts shall be adjusted in the event of certain changes in the capital structure or business of the Company, including any stock split, reverse stock split, stock dividend, combination or reclassification of shares, recapitalization, spin off or other similar change in the Company's capitalization and any non-cash dividend or distribution to holders of common stock, in each case in accordance with the terms of our 2020 Equity Incentive Plan, or any successor plan.

The initial stock option awards and the annual stock option awards shall be subject to the terms and conditions of our Amended and Restated 2020 Equity Incentive Plan, as amended, or any successor plan, and the terms of the option agreements entered into with each director in connection with such awards.

Expenses

Upon presentation of documentation of such expenses reasonably satisfactory to the Company, each non-employee director shall be reimbursed for his or her reasonable out-of-pocket business expenses incurred in connection with the performance of their duties as directors, including travel expenses in connection with their attendance in-person at Board and committee meetings.

Effective: June 16, 2020, as amended June 7, 2022, December 13, 2022 and June 11, 2024.

EXECUTIVE CHAIR AGREEMENT

This Executive Chair Agreement (together with Exhibit A, the "Agreement"), effective January 1, 2022 (the "Effective Date"), is by and between Ian F. Smith, an individual having an address at 45 Commonwealth Avenue, Unit 3, Boston, MA 02116 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 141 Portland Street, Fifth Floor, Cambridge, MA 02139.

WHEREAS, Executive Chair is currently serving as a member and chairman of the Board of Directors of the Company (the "Board");

WHEREAS, Executive Chair has also been providing advisory services to Company pursuant to a consulting agreement between the Parties entered into on January 4, 2021 (the "Prior Agreement") and both parties acknowledge and agree that, as of the Effective Date of this Agreement, the agreed upon services under the Prior Agreement have been completed;

WHEREAS, from and after the close of business on the Effective Date, Executive Chair shall no longer serve under the Prior Agreement, but shall be retained as the Executive Chairman pursuant to the terms and conditions set forth herein; and

NOW THEREFORE, in consideration of the promises and mutual covenants herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, Executive Chair and the Company agree as follows:

1. Services. Company retains Executive Chair, and Executive Chair agrees to provide, part-time advisory services to Company, consistent with his position as Executive Chairman of the Board, and as more fully set forth on Exhibit A attached hereto (the "Services"). It is understood and agreed that the Executive Chair will not be an executive officer of the Company and that Chief Executive Officer of the Company will continue to be the Company's principal executive officer and will continue to report directly to the board of directors and to the Executive Chairman.

2. Compensation. As consideration for Services provided under this Agreement, the Company shall grant, subject to approval of Company's Board, to Executive Chair an option to purchase 194,500 shares of the Company's Common Stock ("Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on January 3, 2021 (the "Grant Date"), and 97,250 restricted stock units (the "RSUs" and together with the RSUs, the "Awards"). The Awards will vest in equal quarterly installments with the first installment vesting three months from the Grant Date and the final installment vesting date on the date that is 12 months from the Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of a change in control of the Company and on terms consistent with board of director equity award agreements, all unvested Awards will accelerate and vest in full.

3. Work-Made-for Hire.

(a) All right, title and interest in any and all writings, ideas, inventions, know-how, designs, improvements or other property created during Executive Chair's advisory relationship relating in any way to the assets, business or operations of the Company, constituting copyrights, patents, trademarks, service marks and related rights or other forms of proprietary rights or information (regardless of whether any such copyrights, patents, trademarks and service marks or other rights have or may be registered) that are created, adapted or improved by Executive Chair (whether alone or in conjunction with any other person or employee), and all material created during Executive Chair's advisory relationship that includes any of the foregoing (collectively, "Covered Material"), shall be owned by the Company and to the extent that it includes copyrightable subject matter, shall be deemed a work made for hire for the Company within the meaning of the United States Copyright Act of 1976 and for all other purposes. If any Covered Material is deemed not to be work made for hire, such Covered Material is hereby assigned by Executive Chair to the Company and Executive Chair shall not have or claim to have, under this Agreement or otherwise, any right, title or interest of any kind or nature whatsoever in such Covered Material.

(b) The Company shall have the right to apply for and obtain registrations in the United States Copyright Office and the United States Patent and Trademark Office, in its own or its designee's name, of its rights in any or all of the Covered Material. If for any reason the rights in any Covered Material are registered, or applied to be registered, in Executive Chair's name, Executive Chair shall assign in writing such application or registration to the Company and hereby authorizes and appoints the Company its agent for the purpose of recording such assignment.

(c) Upon request of Company, Executive Chair shall execute, acknowledge and deliver all applications, assignments or other instruments; make or cause to be made all rightful oaths; testify in all legal proceedings; communicate all known facts which relate to such works, copyrights, inventions, ideas, discoveries, designs and improvements; perform all lawful acts and otherwise render all such assistance as the Company may deem necessary to protect the Company's interest therein including any assistance which the Company shall deem necessary in connection with any proceeding or litigation involving the same. The Company shall reimburse Executive Chair for all reasonable out-of-pocket costs, incurred by Executive Chair in rendering any such assistance requested by the Company pursuant to this Section. In the event that Executive Chair should fail or refuse to execute such documents within a reasonable time, Executive Chair appoints Company as attorney to execute and deliver any such documents on Executive Chair's behalf.

4. Nondisclosure. Executive Chair agrees to (a) hold the Confidential Information (as defined below) in confidence; (b) not disclose any Confidential Information to any third party without the prior written consent of Company; (c) not use the Confidential Information for any purpose except solely as required to perform the Services; and (d) treat Confidential Information with no less than a reasonable degree of care. For purposes of this Agreement, the term "Confidential Information" means any and all information and derivative information, in whatever form or medium, including oral information, concerning or relating to the Company or information of any third party that the Company is under an obligation to keep confidential or that is maintained by the Company as confidential, including, without limitation, intellectual property of the Company, such as, but not limited to, Covered Material, patent applications, copyrights, copyright

applications, and trade secrets; information regarding or resulting from research and development activities performed by or on behalf of the Company and other projects (such as, but not limited to, preclinical and clinical data, design details and specifications, engineering information, and works in process); and business and financial information (such as, but not limited to, current, future, and proposed products and services, financial information and models, information relating to procurement requirements, purchasing, manufacturing, investors, customer lists, customers, suppliers, facilities, product plans, product ideas, business strategies, marketing or business plans, financial or personnel matters, investors, employees, business and contractual relationships, business forecasts, sales, strategies, operations, policies, procedures, commercialization capabilities, and information regarding third parties). Notwithstanding the foregoing, Confidential Information does not include information that Executive Chair can demonstrate: (a) is publicly known and generally available in the public domain other than in consequence of improper action by any person; or (b) was acquired by Executive Chair free and clear of any duty of confidentiality or restricted use and without improper action by the transferor of such information or any other person. Executive Chair shall keep confidential all matters entrusted to Executive Chair by or on behalf of the Company and shall not use or attempt to use any Confidential Information except as may be required in the ordinary course of performing Executive Chair's duties as an advisor to the Company, and Executive Chair shall not use any Confidential Information in any manner that may injure or cause loss or may be calculated to injure or cause loss to the Company, whether directly or indirectly. Nothing in this Agreement prohibits Executive Chair from reporting possible violations of federal or state law or regulations to any governmental agency or entity or self-regulatory institution, including but not limited to the Equal Employment Opportunity Commission, the National Labor Relations Board, the Department of Justice, the Securities and Exchange Commission, Congress, and any Inspector General, or making other disclosures that are protected under the whistleblower provisions of federal or state law or regulation. Prior authorization of the Company shall not be required to make any such reports or disclosures and Executive Chair is not required to notify the Company that Executive Chair has made such reports or disclosures.

5. Compliance with Laws. Executive Chair agrees to provide the Services to Company and its affiliates in accordance with all applicable laws and regulations and the highest professional standards. Executive Chair represents and warrants that Executive Chair has not been, and is not under consideration to be (a) debarred from providing services pursuant to Section 306 of the United States Federal Food Drug and Cosmetic Act, 21 U.S.C. § 335a; (b) excluded, debarred or suspended from, or otherwise ineligible to participate in, any federal or state health care program or federal procurement or non-procurement programs (as that term is defined in 42 U.S.C. § 1320a-7b(f)); (c) disqualified by any government or regulatory agencies from performing specific services, and is not subject to a pending disqualification proceeding; or (d) convicted of a criminal offense related to the provision of health care items or services, or under investigation or subject to any such action that is pending.

6. Compliance with Obligations to Third Parties. Executive Chair represents and warrants to Company that the terms of this Agreement and Executive Chair's performance of Services do not and will not conflict with any of Executive Chair's obligations to any third parties. Executive Chair represents that Executive Chair has not brought and will not bring with Executive Chair to Company or use in the performance of Services any equipment, funds, space, personnel, facilities, confidential information, trade secrets or other resources of any third party which are not generally available to the public, nor will Executive Chair take any other action that would result in a third-party asserting ownership of, or other rights in, any Covered Material. If Executive Chair is a faculty member at or employee of a university or hospital or another organization or company ("Institution"), Executive Chair represents and warrants that Executive Chair is not prohibited by any applicable policy of such Institution, including without limitation any policy addressing conflicts of interest or intellectual property, from performing external consulting services and assigning rights to intellectual property arising from such services to Company or a third party. To the extent Executive Chair is subject to any policy of his/her employer that requires approval of agreements governing external consulting services, Executive Chair represents that such approval has been given and covenants that such approval will be obtained prior to entering into any amendment to this Agreement requiring such approval.

7. Non-hire of Employees and Consultants. During the Term and for a one year period thereafter, Executive Chair will not (except on the Company's behalf), directly or indirectly, alone or as a consultant, partner, officer, director, employee, joint venturer, lender or stockholder of any entity, employ, hire, retain, attempt to employ, hire or retain, or knowingly permit any company or business organization by which Executive Chair is employed or which is directly or indirectly controlled by Executive Chair to employ, hire or retain, any Company employee or consultant, or any such person whose employment or consultancy with the Company has terminated within six months prior to or after Executive Chair's departure from the Company.

8. Nonsolicitation of Employees and Consultants. During the Term and for a one year period thereafter, Executive Chair will not (except on the Company's behalf), directly or indirectly, alone or as a consultant, partner, officer, director, employee, joint venturer, lender or stockholder of any entity, in any manner seek to solicit or induce any Company employee or consultant, or any such person whose employment or consultancy with the Company has terminated within six months prior to or after Executive Chair's departure from the Company, to leave his or her employment or consultancy with the Company, or assist in the recruitment or hiring of any such person.

9. Nondisparagement. Executive Chair shall not at any time, whether during or after the Term, regardless of the reason for such termination, make to any person or entity disparaging, critical or otherwise detrimental comments of a business or personal nature relating to the Company or its personnel.

10. Use of Name. Executive Chair will not use the name, logo, trade name, service mark, or trademark, or any simulation, abbreviation, or adaptation of same, or the name of Company or any of its affiliates for publicity, promotion, or other uses without Company's prior written consent.

11. Company Property. Executive Chair shall not make, use or permit to be used any Company Property otherwise than for the benefit of the Company. The term "Company Property" shall include all Confidential Information; the Company's records, files and data; all Company computers, cellular telephones, personal digital assistants, credit and/or calling cards, keys, access cards and the like; and all other documentation or materials of any nature and in any form, whether written, printed, electronic or in digital format or otherwise, relating to any matter within the scope of the business of the Company or concerning any of its dealings or affairs and any other Company property in Executive Chair's possession, custody or control. Executive Chair further agrees that upon expiration or termination of this Agreement, Executive Chair shall immediately return all Company Property to Company. Executive Chair acknowledges and agree that all Company Property shall be and remain the sole and exclusive property of the Company. Immediately upon the expiration or termination of this Agreement, Executive Chair shall deliver all Company Property in Executive Chair's possession, and all copies thereof, to the Company.

12. Term & Termination. The term of this Agreement will commence on the Effective Date and will expire on December 31, 2022, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term"). Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to Executive Chair and Executive Chair may terminate this agreement, for any reason or no reason, on fifteen (15) days prior written notice to the Company.

13. Notices. All notices required or permitted under this Agreement must be in writing and must be given by directing the notice to the address for the receiving party set forth in this Agreement or at such other address as the receiving party may specify in writing under this procedure. Notices to Company will be marked "Attention: Chief Legal Officer." All notices must be given (i) by personal delivery, with receipt acknowledged, (ii) by prepaid certified or registered mail, return receipt requested, or (iii) by prepaid recognized next business day delivery service. Notices will be effective upon receipt or at a later date stated in the notice.

14. Remedies. Executive Chair agrees that (i) Company may be irreparably injured by a breach of this Agreement by Executive Chair; (ii) money damages would not be an adequate remedy for any such breach; (iii) as a remedy for any such breach Company will be entitled to seek equitable relief, including injunctive relief and specific performance, without being required by Executive Chair to post a bond; and (iv) such remedy will not be the exclusive remedy for any breach of this Agreement.

15. Independent Contractor. Executive Chair is an independent contractor and not an employee of the Company. Executive Chair shall be responsible for all taxes arising from compensation and other amounts paid under this Agreement. Neither federal, state or local income tax, nor payroll tax of any kind, shall be withheld or paid by the Company on Executive Chair's behalf. Executive Chair will not be eligible for, and shall not participate in, any employee pension, health, welfare, or other fringe benefit plan of the Company.

16. Waiver; Amendments. Any waiver by the Company of a breach of any provision of this Agreement shall not operate or be construed as a waiver of any subsequent breach of such provision or any other provision hereof. In addition, any amendment to or modification of this Agreement or any waiver of any provision hereof must be agreed to in writing by both parties.

17.Severability. Executive Chair agrees that each provision and the subparts of each provision herein shall be treated as separate and independent clauses, and the unenforceability of any one clause shall in no way impair the enforceability of any of the other clauses of the Agreement. Moreover, if one or more of the provisions contained in this Agreement shall for any reason be held to be excessively broad as to scope, activity, subject or otherwise so as to be unenforceable at law, such provision or provisions shall be construed by the appropriate judicial body by limiting or reducing it or them, so as to be enforceable to the maximum extent compatible with the applicable law as it shall then appear. Executive Chair hereby further agrees that the language of all parts of this Agreement shall in all cases be construed as a whole according to its fair meaning and not strictly for or against either of the parties.

18.Survival. Executive Chair's obligations under Section 3 through 22 of this Agreement shall survive the expiration or termination of this Agreement and shall be binding upon Executive Chair's successors, heirs, executors, administrators and legal representatives.

19.Assignment. The Company shall have the right to assign this Agreement to its successors and assigns, and all covenants and agreements hereunder shall inure to the benefit of and be enforceable by said successors or assigns. Executive Chair may not assign this Agreement. In no event will Executive Chair assign or delegate responsibility for actual performance of the Services to any third party.

20.Governing Law. This Agreement shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

21.Entire Agreement. This Agreement sets forth the complete, sole and entire agreement between the parties with respect to the subject matter herein and supersedes any and all other agreements, negotiations, discussions, proposals, or understandings, whether oral or written, previously entered into, discussed or considered by the parties.

22.Counterparts. This Agreement may be executed in any number of counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument. A facsimile or portable document format (".pdf") copy of this Agreement, including the signature pages, will be deemed an original.

IN WITNESS HEREOF, Executive Chair has executed this Agreement as of the last date written below.

SOLID BIOSCIENCES INC.	**IAN F. SMITH**
By: /s/ Ilan Ganot	/s/ Ian Smith
Name: Ilan Ganot	
Title: President & Chief Executive Officer	

EXHIBIT A

Executive Chairman Services

The Company requires, in addition to the traditional duties as Chairman of the Board, that the Executive Chair, as Executive Chairman, will be available to perform the duties customarily related to this function, including:

- Provide mentorship and guidance to the Company's Chief Executive Officer (who will remain the Company's principal executive officer) and senior leadership team on operational and strategic matters; Chief Executive Officer will continue to report to the Board, as well as to the Executive Chair
- Act as a liaison between the Company's senior management and the Board;
- Participate in regular meetings with the Chief Executive Officer and Chief Operating Officer to further advance the Company's strategic initiatives;
- Make himself available as reasonably requested by the Board or Executive Chair officers of the Company to fulfill such other duties as may be reasonably requested, consistent with his status as the Executive Chair Chairman; and
- Devote between thirty-five to fifty (35-50) hours per month, including one (1) full day per week, on a schedule and at a location(s) mutually agreed between Executive Chair and the Company's Chief Executive Officer, to include flexible availability to the Company's Chief Executive Officer and Chief Operating Officer

FIRST AMENDMENT TO

EXECUTIVE CHAIR AGREEMENT

THIS FIRST AMENDMENT TO EXECUTIVE CHAIR AGREEMENT is effective September 30, 2022 (the "First Amendment Date"), is by and between Ian F. Smith, an individual having an address at 104 Meadowbrook Road, Weston, MA, 02493 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 500 Rutherford Ave., Charlestown, MA 02129.

WHEREAS, Executive Chair and the Company are parties to that certain Executive Chair Agreement ("Agreement"), effective as of January 1, 2022 (the "Effective Date"), and

WHEREAS, the parties desire to amend the Agreement in manner set forth below.

NOW, THEREFORE, in consideration of the premises and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties agree as follows:

1. Compensation. Section 2 of the Agreement is amended by inserting a new Section 2A as follows: and restated in its entirety as follows:

"2A. Compensation – 2023. As consideration for Services provided under this Agreement from and after January 1, 2023, the Company shall, subject to approval of Company's Board, grant to Executive Chair equity awards (the "2023 Awards") with an aggregate total Black Scholes value of $700,000, consisting of (i) 50% stock options (the "2023 Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on January 2, 2023 (the "2023 Grant Date") and (ii) 50% restricted stock units ("2023 RSUs"), in each case adjusted, as applicable and appropriate, to reflect fully the effect of any reclassification, stock split, reverse split, stock dividend, reorganization, recapitalization or other like change with respect to Company Common Stock occurring (or for which a record date is established) after the date of this Amendment and prior to the 2023 Grant Date. The 2023 Awards will vest in equal quarterly installments with the first installment vesting three months from the 2023 Grant Date and the final installment vesting date on the date that is 12 months from the 2023 Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of (i) the early termination of this Agreement prior to the expiration of the Term and/or (ii) a change in control of the Company and on terms consistent with board of director equity award agreements, all unvested 2023 Awards will accelerate and vest in full."

2. Term. Section 12 of the Agreement is amended and restated in its entirety as follows:

"Term & Termination. The term of this Agreement will commence on the Effective Date and will expire on December 31, 2023, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term").Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to Executive Chair and Executive Chair may terminate this agreement, for any reason or no reason, on fifteen (15) days prior written notice to the Company. The Board shall periodically review the ongoing benefit of the Executive Chair position to the Company and may elect to terminate the Agreement in accordance with the preceding sentence following any such review."

3. Governing Law. This First Amendment shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

4. Entire Agreement. Except as amended hereby, the Agreement shall remain in full force and effect as originally written..

[Signature page follows]

IN WITNESS WHEREOF, the parties have executed this First Amendment to Executive Chair Agreement as of the date first above written.

SOLID BIOSCIENCES INC.

By: /s/ Ilan Ganot

Name: Ilan Ganot

Title: Chief Executive Officer

IAN F. SMITH

/s/ Ian F. Smith

SECOND AMENDMENT TO

EXECUTIVE CHAIR AGREEMENT

THIS SECOND AMENDMENT TO EXECUTIVE CHAIR AGREEMENT ("Amendment") is effective January 1, 2024 (the "Second Amendment Date"), is by and between Ian F. Smith, an individual having an address at 104 Meadowbrook Road, Weston, MA, 02493 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 500 Rutherford Ave., Charlestown, MA 02129.

WHEREAS, Executive Chair and the Company are parties to that certain Executive Chair Agreement ("Agreement"), effective as of January 1, 2022 (the "Effective Date"), and

WHEREAS, the parties desire to amend the Agreement in manner set forth below.

NOW, THEREFORE, in consideration of the premises and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties agree as follows:

1.Definitions. Each capitalized term used but not defined herein shall have the meaning ascribed to such term in the Agreement.

2.Compensation. Section 2 of the Agreement is amended by inserting a new Section 2B as follows:
"2A. Compensation – 2023. As consideration for Services provided under this Agreement from and after January 1, 2024, the Company shall, subject to approval of Company's Board, grant to Executive Chair equity awards (the "2024 Awards") with an aggregate total Black Scholes value of $150,000, consisting of (i) 50% stock options (the "2024 Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on 2024 Grant Date and (ii) 50% restricted stock units ("2024 RSUs"), in each case adjusted, as applicable and appropriate, to reflect fully the effect of any reclassification, stock split, reverse split, stock dividend, reorganization, recapitalization or other like change with respect to Company Common Stock occurring (or for which a record date is established) after the date of this Amendment and prior to the Grant Date. The 2024 Awards will vest in equal quarterly installments with the first installment vesting three months from the 2024 Grant Date and the final installment vesting date on the date that is 12 months from the 2024 Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of (i) the early termination of this Agreement prior to the expiration of the Term and/or (ii) a change in control of the Company and on terms consistent with Board of Director equity award agreements, all unvested 2024 Awards will accelerate and vest in full. The Company's external compensation consultants evaluated the cash rate and found the rates to be appropriate. The consultants were present for the official recommendation to the Compensation Committee."

3.Term. Section 12 of the Agreement is amended and restated Second in its entirety as follows:

"Term & Termination. The term of this Agreement will commence on the Effective Date and will expire on December 31, 2024, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term"). Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to Executive Chair, and Executive Chair may terminate this agreement, for any reason or no reason, on fifteen (15) days prior written notice to the Company. The Board shall, on or about June 1, 2024, and periodically thereafter, review the ongoing benefit of the Executive Chair position to the Company and may elect to terminate the Agreement in accordance with the preceding sentence following any such review in accordance with the preceding sentence.

3. Remaining Provisions of the Agreement. Except as provided herein, each of the other provisions of the Agreement shall remain in full force and effect.

4. References. Upon the effectiveness of this Amendment, on and after the date hereof, each reference in the Agreement to "this Agreement," "hereunder," "hereof," "herein," or words of like important shall mean and be reference to the Agreement, as amended hereby.

5. Governing Law. This Second Amendment shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

6. Counterparts. This Amendment may be executed in one or mor counterparts, including by electronic (PDF) transmission, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

7. Entire Agreement. This Agreement, as supplemented and modified by this Amendment contains the Parties' entire agreement, and there are no promises or conditions in any other agreement between the Parties, whether oral or written, concerning the subject matter hereof. As supplemented and modified by this Amendment., the agreement supersedes any prior written or oral agreements between the Parties concerning the subject matter hereof.

[Signature page follows]

IN WITNESS WHEREOF, the parties have executed this Amendment No. 2 to Executive Chair Agreement as of the date first above written.

SOLID BIOSCIENCES INC.

By: /s/ Bo Cumbo

Name: Bo Cumbo

Title: President and CEO

Date: 12/31/2023

IAN F. SMITH

/s/ Ian F. Smith

Date: 01/01/2024

THIRD AMENDMENT TO

EXECUTIVE CHAIR AGREEMENT

THIS THIRD AMENDMENT TO EXECUTIVE CHAIR AGREEMENT ("Amendment") is effective January 1, 2025 (the "Third Amendment Date"), is by and between Ian F. Smith, an individual having an address at 104 Meadowbrook Road, Weston, MA, 02493 (the "Executive Chair"), and Solid Biosciences Inc., a Delaware corporation (together with its affiliates, the "Company"), having an address at 500 Rutherford Ave., 3rd Floor, Charlestown, MA 02129.

WHEREAS, the Executive Chair and the Company are parties to that certain Executive Chair Agreement ("Agreement"), effective as of January 1, 2022 and amended to date by that certain Third Amendment to Executive Chair Agreement (the "Effective Date"), and

WHEREAS, the parties desire to further amend the Agreement in the manner set forth below.

NOW, THEREFORE, in consideration of the premises and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties agree

as follows:

1. Definitions. Each capitalized term used but not defined herein shall have the meaning ascribed to such term in the Agreement.

2. Compensation. Section 2 of the Agreement is amended by inserting a new Section 2B as follows:
"2B. Compensation – 2025. As consideration for Services provided under this Agreement from and after January 1, 2025, the Company shall, subject to approval of Company's Board, grant to Executive Chair equity awards (the "2025 Awards") with an aggregate total Black Scholes value of $150,000.00 consisting of (i) 50% stock options (the "2025 Stock Options") at an exercise price per share equal to the closing price of the Company's Common Stock on the 2025 Grant Date and (ii) 50% restricted stock units ("2025 RSUs"), in each case adjusted, as applicable and appropriate, to reflect fully the effect of any reclassification, stock split, reverse split, stock dividend, reorganization, recapitalization or other like change with respect to Company Common Stock occurring (or for which a record date is established) after the date of this Amendment and prior to the Grant Date. The 2025 Awards will vest in equal quarterly installments with the first installment vesting three months from the 2025 Grant Date and the final installment vesting date on the date that is 12 months from the 2025 Grant Date, subject to continued services as a Director of the Company. Additionally, in the event of (i) the early termination of this Agreement prior to the expiration of the Term and/or (ii) a change in control of the Company and on terms consistent with Board of Director equity award agreements, all unvested 2025 Awards will accelerate and vest in full. The Company's external compensation consultants evaluated the cash rate and the conversion rate and found the rates to be appropriate. The consultants were present for the official compensation recommendation to the Compensation Committee."

3. Term. Section 12 of the Agreement is amended and restated Third in its entirety as follows:

"Term & Termination. The term of this Agreement will commence on the Effective Date. It will expire on December 31, 2025, unless extended by mutual written agreement of the parties or earlier terminated in accordance with this Section 12 (the "Term"). Notwithstanding anything to the contrary herein, the Company may terminate this Agreement, for any or no reason, on written notice to the Executive Chair, and the Executive Chair may terminate this Agreement, for any reason or no reason, on fifteen (15) days' prior written notice to the Company. The Board shall, on or about June 1, 2025, and periodically thereafter, review the ongoing benefit of the Executive Chair position to the Company and may elect to terminate the Agreement following any such review in accordance with the preceding sentence.

3. Remaining Provisions of the Agreement. Except as provided herein, each of the other provisions of the Agreement shall remain in full force and effect.

4. References. Upon the effectiveness of this Amendment, on and after the date hereof, each reference in the Agreement to "this Agreement," "hereunder," "hereof," "herein," or words of like import shall mean and be a reference to the Agreement, as amended hereby.

5. Governing Law. This Third Amendment shall be governed by and construed in accordance with the laws of the Commonwealth of Massachusetts, without giving effect to the principles of conflicts of laws of such state. The parties agree to submit to the exclusive jurisdiction of the state and federal courts located in the Commonwealth of Massachusetts and waive any defense of inconvenient forum to the maintenance of any action or proceeding in such courts.

6. Counterparts. This Amendment may be executed in one or more counterparts, including by electronic (PDF) transmission, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

7. Entire Agreement. The Agreement, as supplemented and modified by this Amendment, contains the Parties' entire agreement, and there are no promises or conditions in any other agreement between the Parties, whether oral or written, concerning the subject matter hereof. As supplemented and modified by this Amendment, the Agreement supersedes any prior written or oral agreements between the Parties concerning the subject matter hereof.

[Signature page follows]

IN WITNESS WHEREOF, the parties have executed this Amendment No. 2 to the Agreement below.

SOLID BIOSCIENCES INC.

By:/s/ Bo Cumbo
Name: Bo Cumbo
Title: President and CEO
Date: 01/06/2025

By: /s/ Ian F. Smith

Name: Ian F. Smith

Date:01/06/2025

Exhibit 10.50

Execution Version

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

RESEARCH, COLLABORATION & LICENSE AGREEMENT

DATED AS OF NOVEMBER 4, 2020

BY AND BETWEEN

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

AND

FA212 LLC

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I

UNIVERSITY OF PENNSYLVANIA

RESEARCH, COLLABORATION & LICENSE AGREEMENT

This Research, Collaboration & License Agreement (this "**Agreement**") is dated as of November 4, 2020 (the "**Effective Date**") by and between The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation ("**Penn**"), and FA212 LLC, a limited liability company organized under the laws of the State of Delaware ("**Licensee**"). Penn and Licensee may be referred to herein as a "**Party**" or, collectively, as "**Parties**".

RECITALS:

WHEREAS, Penn, through Dr. James Wilson ("**Dr. Wilson**") and the Wilson Lab (as defined below), have technology and expertise in the research and development of gene therapy products; and

WHEREAS, the Research Program contemplated by this Agreement is of mutual interest to Licensee and Penn and furthers the educational, scholarship and research objectives of Penn as a nonprofit, tax-exempt, educational Penn, and may benefit Licensee and Penn through the creation or discovery of new inventions and the development and commercialization of FA Gene Therapy Products (as defined below).

NOW, THEREFORE, in consideration of the various promises and undertakings set forth herein, the Parties agree as follows:

ARTICLE 1
DEFINITIONS

Unless otherwise specifically provided herein, the following terms shall have the following meanings:

1.1 "**AAV**" means adeno-associated virus.

1.2 "**Affiliate**" means a Person that controls, is controlled by or is under common control with a Party, but only for so long as such control exists. For the purposes of this Section 1.2, the word "control" (including, with correlative meaning, the terms "controlled by" or "under the common control with") means the actual power, either directly or indirectly through one or more intermediaries, to direct the management and policies of such Person or entity, whether by the ownership of more than fifty percent (50%) of the voting stock of such entity, or by contract or otherwise.

1.3 "**BLA**" means (a) a Biologics License Application as defined in the FD&C Act and the regulations promulgated thereunder, (b) a Marketing Authorization Application in the European Union ("**MAA**"), or (c) any equivalent or comparable application, registration or certification in any other country or region.

1.4 "**Budget**" means the initial budget set forth in Exhibit D attached hereto, as the same may be amended from time to time with written approval of the Parties in accordance with Section 2.6.1(e).

1.5 "**Calendar Quarter**" means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 and December 31 of each Calendar Year.

1.6 "**Calendar Year**" means each successive period of twelve (12) months commencing on January 1 and ending on December 31.

1.7 "**Clinical Trial**" means a human clinical study in which a FA Gene Therapy Product is administered to human subjects that is designed to (a) determine the safety, tolerability, pharmacological activity or pharmacokinetics in healthy individuals or the target patient population and support continued testing; (b) determine the safety and efficacy in the target patient population and to generate sufficient data to permit commencement of Pivotal Clinical Trials; (c) establish that the product is safe and efficacious for its intended use and to provide adequate data and information for inclusion in product labeling, which trial is intended to support Regulatory Approval, including all Pivotal Clinical Trials; or (d) to support continued Regulatory Approval, including clinical trials conducted as required by post-marketing commitments.

1.8 "**Combination Product**" means a FA Gene Therapy Product that is delivered with one or more additional active ingredients and/or other patentable items (which are themselves not products for a single price) incident to the administration of any such FA Gene Therapy Product, including companion diagnostics, in each such case when any of the foregoing are co-formulated, co- packaged or sold under one pricing scheme (whether payment of such price is paid to the same or to more than one seller).

1.9 " **Commercially Reasonable Efforts**" means the efforts and resources that [**]. Without limiting the foregoing, Commercially Reasonable Efforts requires, with respect to such obligations, that the Party [**].

1.10 "**Compulsory License**" means a compulsory license under the Penn Patent Rights obtained by a Third Party through the order, decree, or grant of a competent Governmental Authority or court, authorizing such Third Party to develop, make, have made, use, sell, offer to sell or import a FA Gene Therapy Product in any country.

1.11 "**Confidential Information**" of a Party, means (a) information relating to the business, operations or products of a Party or any of its Affiliates, including any Know-How, that such Party discloses to the other Party under this Agreement, or otherwise becomes known to the other Party by virtue of this Agreement, and (b) the terms, but not the existence of this Agreement.

1.12 "**Control**" or "**Controlled**" means, with respect to intellectual property rights, that a Party or one of its Affiliates owns or has a license or sublicense to such intellectual property rights and has the ability to provide, grant a license or sublicense to, or assign its right, title and interest in and to, such intellectual property rights as provided for in the Agreement

without violating the terms of any other agreement or other arrangement with any Third Party.

1.13 "**Development Candidate**" means (a) a Drug Candidate designated as a "Development Candidate" by the JSC or (b) any Drug Candidate with respect to which the pharmacokinetic and toxicology studies identified in the Research Plan are commenced with the intent to satisfy the requirements for filing an IND with respect to such Drug Candidate.

1.14 "**Development Transition Point**" or "**DTP**" means the date on which Penn delivers to Licensee a report setting forth the Research Results sufficient for filing of an IND for a FA Gene Therapy Product.

1.15 "**Drug Candidate**" means a FA Gene Therapy Product candidate discovered, conceived or developed by Penn, or delivered to Licensee, in each case, under the Research Plan during the Research Term. For clarity, "Drug Candidate" includes only those FA Gene Therapy Products that are worked on pursuant to the Research Plan.

1.16 "**EMA**" means the European Medicines Agency and any successor agency thereto.

1.17 "**FA Gene Therapy Product**" means a Product consisting of the AAV vector [**] are listed on the attached Exhibit A. In the event that there is an amendment to the foregoing sequences during the Research Program, the foregoing FA Gene Therapy Product definition shall be revised through a written amendment to include such revised sequences.

1.18 "**FDA**" means the United States Food and Drug Administration and any successor agency thereto.

1.19 "**FD&C Act**" means the United States Federal Food, Drug and Cosmetic Act, as amended.

1.20 "**Field of Use**" shall mean all uses in humans. For clarity, [**].

1.21 **"FIH**" means on a FA Gene Therapy Product-by-FA Gene Therapy Product basis, a first in human Clinical Trial for a FA Gene Therapy Product.

1.22 "**First Commercial Sale**" means, on a country-by-country basis, the first commercial transfer or disposition for value of a FA Gene Therapy Product to a Third Party in such country by Licensee, or any of its Affiliates or Sublicensees for end-use, consumption or commercial distribution of such FA Gene Therapy Product in such country after all Regulatory Approvals have been obtained with respect to such FA Gene Therapy Product in such country. Sales prior to receipt of Regulatory Approval are not deemed to be a First Commercial Sale in that country, including, but not limited to, so-called "treatment IND sales," "named patient sales," "early access programs" and "compassionate use sales" and any Sales to any government, foreign or domestic, including purchases for immediate sale and/or stockpiling purposes or FA Gene Therapy Products sold for use in Clinical Trials.

1.23 "**FPFD**" means, on a FA Gene Therapy Product-by-FA Gene Therapy Product basis with respect to each Clinical Trial, the first dosing of the first patient in such Clinical Trial.

1.24 "**GAAP**" means United States generally accepted accounting principles applied on a consistent basis.

1.25 " **Governmental Authority**" means any: (a) nation, principality, state, commonwealth, province, territory, county, municipality, district or other jurisdiction of any nature; (b) federal, provincial, state, local, municipal, foreign or other government; (c) governmental or quasi-governmental authority of any nature (including any governmental division, subdivision, department, agency, bureau, branch, office, commission, council, board, instrumentality, officer, official, representative, organization, unit, body or entity and any court or other tribunal); (d) multi-national or supranational organization or body; or (e) individual, entity, or body exercising, or entitled to exercise, any executive, legislative, judicial, administrative, regulatory, police, military or taxing authority or power of any nature.

1.26 "**IND**" means an Investigational New Drug Application as defined in the FD&C Act and the regulations promulgated thereunder, or the equivalent application to the equivalent Regulatory Authority in any other regulatory jurisdiction, including a Clinical Trial Authorization to the European Medicines Agency, the filing of which is necessary to initiate or conduct clinical testing of a pharmaceutical product in humans in such jurisdiction.

1.27 "**Indication**" means the treatment of Friedreich's ataxia through AAV delivery of nucleic acid polynucleotides that encode for the expression of frataxin, or a functional fragment thereof.

1.28 " **Know-How**" means intellectual property, data, results, pre-clinical and clinical protocols and study data, chemical structures, chemical sequences, information, inventions, formulas, techniques, methods, processes, procedures and developments. "Know-How" does not include Penn Patent Rights claiming any of the foregoing.

1.29 "**Law**" or "**Laws**" means all applicable laws, statutes, rules, regulations, ordinances, guidances and other pronouncements having the binding effect of law of any Governmental Authority.

1.30 "**Licensed Penn Know-How**" means all Know-How that is Controlled by Penn and (a) developed by the Wilson Lab as of the Effective Date and available for licensing, (b) developed in the Wilson Lab under the Research Program, or (c) developed by the Wilson Lab during the Research Term in research not funded by Licensee and available for licensing, and in each case (a), (b) and (c), to the extent such is necessary or reasonably useful to develop, make, use, sell, offer for sale or import FA Gene Therapy Products in the Field of Use. Licensed Penn Know-How does not include Licensed Penn Manufacturing Know-How.

1.31 "**Licensed Penn Manufacturing Know-How**" means all Know-How relating to the methods used in manufacturing or producing an AAV contained in any FA Gene Therapy Product that is Controlled by Penn and (a) developed by the Wilson Lab as of the Effective Date of the Agreement and available for licensing, (b) developed in the Wilson Lab under

the Research Program, or (c) developed by the Wilson Lab during the Research Term in research not funded by Licensee and available for licensing, and in each case (a), (b) and (c), to the extent such is necessary or reasonably useful to develop, make, use, sell, offer for sale or import a FA Gene Therapy Product in the Field of Use. For clarity, Licensed Penn Manufacturing Know-How does not include the [**] cell line, and any transfer of the [**] cell line shall be "as-is" and subject to an MTA executed by the Parties. Any Materials transferred to Licensee shall be subject to an MTA executed by the Parties.

1.32 "**Manufacturing Patent Rights**" means (a) the Patent Rights Controlled by Penn (i) [**], and (i) [**] and set forth on Exhibit B, (b) any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues thereof, including divisions, but excluding continuations-in-part except to the extent of claims entirely supported in the specification and entitled to the priority date of the parent application, and (c) any corresponding foreign Patent Rights to the foregoing.

1.33 "**Materials**" means any biological or chemical materials Controlled by Penn and provided to Licensee under this Agreement and pursuant to the terms of a MTA entered into between the Parties, in each case, that are reasonably necessary (and available from Penn) to exploit the licenses granted to Licensee hereunder, including cell lines, viral seed stocks, product-specific reference materials, platform or product specific assay controls and reagents that are not available as standard commercial items.

1.34 "**MTA**" means a Material Transfer Agreement to be entered into between the Parties.

1.35 "**Net Sales**" means the gross consideration invoiced or received by Licensee or any of its Affiliates or Sublicensees for Sales of FA Gene Therapy Product (including any cash amounts plus the fair market value of any other forms of consideration), less the following deductions (to the extent included in and not already deducted from the gross amounts invoiced or otherwise charged) to the extent reasonable and customary:

[**].

Even if there is overlap between any of deductions described above, each individual item shall only be deducted once in the overall Net Sales calculation.

In the case of a Combination Product, the Parties shall negotiate in good faith, but in no event later than [**] before the expected launch of such Combination Product, an allocation of Net Sales of such Combination Product to the respective FA Gene Therapy Product components and other component(s) thereof, as the case may be, based on the fair market value of such components for the purposes of determining a FA Gene Therapy Product specific allocation of such Net Sales. Payments related to such Combination Product under this Agreement, including Royalties and Milestone Payments, will be calculated, due and payable based only on such allocated Net Sales.

In case of disagreement and failure by the Parties to agree upon an allocation of Net Sales of such Combination Product to the respective FA Gene Therapy Product components and other component(s) thereof, [**].

1.36 "**Patent Rights**" means (a) patents and patent applications, together with any unlisted patents and patent applications claiming priority thereto, and any continuations, continuations-in-part (to the extent related directly to the subject matter of the parent application or containing new information developed pursuant to the Research Program), reissues, reexamination certificates, substitutions, divisionals, supplementary protection certificates, renewals, registrations, extensions including all confirmations, revalidations, patents of addition, PCTs, and pediatric exclusivity periods and all foreign counterparts thereof, and any patents issued or issuing with respect to any of the foregoing and (b) all official correspondence relating to the foregoing.

1.37 "**Penn Patent Rights**" means Penn Patent Rights A and Manufacturing Patent Rights collectively.

1.38 "**Penn Patent Rights A**" means (a) the Patent Rights listed in Exhibit C Controlled by Penn as of the Effective Date, (b) any Patent Rights Controlled by Penn and conceived and reduced to practice by the Wilson Lab in the conduct of the Research Program, (c) any continuations, provisionals, continued prosecution applications, substitutions, extensions and term restorations, registrations, confirmations, reexaminations, renewals or reissues thereof, including divisions, but excluding continuations-in-part except to the extent of claims entirely supported in the specification and entitled to the priority date of the parent application for any of the foregoing, and (d) any corresponding foreign Patent Rights to the foregoing.

1.39 "**Person**" means any natural person, corporation, firm, business trust, joint venture, association, organization, company, partnership or other business entity, or any government or agency or political subdivision thereof.

1.40 "**Pivotal Clinical Trial**" means a human Clinical Trial of an FA Gene Therapy Product in an indicated patient population that is designed to establish that such FA Gene Therapy Product is safe and efficacious for its intended use and to provide adequate data and information for inclusion in product labeling, which Clinical Trial is intended to support marketing approval of such FA Gene Therapy Product, including all tests and studies that are required by the FDA or EMA from time to time, pursuant to Law or otherwise, including the clinical trials referred to in 21 C.F.R. §312.21(c), as amended.

1.41 "**Product**" means any (a) process, service or method covered by a Valid Claim or whose use or practice would, absent the License, constitute an infringement, inducement of infringement or contributory infringement of any Valid Claim, or would infringe a Valid Claim once issued ("**Method**"); (b) article, composition, apparatus, substance, chemical or any other material covered by a Valid Claim or whose manufacture, import, use, offer for sale or sale would, absent the License, constitute an infringement, inducement of infringement or contributory infringement of any Valid Claim or would infringe a Valid Claim once issued; (c) service, article, composition, apparatus, chemical, substance or any other material made, used or sold by or utilizing or practicing a Method, or (d) primarily uses or incorporates the Licensed Penn Know-How.

1.42 "**Regulatory Approval**" means, with respect to a pharmaceutical product in any regulatory jurisdiction, approval from the applicable Regulatory Authority sufficient for the manufacture, distribution, use, marketing and sale of such pharmaceutical product in such jurisdiction in accordance with Laws. "Regulatory Approval" does not include authorization by a Regulatory Authority to conduct "named patient", "compassionate use" or other similar activities.

1.43 "**Regulatory Authority**" means any Governmental Authority, including the FDA or EMA, or any successor agency thereto, that has responsibility for granting any licenses, registrations, authorizations, clearance or approvals or granting pricing or reimbursement approvals necessary for the marketing and sale of a pharmaceutical product in any country.

1.44 "**Representatives**" means, with respect to a Party, such Party's and its Affiliates' officers, directors, employees, licensees, sublicensees, consultants, contractors, collaborators, professional advisors, investigators, current and potential investors, attorneys, accountants and agents.

1.45 "**Research Plan**" means the initial work plan(s) set forth in Exhibit D attached hereto, as the same may be amended from time to time with written approval of the JSC.

1.46 "**Research Program"** means the research and development program for the Indication conducted by Penn in the Wilson Lab and the Service Center Cores in accordance with the Research Plan and Budget.

1.47 "**Research Results**" means all any and all ideas, information, inventions, developments, animate and inanimate materials, including live animals, discoveries, software, Know-How, Methods, techniques, formulae, data, software, processes, methodologies, techniques, biological materials, software and works of authorship, whether patentable or copyrightable, that are first conceived, discovered, developed or reduced to practice, or generated in the performance of the Research Program by the Wilson Laboratory, including any unpatentable inventions discovered, developed or conceived in the conduct of the Research Program. Research Results expressly excludes Penn Patent Rights.

1.48 "**Research Term**" means the period commencing on the Effective Date and ending at DTP.

1.49 "**Royalty Term**" means the period commencing on the First Commercial Sale of a FA Gene Therapy Product in such country and terminating on the latest of (a) expiration or abandonment of the last applicable Valid Claim covering the manufacture, use or sale of such FA Gene Therapy Product in such country, (b) [**] after First Commercial Sale of such FA Gene Therapy Product in such country or (c) expiration of market exclusivity covering such FA Gene Therapy Product in such country.

1.50 "**Sale**" means any transaction (other than a Sublicense) for which consideration is received or invoiced by Licensee, its Affiliates or Sublicensees for sale, use, lease, transfer or other disposition of a FA Gene Therapy Product to or for the benefit of a Third Party. For clarity, the sale, use, lease, transfer or other disposition of a FA Gene Therapy Product by Licensee or any of its Affiliates or Sublicensees to another of these entities for resale by such entity to a Third Party shall not be deemed a Sale.

1.51 "**Service Center Cores**" means the following core laboratories at Penn that report directly to Dr. Wilson, including [**].

1.52 "**Sponsor**" means the entity responsible for the initiation, management, financing and conduct of a Clinical Trial.

1.53 "**Sublicensee**" means a Person (including any Affiliate of Licensee) to which a Sublicense is granted pursuant to the terms of Section 3.4.

1.54 "**Sublicense Documents**" means any and all agreements, amendments or written understandings entered into with a Sublicensee (including its Affiliates) that are directly or indirectly related to a Sublicense, the Penn Patent Rights or FA Gene Therapy Product. For clarity, a development agreement or distribution agreement for a FA Gene Therapy Product is a Sublicense Document.

1.55 "**Sublicense Income**" means income received by Licensee or its Affiliates in consideration for a Sublicense or other agreement providing the right to negotiate or obtain a Sublicense. Sublicense Income includes income received from a Sublicensee in the form of license issue fees and milestone payments, but specifically excludes [**].

1.56 "**Tax**" or "**Taxes**" means all taxes, duties, fees, premiums, assessments, imposts, levies, rates, withholdings, dues, government contributions and other charges of any kind whatsoever, whether direct or indirect, together with all interest, penalties, fines, additions to tax or other additional amounts, imposed by any Governmental Authority.

1.57 "**Third Party**" means any Person other than Penn, Licensee or any of their respective Affiliates.

1.58 "**United States**" or "**U.S.**" means the United States of America, its territories and possessions.

1.59 "**USD**" or "**$**" means the lawful currency of the United States of America.

1.60 "**Valid Claim**" means a claim of (a) an issued and unexpired patent in Penn Patent Rights which claim has not been revoked or held unenforceable or invalid by a decision of a court or governmental agency of competent jurisdiction from which no further appeal can be taken or has been taken within the time allowed for appeal, and has not been abandoned, disclaimed, denied or admitted to be invalid or unenforceable through reissue or disclaimer; or (b) a pending patent application that is included in Penn Patent Rights which was filed and is being diligently prosecuted, which has not been pending for more than [**] from the earliest priority date claimed by such pending patent application, and has not been abandoned or finally disallowed without the possibility of appeal or re-filing of the application.

1.61 "**Wilson Lab**" means Dr. Wilson and all individuals who are under the direct supervision or control of Dr. Wilson at Penn; provided that [**] "Wilson Lab."

1.62 **Other Terms**. The definition of each of the following terms is set forth in the section of the Agreement indicated below:

Defined Term	Section
"**Achievement Date**"	5.5
"**Adjustment Equity**"	4.1
"**Advance Payment**"	6.2.3
"**Agreement**"	Preamble
"**Capital Funding**"	5.1
"**Clinical Trial Diligence Requirement**"	5.5
"**Common Equity**"	4.1
"**Development Milestone**"	4.2
"**Development Milestone Payments**"	4.2
"**Diligence Events**"	5.5
"**Disclosing Party**"	7.1
"**Dr. Wilson**"	Recitals
"**DUA**"	2.7
"**Effective Date**"	Preamble
"**Financial Report**"	4.6
"**Historic Patent Expenses**"	6.2.1
"**Infringement Notice**"	6.3.1
"**Joint Steering Committee**" or "**JSC**"	2.6.1
"**License**"	3.1
"**Licensee**"	Preamble
"**Licensee Data**"	2.7
"**Milestone Payments**"	4.2.1
"**Net Sales Milestone**"	4.2.1
"**Notice Date**"	2.4
"**Ongoing Patent Costs**"	6.2.2
"**Party**"	Preamble
"**Parties**"	Preamble
"**Patent Costs**"	6.2.3
"**Patent Counsel**"	6.1.1
"**Penn**"	Preamble
"**Penn Data**"	2.7
"**Penn Indemnitees**"	9.1.1
"**Progress Report**"	5.6.1
"**Prosecution Request**"	6.1.2

Defined Term	Section
"**Receiving Party**"	7.1
"**Research Support Amount**"	2.3
"**Royalty**"	4.3
"**Royalties**"	4.3
"**Sales Milestone Payments**"	4.2.1
"**SDR Report**"	3.4.4
"**Sublicense**"	3.4.1
"**Term**"	10.1
"**Third Party IP**"	4.3.3(a)

ARTICLE 2
COLLABORATION PROGRAM; GOVERNANCE

2.1 **Overall Project**. The Parties desire to collaborate with respect to the research and development of FA Gene Therapy Products for the Indication. As more specifically outlined herein, Penn will be responsible for research and development activities allocated to Penn under this Agreement until the DTP and as set forth in the Research Plan. Licensee will be responsible for those activities allocated to Licensee under this Agreement and as set forth in the Research Plan and for regulatory strategy and operations, clinical development, GMP manufacture, and commercialization of FA Gene Therapy Products, all as more fully set forth herein.

2.2 **Research**.

2.2.1 **Penn's Responsibilities under Research Program**. Penn will conduct the Research Program in accordance with the Research Plan, Budget and the other terms and conditions of this Agreement. Without limiting the foregoing, Penn will be responsible, in consultation with Licensee through the JSC, and at Licensee's expense, for all research and development activities up to completion of the IND enabling studies, including [**] to support preclinical development of FA Gene Therapy Product(s) for the Indication under the Research Program through DTP. Penn will use [**] to conduct the Research Program in accordance with the Research Plan and Budget to achieve DTP for at least one (1) FA Gene Therapy Product within [**] of the Effective Date. In addition, Penn will also be responsible, at Licensee's expense as reflected in the Budget, for manufacturing to support toxicology studies designed or otherwise required to meet the requirements for an IND filing for a FA Gene Therapy Product. Penn shall conduct the Research Program consistent with Good Laboratory Practices and in compliance with all Laws, including FDA regulations and guidances.

2.2.2 **Licensee's Responsibilities**. Licensee, its Affiliate or Sublicensee will be responsible, at its own expense, for submission of an IND and all post-DTP FA Gene Therapy Product development activities including all manufacturing, clinical testing, Regulatory Approvals, marketing and commercialization. Licensee, or its

designee, shall be responsible as Sponsor for the IND submission. Licensee shall hire employees or engage consultants with expertise in regulatory and clinical matters.

2.2.3 The JSC shall review the Research Plan at least [**]. The JSC may amend the Research Plan at any time, including amendments to include further activities, provided that any corresponding revisions to the Budget shall be subject to the approval of the Parties in accordance with Section 2.6.1(e).

2.2.4 Licensee may, at its option and in its discretion, request that Penn provide manufacturing to support the FIH Clinical Trial for a FA Gene Therapy Product. The Parties, acting through the JSC, agree to use reasonable efforts to negotiate a manufacturing plan setting forth the terms of GMP manufacturing through the use of a mutually agreed upon CMO prior to the DTP; provided that the Licensee shall have final decision making authority with respect to all GMP manufacturing.

2.2.5 Penn shall maintain records of the activities conducted under and the results of the Research Program (including the Research Results) in sufficient detail and in good scientific manner appropriate for patent purposes, consistent with Good Laboratory Practices, in compliance with all Laws, and to properly reflect all work done and results achieved. Penn will provide task-based, scientific reports of the progress and results of the Research Program on the schedule specified in the Research Plan or on another schedule to be agreed in writing by the Parties. Penn shall maintain records of the use of the funds provided by Licensee and shall make such records available to Licensee upon reasonable notice during Penn's normal business hours, but not more frequently than [**]. All Research Results will constitute Licensed Know-How and will be included within the scope of the License granted by Penn to Licensee under this Agreement.

2.2.6 Through the JSC, during the performance of activities under the applicable Research Plan, the Parties will discuss potential Development Candidates and seek to identify and mutually agree upon a Development Candidate arising out of the applicable Research Program.

2.2.7 The Parties hereby acknowledge that there are inherent uncertainties involved in the research and development of products and such uncertainties form part of the business risk involved in undertaking a Research Program. Accordingly, prior to the completion of the applicable Research Plan, if Penn notifies Licensee that it has not developed or has otherwise failed to identify a suitable candidate to propose as a Development Candidate, then the Parties, acting through the JSC, may amend such Research Plan pursuant to Section 2.6.1(b)(v). If the Research Plan is not amended, or the Research Program is terminated by either Party pursuant to Section 2.4, then Penn shall have no further obligations to Licensee under the Research Plan, and any remaining Research Support Amount allocated to the Research Program pursuant to the then current Budget (minus reasonable and documented wind-down and non-cancellable expenses with respect to the activities under the Research Plan) shall be refunded to Licensee.

2.2.8 Each Party will have the right to engage Third Party subcontractors to perform certain of its obligations under this Agreement; provided that Penn's right to engage Third Party subcontractors (except approved contract manufacturing organizations set forth on Exhibit H) is subject to Licensee's prior written consent, which may not be unreasonably withheld. Any subcontractor to be engaged by a Party to perform a Party's obligations set forth in the Agreement will meet the qualifications typically required by such Party for the performance of work similar in scope and complexity to the subcontracted activity and will enter into such Party's standard nondisclosure agreement consistent with such Party's standard practices which agreement shall be as least as protective as the nondisclosure obligations set forth herein. Any Party engaging a subcontractor hereunder will remain responsible and obligated for such activities and will not grant rights to such subcontractor that interfere with the rights of the other Party under this Agreement.

2.3 **Funding of the Research Program**. During the Research Term, Licensee shall provide an amount of $[**] in research and development funding in accordance with the agreed upon Research Plan ("**Research Support Amount**") to Penn to fund certain administrative expenses and the research and development work (and manufacturing costs) conducted in furtherance of the Research Program, including all pre-clinical discovery, research and development activities through IND enabling studies for the FA Gene Therapy Product(s). Such Research Support Amount shall be inclusive of Penn's standard indirect charges. Payments of the Research Support Amount shall be made [**] in advance of the Research Program work being conducted in the applicable [**]. Licensee shall remit such funds in each [**] of the Research Term in accordance with a payment schedule set forth in the Budget and such funds will be allocated solely to the Research Program as set forth in the Research Plan and Budget. The initial Research Plan and Budget (including the payment schedule) for the Research Program are attached hereto as Exhibit D.

2.3.1 If at any time Penn determines that it will require additional funds for the Research Program, it will notify Licensee through the JSC and provide a good faith estimate and itemized budget of the additional amount. Notwithstanding the foregoing, changes to the scope of or Budget for the Research Plan in any Calendar Year will require approval of the JSC if such proposed amendment to the Budget reflects an increase of greater than [**] percent ([**]%) of the agreed upon Budget for such Calendar Year, or $[**], whichever is less.

2.3.2 Unless otherwise indicated in the Research Plan or Budget, title to any equipment, laboratory animals, or any other tangible materials made or acquired with funds provided under this Agreement will vest in Penn, and such equipment, animals, or tangible materials will remain the property of Penn following termination or expiration of this Agreement (but subject to any license grants to Licensee hereunder).

2.4 **Unavailability of Dr. Wilson**. If Dr. Wilson becomes unavailable to oversee and support the performance of the research under the Research Plan for any reason, Penn may propose another member of its faculty who is acceptable to Licensee, in its sole discretion, to oversee the performance of the Research Program. If a substitute faculty member

acceptable to Licensee has not been agreed upon within [**] after Dr. Wilson is no longer available to oversee and support the performance of the Research Plan, Licensee may terminate this Agreement upon written notice thereof to Penn, subject to the provisions of Article 10.

2.5 **Termination of Research Program**. In the event of a material disagreement with respect to the conduct of the Research Program between the Parties, each Party shall have the right to terminate the Research Program upon [**] written notice to Licensee (the "**Notice Date**"). In the event of such termination of the Research Program, Penn will (i) wind-down the Research Program within [**] after such Notice Date, (ii) if applicable, refund all Research Support Amounts not spent by Penn as of the Notice Date, less reasonable and documented wind-down costs and non-cancellable costs and expenses, and (iii) reasonably cooperate with Licensee to transfer data and information necessary to allow Licensee to continue the Research Program.

2.6 **Governance**.

2.6.1 **Joint Steering Committee**.

(a) Formation; Composition. Within [**] of the Effective Date, the Parties will establish a joint steering committee (the "**Joint Steering Committee**" or "**JSC**") comprised of [**] representatives from each Party, each with sufficient seniority within the applicable Party to make decisions arising within the scope of the JSC's responsibilities. The JSC may change its size from time to time by mutual consent of its members, provided that the JSC will consist at all times of an equal number of representatives of each of Penn and Licensee. Each Party may replace its JSC representatives at any time upon written notice to the other Party. The JSC will have the right to create any subcommittees as the JSC sees fit (including a research subcommittee or IP subcommittee).

(b) Specific Responsibilities. The JSC will:

(i) oversee the Research Program; provided, however, that [**] except as otherwise expressly agreed in the Research Plan;

(ii) subject to Section 2.6.1(f), select and designate Development Candidate(s);

(iii) on or before [**] of each year, review the Budget and recommend any changes to the Parties in accordance with Section 2.3.1;

(iv) subject to Section 2.2.1, negotiate a manufacturing plan, including, subject to Section 2.8, the selection of a GMP contract manufacturer prior to the DTP;

(v) approve any amendments to the Research Plan (including any changes to the Budget that are greater than [**] percent ([**]%) of the then-current Budget for the then-current Calendar Year) or $[**], whichever is less;

(vi) review [**] reports prepared by Penn's project management team or joint development committee;

(vii) resolve any disagreement between the Parties relating to the Research Program or Research Plan;

(viii) establish such additional subcommittees as it deems necessary to achieve the objectives and intent of the Research Program;

(ix) use good faith efforts to resolve any disagreement or issues between the Parties relating to the Research Program or the Research Plan; and

(x) perform such other functions as appropriate to further the purposes of this Agreement, in each case as agreed in writing by the Parties.

(c) Reporting. Each Party shall keep the JSC informed on the progress of the activities under the Research Program then currently ongoing under a Research Plan, including delivering written updates each [**] of its progress under the Research Plan to the JSC at least [**] in advance of each JSC meeting.

(d) Meetings. During the performance of the Research Plan by Penn, the JSC will meet at least each [**]. Following the completion of Penn's performance of the Research Plan, the Parties may agree to meet to discuss items previously addressed by the JSC. The JSC may meet in person, by videoconference or by teleconference. Notwithstanding the foregoing, at least [**] will be in person unless the Parties mutually agree in writing to waive such requirement. In-person JSC meetings will be held at locations alternately selected by Penn and by Licensee; provided, however, that Licensee shall reimburse Penn for its JSC representatives' costs in connection with attending such in-person JSC meeting at a location other than Penn. Meetings of the JSC will be effective only if at all representatives of each Party are present or participating in such meeting. The JSC shall keep accurate minutes of its deliberations which shall record all proposed decisions and all actions recommended or taken. The secretary of the JSC (as appointed by the members of the JSC) shall be responsible for the preparation of draft minutes. Draft minutes shall be sent to all members of the JSC within [**] after each meeting and shall be approved, if appropriate, at the next meeting. All records of the JSC shall at all times be available to both Penn and Licensee.

(e) Decision-Making. Except as otherwise set forth in this Agreement, the Parties shall cause their respective members on the JSC to use commercially reasonable efforts to reach a consensus on those matters within the purview of the JSC; provided that the representatives from Penn shall have [**] and representatives from Licensee shall have [**]. Notwithstanding the foregoing, any changes to the scope of, or Budget for, the Research Program

may occur through a recommendation by the JSC and shall require approval of both Parties.

(f) Development Candidate. Notwithstanding the above, with respect to the final approval and selection of a Development Candidate, Licensee shall have final decision- making authority and shall have the final say on the matter, except [**].

2.7 **Research Results**. All right, title and interest in the Research Results generated by Penn under the Research Program shall be owned by Penn ("**Penn Data**"). All right, title and interest in the data generated by Licensee under the Research Program shall be owned by Licensee ("**Licensee Data**") and may be transferred to Penn, including as applicable, under a data usage agreement ("**DUA**"). For the avoidance of doubt, Penn Data shall constitute Research Results and Licensed Know-How and will be included within the scope of the License. The receipt of Licensee Data by Penn and any use or restrictions relating to such Licensee Data on Penn shall be set forth in the DUA.

2.8 **Tech Transfer**. Following the earlier of (i) [**], or (ii) [**]. The selection of the GMP contract manufacturer shall be subject to good faith discussions at the JSC; provided, however that Licensee shall have final decision-making authority with respect to selection of such GMP contract manufacturer.

2.9 **Non-Collaboration**. For a period of [**] from the earlier of: (a) expiration or termination of the Research Program and (b) submission of an IND for the FA Gene Therapy Product, the Wilson Lab shall not collaborate with a Third Party for a gene therapy product for the Indication; provided that the foregoing is subject to the Retained Rights set forth in Section 3.2, and would further not restrict the Wilson Lab from obtaining grant funding from any governmental entity nor from collaborating with or obtaining grant funding from a non-commercial Third Party that does not obtain any right in or to any data, results, inventions or other intellectual property arising in connection with activities conducted under such collaboration or supported by the applicable funding, as the case may be.

ARTICLE 3
LICENSES AND OTHER RIGHTS

3.1 **Grant of Licenses**. Subject to the terms and conditions of this Agreement, Penn hereby grants to Licensee (a) an exclusive, worldwide, royalty-bearing right and license, with the right to sublicense through multiple tiers, under Penn Patent Rights A and (b) a non-exclusive, worldwide, royalty- bearing right and license, without the right to sublicense (except in conjunction with a sublicense to Penn Patent Rights A or a FA Gene Therapy Product), under the Manufacturing Patent Rights, Licensed Penn Know-How, and Licensed Penn Manufacturing Know-How; for each of (a) and (b) to make, have made, use, sell, offer for Sale and import FA Gene Therapy Products for the Indication in the Field of Use during the Term ("**License**").

3.2 **Penn Retained Rights**. Notwithstanding the License, Penn retains the right under the Penn Patent Rights A to (a) conduct educational, academic research and clinical/patient

care activities at Penn's and its Affiliates' hospital facilities itself (including sponsored research) and (b) authorize non-commercial Third Parties to conduct education, academic research and clinical/patient care activities; provided however that [**].

3.3 **U.S. Government Rights**. The License is expressly subject to all applicable provisions of any license to the U.S. government executed by Penn and is subject to any overriding obligations to the U.S. government under 35 U.S.C. §§200-212, applicable governmental implementing regulations, and the U.S. government sponsored research agreement or other guidelines, including that products that result from intellectual property funded by the [**].

3.4 **Grant of Sublicense by Licensee**.

3.4.1 Penn grants to Licensee the right to grant sublicenses, in whole or in part, under the License (each, a "**Sublicense**") subject to the terms and conditions of this Agreement and specifically this Section 3.4. The term Sublicense shall include any grant of rights under the License by a Sublicensee to any downstream Third Party, such downstream Third Party shall also be considered a Sublicensee for purposes of this Agreement.

3.4.2 All Sublicenses will be (a) issued in writing, (b) to the extent applicable, include all of the rights of Penn and require the performance of obligations due to Penn (and, if applicable, the U.S. government under 35 U.S.C. §§200-212) contained in this Agreement and (c) shall include no less than the following terms and conditions:

(a) reasonable record keeping, audit and reporting obligations sufficient to enable Licensee and Penn to reasonably verify the payments due to Licensee and Penn under such Sublicense and to reasonably monitor such Sublicensee's progress in developing and/or commercializing FA Gene Therapy Product, provided that such obligations shall be no less stringent that those provided in this Agreement for Licensee;

(b) infringement and enforcement provisions that do not conflict with the restrictions and procedural requirements imposed on Licensee and do not provide greater rights to Sublicensee than as provided in Section 6.3;

(c) confidentiality provisions with respect to Confidential Information of Penn consistent with the restrictions on Licensee in Article 7 of this Agreement;

(d) covenants by Sublicensee that are equivalent to those made by Licensee in Section 8.4;

(e) a requirement of indemnification of Penn by Sublicensee that is equivalent to the indemnification of Penn by Licensee under Section 9.1 of this Agreement;

(f) a requirement of obtaining and maintaining insurance by Sublicensee that is equivalent to the insurance requirements of Licensee under Section 9.2 of

this Agreement, including coverage under such insurance of Penn as provided in Section 9.2;

(g) a restriction on use of Penn's names consistent with Section 11.4 of this Agreement;

(h) a requirement of antidiscrimination by Sublicensee no less stringent than that provided in Section 11.5 of this Agreement; and

(i) a requirement that Penn is a third party beneficiary of such Sublicense.

Any Sublicense that does not include all of the terms and conditions set forth in this Section 3.4.2 or which is not issued in accordance with the terms and conditions set forth in this Section 3.4, shall be considered null and void with no further notice from Penn.

3.4.3 Within [**] after of the execution of a Sublicense Document, Licensee shall provide a complete and accurate copy of such Sublicense Document to Penn, in English. Penn's receipt of a Sublicense Document, however, will constitute neither an approval nor disapproval of the Sublicense Document nor a waiver of any right of Penn or obligation of Licensee under this Agreement. Any Sublicense Document provided to Penn pursuant to this Section 3.4.3 may be redacted with respect to matters unnecessary to show compliance with the terms of this Section 3.4; provided that, upon written request by Penn, Licensee shall provide an un-redacted version of any Sublicense Document to Penn's counsel. [**].

3.4.4 Licensee shall provide [**] Sublicense Development Report on or before [**] during the Term ("**SDR Report**"), a form of which is attached hereto as Exhibit E.

3.5 **No Implied License**. Each Party acknowledges that the rights and licenses granted in this Agreement are limited to the scope expressly granted. Accordingly, except for the rights expressly granted under this Agreement, no right, title, or interest of any nature whatsoever is granted whether by implication, estoppel, reliance, or otherwise, by either Party to the other Party. All rights with respect to any Know-How, Patent Right or other intellectual property right rights that are not specifically granted herein are reserved to the owner thereof.

ARTICLE 4
FINANCIAL PROVISIONS

4.1 **Equity Issuance**. On the Effective Date, and in partial consideration of the rights and licenses granted to Licensee under this Agreement, Licensee and Penn shall enter into an Equity Issuance Agreement in the form attached hereto as Exhibit F. Licensee shall issue Penn common units of Licensee ("**Common Equity**"), and which is equal to [**] percent ([**]%) of the fully-diluted equity of Licensee outstanding, measured on a fully diluted basis, as of the Effective Date, assuming the exercise, conversion and exchange of all outstanding securities of the Licensee for or into common units of Licensee. Licensee shall also issue additional Common Equity to Penn ("**Adjustment Equity**") until such time as

[**] USD ($[**]) has been raised by Licensee in net proceeds from the sales of securities of the Licensee or in other financings, including instruments convertible into equity of the Licensee, so that after issuance of the Adjustment Equity, Penn still owns [**] percent ([**]%) of the fully-diluted equity of Licensee outstanding on a fully diluted basis as of the date of such issuance. For clarity, Adjustment Equity shall be issued at no additional consideration from Penn to Licensee. After such time as [**] USD ($[**]) has been raised by Licensee in net proceeds from the sales of securities of the Licensee, Penn shall have the option to purchase, either by itself or through its affiliates or [**], additional equity in subsequent financing rounds in order to maintain its ownership percentage in Licensee on the same terms as other round investors. Penn agrees to become a party to all investor agreements (including, but not limited to any limited liability company agreement, voting agreement, investor rights agreement or right of first refusal and co-sale agreement) in connection with the issuance of Common Equity to Penn by Licensee, whether existing as of the Effective Date or thereafter entered into among Licensee and its investors.

4.2 **Milestone Payments**. Licensee shall pay Penn the following non-refundable and non-creditable milestone payments (the "**Development Milestone Payments**") within [**] after Licensee, or any Affiliate or Sublicensee (including sub-sublicensees), achieves the corresponding development milestone (each, a "**Development Milestone**") with respect to the first FA Gene Therapy Product:

Development Milestone	**Development Milestone Payment**
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
Total Development Milestone Payments to Penn	**$13,500,000**

For clarity, any earlier Development Milestone that has not been achieved at the time of achievement of a later Development Milestone shall be deemed achieved and payable upon achievement of the later Development Milestone by the first FA Gene Therapy Product; provided that in no event will any Development Milestone Payment in respect of any Development Milestone be paid more than once. In no event shall Licensee owe greater than $13,500,000 in Development Milestone Payments.

4.2.1 **Sales Milestone Payments**. Licensee shall pay Penn the following non-refundable and non-creditable milestone payments (each, a "**Sales Milestone Payment**", and together with the Development Milestone Payments, the "**Milestone Payments**") within [**] after Licensee, or any Affiliate or Sublicensee (including sub-sublicensees) achieves the corresponding annual Net Sales milestone (each, a "**Net Sales Milestone**") for the first FA Gene Therapy Product:

Net Sales Milestone	**Sales Milestone Payment**
1) Annual Net Sales of FA Gene Therapy reach $[**]	[**]
2) Annual Net Sales of FA Gene Therapy Product reach $[**]	[**]

Net Sales Milestone	Sales Milestone Payment
3) Annual Net Sales of FA Gene Therapy Product reach $[**]	[**]
Total Sales Milestone Payments to Penn	**$27,500,000**

For clarity, any earlier Sales Milestone that has not been achieved at the time of achievement of a later Sales Milestone shall be deemed achieved and payable upon achievement of the later Sales Milestone for the first FA Gene Therapy Product; provided that in no event will any Sales Milestone Payment in respect of any Sales Milestone be paid more than once. For clarity, the foregoing Sales Milestone Payments shall be due once with respect to the first FA Gene Therapy Product to achieve the applicable Net Sales Milestone, and in no event shall Licensee owe greater than $27,500,000 in Sales Milestone Payments.

4.3 **Royalties**.

4.3.1 **Royalty**. As further consideration for the License, during the Royalty Term on a country- by-country, FA Gene Therapy Product-by-FA Gene Therapy Product basis, Licensee shall pay Penn on a Calendar Quarter basis a non-refundable and non-creditable royalty (each, a "**Royalty**" or collectively, the "**Royalties**") on all Net Sales of a FA Gene Therapy Product by Licensee, its Affiliate, or Sublicense (including sub-sublicensees) in accordance with the chart below.

Annual Worldwide Net Sales of each FA Gene Therapy Product	Royalty Rate
Less than $[**]	[**]%
Greater than or equal to $[**] and less than or equal to $[**]	[**]%
Greater than $[**]	[**]%

4.3.2 **Step Down**. On a country-by-country and FA Gene Product-by-FA Gene Product basis, during any portion of the Royalty Term in which the applicable FA Gene Therapy Product is not covered by at least one Valid Claim of the Penn Patent Rights A in the applicable country, then the Royalty rate set forth in Section 4.3.1 shall be reduced to [**] percent ([**]%) of the Royalty rate otherwise payable pursuant to Section 4.3.1.

4.3.3 **Royalty Reductions**.

(a) If after the Effective Date Licensee determines upon the advice of outside intellectual property counsel to license or otherwise acquire rights to Patent Rights from a Third Party is reasonably necessary to develop, commercialize, or manufacture a FA Gene Therapy Product ("**Third Party IP**"), Licensee may obtain such rights from such Third Party.

(b) If Licensee, its Affiliate or a Sublicensee is required to pay any Third Party amounts with respect to Third Party IP, Licensee may deduct [**] percent

([**]%) such amounts paid to such Third Party from any Royalty payments otherwise due to Penn under Section 4.3 of this Agreement, subject to Section 4.3.3(c) below.

(c) If Licensee, its Affiliate, its Sublicensee or Penn is required by Law to grant a Compulsory License in any country, then the Royalty due to Penn for such country by Licensee shall be adjusted to equal such lower Royalty rate granted to such Third Party under such Compulsory License, but in no circumstances shall the Royalty rate due to Penn be reduced by more than [**] percent ([**]%) of the then applicable Royalty rate.

(d) Notwithstanding anything in this Agreement to the contrary, in no event will the deductions under this Section 4.3.2 reduce the Royalty payable in respect of Net Sales of such FA Gene Therapy Product in such country by more than [**] percent ([**]%) of the Royalty as set forth in Section 4.3.1 above.

4.3.4 **Calculations**. Licensee must pay Royalties owed to Penn on a Calendar Quarter basis on or before the following dates:

(a) [**] for any Sales that took place on or before the last day of the Calendar Quarter ending December 31, of the prior year (and for clarity, such Royalties shall contain a final true-up based on the total Annual Net Sales in such prior year based on the royalty tiers set forth in Section 4.3.1;

(b) [**] for any Sales that took place on or before the last day of the Calendar Quarter ending March 31 of such Calendar Year;

(c) [**] for any Sales that took place on or before the last day of the Calendar Quarter ending June 30 of such Calendar Year; and

(d) [**] for any Sales that took place on or before the last day of the Calendar Quarter ending September 30 of such Calendar Year

4.4 **Sublicensee Revenue**. Licensee shall pay Penn a percentage of any Sublicensee Income received by Licensee from a Sublicensee (including sub-sublicensees) of any Penn Patent Right or Licensed Penn Know-How for a FA Gene Therapy Product in accordance with the table below.

Stage in FA Gene Therapy Product Development at which Sublicense Is Granted	**Percentage of Sublicensing Income Payable to Penn**
[**]	[**]%
[**]	[**]%
[**]	[**]%

4.4.1 Licensee will make such payment to Penn on or before the following dates:

(a) [**] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending December 31, of the prior year;

(b) [**] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending March 31 of such Calendar Year;

(c) [**] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending June 30 of such Calendar Year; and

(d) [**] for any Sublicense Income received by Licensee on or before the last day of the Calendar Quarter ending September 30 of such Calendar Year.

4.5 **Mode of Payment and Currency**. All payments to Penn hereunder shall be made by deposit of USD in the requisite amount to the "The Trustees of the University of Pennsylvania" and will be made by delivery to any one of the following:

For funding of the performance of the Research Program by Penn:

By ACH/Wire:
[**]
Payment should include
the necessary amount to cover
any bank charges incurred.

For all other payments to Penn under this Agreement:

By ACH/Wire:	**By Check (lockbox):**
[**]	The Trustees of the University of Pennsylvania
Payment should include the *necessary amount to cover* *any bank charges incurred.*	c/o Penn Center for Innovation PO Box 785546 Philadelphia, PA 19178-5546

Payments under this Agreement shall be made in USD. All Royalties payable shall be calculated first in the currency of the jurisdiction in which payment was made, and if not in the United States, then converted into USD. The exchange rate for such conversion shall be the average of the rate quoted in The Wall Street Journal for the last business day of each month in the Calendar Quarter for such Royalty payment made.

4.6 **Royalty and Penn Sublicense Income Reports**. Following the First Commercial Sale, for each Calendar Quarter during the Term within [**] after the end of such Calendar Quarter, Licensee shall deliver to Penn a report (each, a "**Financial Report**") setting out such details as are reasonably necessary to calculate the Royalty and Sublicense Income due to Penn under this Article 4 for such Calendar Quarter, including:

[**].

Each Financial Report shall be in the form of the sample report attached hereto as Exhibit G.

4.7 **Late Payments**. In addition to any other remedies available to Penn, including the right to terminate this Agreement in accordance with Article 10, any failure by Licensee to make a payment within [**] after the date when due shall obligate Licensee to pay computed interest, the interest period commencing on the due date and ending on the actual payment date, to Penn at a rate per annum equal to [**] percent ([**]%) per month, or the highest rate allowed by Law, whichever is lower.

4.8 **Default Payment**. In the event of default in payment of any payment owing to Penn under the terms of this Agreement, and if it becomes necessary for Penn to undertake legal action to collect said payment, Licensee shall pay reasonable, documented legal fees and costs incurred in connection therewith.

4.9 **Accounting**. Each Party shall calculate all amounts, and perform other accounting procedures required, under this Agreement and applicable to it in accordance with GAAP.

4.10 **Books and Records**. Licensee will keep accurate books and records of all FA Gene Therapy Products developed, manufactured, used or sold and all Sublicenses, collaboration agreements and joint venture agreements entered into by Licensee that involved a Sublicense of the Penn Patent Rights. Licensee will preserve these books and records for at least [**] from the date of the Financial Report to which they pertain. Upon reasonable notice, key personnel, books and records pertaining to the calculation of Royalties, Milestone Payments or Sublicensing Income will be made reasonably available and will be open to examination by representatives or agents of Penn reasonably acceptable to Licensee (and who has executed an appropriate confidentiality agreement reasonably acceptable to Licensee that requires such representative or agent to keep any information learned by it confidential except as needed to report its conclusions to Penn), during Licensee's regular office hours to determine their accuracy and assess Licensee's compliance with the terms of this Agreement, provided that Licensee shall not have an obligation to provide access more than [**] period.

4.11 **Audits**. In addition to the right of Penn to examine the books and records and interview key personnel as provided in Section 4.10 above, Penn, at its own cost, through an independent auditor [**] (and who has executed an appropriate confidentiality agreement reasonably acceptable to Licensee that requires the auditor to keep any information learned by it confidential except as needed to report its audit conclusions to Penn), may inspect and audit the relevant records of Licensee pertaining to the calculation of any Milestone Payments, Royalties and Sublicense Income due to Penn under this Agreement. Licensee shall provide such auditors with access to the records during reasonable business hours. Such access need not be given to any such set of records more often than [**] or more than [**] after the date of any report to be audited. Penn shall provide Licensee with written notice of its election to inspect and audit the records related to the Milestone Payments, Royalties and Sublicensing Income due hereunder not less than [**] prior to the proposed date of review of Licensee's records by Penn's auditors. Should the auditor find any underpayment of Milestone Payments, Royalties or Sublicense Income by Licensee to

Penn, Licensee shall (a) promptly pay Penn the amount of such underpayment; (b) if such underpayment equals or exceeds the higher of (i) [**] dollars ($[**]) or (ii) [**] percent ([**]%) of Milestone Payments, Royalties or Penn Sublicense Income paid during the time period audited, reimburse Penn for the cost of the audit; and (c) provide such auditors with an audit right exercisable within [**] after Penn receives the audit report. If the auditor finds overpayment by Licensee, then Licensee shall have the right to deduct the overpayment from any future Milestone Payments, Royalties or Sublicensing Income due to Penn by Licensee or, if no such future Milestone Payments, Royalties or Sublicensing Income are payable, then Penn shall refund the overpayment to Licensee within [**] after Penn receives the audit report. Licensee may designate competitively sensitive information which such auditor may see and review but which it may not disclose to Penn; provided, however, that such designation shall not restrict the auditor's investigation or conclusions.

4.12 **Taxes**. All payments made by Licensee to Penn under the Agreement shall be made free and clear of and without any deduction for or on account of any Taxes on or with respect to such payments.

ARTICLE 5
DILIGENCE; CLINICAL DEVELOPMENT; REGULATORY AFFAIRS; COMMERCIALIZATION

5.1 **Financial Diligence**. Licensee agrees to have sufficient Capital Funding in order to support the funding of the Research Program hereunder. Within [**] from the Effective Date, Licensee agrees to have capital commitments in place equal to a minimum of $[**] across all fundraising rounds, tranches and sources (excluding any prior amounts paid or gifted to Penn) to fund its operations, including cash for the purchase of capital stock, debt financing, lines of credit and grant funding (collectively referred to as "**Capital Funding**"). During the period commencing on the Effective Date and ending on the [**] of the Effective Date, Licensee agrees to raise a cumulative amount equal to the Research Support Amount in Capital Funding, but acknowledges that additional Capital Funding may be needed to support its obligations under the Agreement.

5.2 **Development Diligence by Licensee**. Licensee shall use Commercially Reasonable Efforts to actively develop and commercialize one (1) FA Gene Therapy Product in the Field of Use for the Indication.

5.3 **Development Plan**. Following the DTP until the First Commercial Sale for the first FA Gene Therapy Product, Licensee shall provide Penn with a development plan for a FA Gene Therapy Product or update thereof no later than [**]. The development plan shall include a timeline of detailed activities to be conducted by Licensee, its Affiliates and Sublicensees, and Licensee shall provide Penn with [**] progress reports regarding achievements and activities under such development plan.

5.4 **Regulatory**.

5.4.1 Except as set forth in Section 2.2.1, as between the Parties, Licensee shall have responsibility for and decision-making over all regulatory activities for the FA

Gene Therapy Products. As between the Parties, Licensee will have the right to conduct all communications with Regulatory Authorities, including all meetings, conferences and discussions (including advisory committee meetings), with regard to FA Gene Therapy Products. Licensee will lead and have control over preparing and submitting all regulatory filings related to the FA Gene Therapy Products, including all applications for Regulatory Approval; provided, however, that Licensee shall provide Penn with copies of all such applications prior to submission. Licensee will own solely any and all applications for Regulatory Approvals (including INDs), Regulatory Approvals, and other regulatory filings related to any FA Gene Therapy Product which will be held in the name of Licensee or its designees.

5.4.2 Until the First Commercial Sale, Penn shall have the right to participate as an observer in all material meetings, conferences, and discussions by Licensee with Regulatory Authorities pertaining to development of the corresponding FA Gene Therapy Products and Regulatory Approvals. Licensee shall provide Penn with reasonable advance notice of all such meetings and other contact and shall provide advance copies of all related documents and other relevant information relating to such meetings or other contact, including any documents that Licensee proposes to submit to any Regulatory Authority.

5.4.3 Penn (through Dr. Wilson and other Wilson Lab personnel) will cooperate with any reasonable request from Licensee (or its designee) in connection with interactions with Regulatory Authorities relating to a FA Gene Therapy Product or with respect to obtaining and maintaining any Regulatory Approval for a FA Gene Therapy Product including, at Licensee's cost: (a) [**], (b) [**], and (c) [**].

5.5 **Structured Development Diligence Events**. In addition to the development plan, Licensee shall use Commercially Reasonable Efforts to achieve the following diligence events (the "**Diligence Events**") with respect to one (1) FA Gene Therapy Product by the corresponding Achievement Date (the "**Achievement Date**"):

Diligence Event	**Achievement Date**
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

Licensee may extend any Achievement Date for a Diligence Event by [**] increments, other than the Clinical Trial Diligence Requirement, but not more than [**] per Diligence Event, by providing prior written notice to Penn in advance of the Achievement Date, as amended, along with a payment of [**] USD ($[**]) for each extension. Notwithstanding the foregoing, Licensee's diligence obligations require the Licensee to use Commercially Reasonable Efforts to achieve the Clinical Trial Diligence Requirement by the Achievement Date.

5.6 **Progress Reports**.

5.6.1 Following the expiration or earlier termination of the Research Program, but prior to the First Commercial Sale of a FA Gene Therapy Product, Licensee, on [**] basis, but in no event later than [**], shall submit to Penn a progress report (each, a "**Progress Report**") covering Licensee's (and any Affiliates' and Sublicensees') material activities related to the development of all FA Gene Therapy Products and the obtaining of Regulatory Approvals necessary for commercialization of FA Gene Therapy Products.

5.6.2 Each Progress Report must include all of the following for each [**] period:

[**].

ARTICLE 6
INTELLECTUAL PROPERTY

6.1 **Patent Filing Prosecution and Maintenance**.

6.1.1 Penn Patent Rights will be held in the name of Penn and obtained with counsel selected by Penn and reasonably acceptable to Licensee ("**Patent Counsel**"). Penn shall Control all actions and decisions with respect to the filing, prosecution and maintenance of Penn Patent Rights and will consider any reasonable comments or suggestions by Licensee with respect to same; provided, however, that with respect to Penn Patent Rights claiming solely a FA Gene Therapy Product, Penn shall have an obligation to consider in good faith any reasonable comments provided by Licensee. Penn will instruct Patent Counsel to copy Licensee, or its counsel, on all correspondence related to Penn Patent Rights A (including copies of each patent application, office action, response to office action, request for terminal disclaimer, and request for reissue or reexamination of any patent or patent application) and to interact with Licensee, or its counsel, with respect to the preparation, filing, prosecution and maintenance of Penn Patent Rights A. Penn has the right to take action to preserve rights and minimize cost whether or not Licensee has commented, and will use Commercially Reasonable Efforts to not allow any Penn Patent Rights for which Licensee is licensed and is underwriting the costs to lapse or become abandoned without Licensee's written authorization under this Agreement, except for filing of continuations, divisionals, or the like that substitute for the lapsed application, provided that, Penn shall have no requirement to file, prosecute, or maintain Penn Patent Rights if Licensee is not current with the Patent Cost obligations as set forth in this Agreement. For the purposes of this Agreement, "maintenance" of the Penn Patent Rights includes inter parties patent review proceedings before the USPTO or a similar patent administration outside the U.S. For further clarity, validity challenges raised in infringement litigation will be handled per Section 6.3. Penn shall not discontinue prosecution or maintenance activities with respect to Penn Patent Rights solely covering an FA Gene Therapy Product (other than strategic abandonment), provided that Licensee is not in breach of Section 6.2 relating to all Patent Costs.

6.1.2 Licensee has the right to request a country filing via a written request to Penn [**] prior to the deadline set by the patent office in the territory in which filing is to take place ("**Prosecution Request**"). The absence of a given Prosecution Request by such deadline will be considered an election not to secure the Patent Rights associated with the specific phase of patent prosecution in such territory, and such patent application(s) and patent(s) will not be part of Penn Patent Rights and therefore not subject to this Agreement, including the License, and Licensee will have no further rights or license to them.

6.1.3 Patent Term Extension. The Parties agree to meet and discuss and mutually agree which Patent within the Penn Patent Rights the Parties would seek patent term extensions and the Parties shall cooperate fully with each other in making such filings or actions, for example and without limitation, making available all required regulatory Data and information, and executing any required authorizations to apply for such patent term extension. For clarity, all expenses incurred in connection with activities of Penn with respect to the Penn Patent Rights pursuant to this Section 6.1.3 for the FA Gene Therapy Product shall be considered a Patent Cost.

6.2 **Patent Costs**.

6.2.1 Within [**] of the Effective Date, Licensee shall reimburse Penn for all reasonable and documented out-of-pocket expenses actually incurred by Penn prior to the Effective Date for the prosecution, maintenance and enforcement of the Penn Patent Rights A ("**Historic Patent Expenses**") that have not otherwise been reimbursed by other licensees. The Historic Patent Expenses are $[**].

6.2.2 **Prosecution and Maintenance Costs during the Term**. Licensee shall be responsible for payment to Penn of all documented patent prosecution, maintenance and enforcement costs for the Penn Patent Rights A incurred by Penn during the Term ("**Ongoing Patent Costs**"). For Penn Patent Rights licensed to more than one licensee, Licensee shall be responsible for payment to Penn of an equal pro rata share of such Ongoing Patent Costs based on the number of licensees for such Penn Patent Rights.

6.2.3 At any time, at Penn's request, Licensee shall pay in advance the Patent Counsel's estimated costs for undertaking material patent actions before Penn authorizes the Patent Counsel to proceed ("**Advance Payment**"). Notwithstanding whether Licensee makes an Advance Payment for any patent action, Licensee shall bear all Patent Costs incurred during the Term and shall pay such amounts within [**] of receipt of invoice for such patent actions. For clarity, the term "**Patent Costs**" means and includes Historic Patent Costs and Ongoing Patent Costs. For clarity, this Section 6.2.3 [**].

6.3 **Infringement**.

6.3.1 If either Party believes that an infringement by a Third Party with respect to any Penn Patent Right is occurring or may potentially occur, the knowledgeable Party will provide the other Party with (a) written notice of such infringement or potential infringement and evidence of such infringement or potential infringement (the "**Infringement Notice**"). During the period in which, and in the jurisdiction where, Licensee has exclusive rights under this Agreement, neither Penn or Licensee will notify such a Third Party (including the infringer) of infringement or put such Third Party on notice of the existence of Penn Patent Rights without first obtaining the written consent of the other Party. If Licensee puts such infringer on notice of the existence of any Penn Patent Right without the prior written consent of Penn, then Licensee's right to initiate a suit under Section 6.3.2 below will terminate immediately without the obligation of Penn to provide notice to Licensee. Both Penn and Licensee will use their diligent efforts to cooperate with each other to terminate such infringement without litigation.

6.3.2 If infringing activity of potential commercial significance has not been abated within [**] following the date the Infringement Notice for such activity was provided, then during the period in which, and in the jurisdiction where, Licensee has exclusive rights under this Agreement, Licensee may institute suit for patent infringement against the infringer after providing Penn (a) a written estimate of the expenses that would be reasonably incurred in connection with such action, including an estimate from [**] outside law firms regarding the legal costs associated with such suit and (b) financial records reasonably sufficient to reasonably demonstrate that Licensee has the financial wherewithal to pay such expenses as they fall due through the conclusion of such suit by means of judgment or other final non-appealable decision. Penn may voluntarily join such suit at Licensee's reasonable expense, but may not thereafter commence suit against the infringer for the acts of infringement that are the subject of Licensee's suit or any judgment rendered in such suit. Licensee may not join Penn in a suit initiated by Licensee without Penn's prior written consent, such consent not to be unreasonably withheld. If in a suit initiated by Licensee, Penn is involuntarily joined other than by Licensee, then Licensee will pay any costs incurred by Penn arising out of such suit, including any legal fees of counsel that Penn selects and retains to represent it in the suit. Licensee shall be free to enter into a settlement, consent judgment or other voluntary disposition, provided that any settlement, consent judgment or other voluntary disposition that (i) limits the scope, validity or enforcement of Penn Patents or (ii) admits fault or wrongdoing on the part of Licensee or Penn must be approved in advance by Penn in writing. Licensee's request for such approval shall include complete copies of final settlement documents, a detailed summary of such settlement, and any other information material to such settlement. Penn shall provide Licensee notice of its approval or denial within [**] of any request for such approval by Licensee, provided that (x) in the event Penn wishes to deny such approval, such notice shall include a detailed written description of Penn's reasonable objections to the proposed settlement, consent judgment, or other voluntary disposition and Penn shall be deemed to have approved of such proposed settlement, consent judgment, or other voluntary disposition in the event it fails to provide such notice within such [**] period in accordance herewith.

6.3.3 If, within [**] following the date the Infringement Notice was provided, infringing activity of potential commercial significance has not been abated and if Licensee has not brought suit against the infringer, then Penn may institute suit for patent infringement against the infringer. If Penn institutes such suit, then Licensee may not join such suit without the prior written consent of Penn and may not thereafter commence suit against the infringer for the acts of infringement that are the subject of Penn's suit or any judgment rendered in such suit.

6.3.4 Notwithstanding Sections 6.3.2 and 6.3.3, in the event that any Penn Patent Rights are infringed by a Third Party (a) prior to the First Commercial Sale of a FA Gene Therapy Product in the United States or (b) if any of the infringed Penn Patent Rights are also licensed by Penn to a Third Party prior to any enforcement action being taken by either Party regarding such infringement, the Parties shall discuss, and will mutually agree, in writing, as to how to handle such infringement by such Third Party.

6.3.5 Any recovery or settlement received in connection with any suit will first be shared by Penn and Licensee equally to cover any litigation costs each incurred and next shall be paid to Penn or Licensee to cover any litigation costs it incurred in excess of the litigation costs of the other. Any remaining recoveries shall be allocated as follows:

For any portion of the recovery or settlement, other than for amounts attributable and paid as enhanced damages for willful infringement:

(a) for any suit that is initiated by Licensee and in which Penn was not a party in the litigation, Penn shall receive [**] percent ([**]%) of the recovery and the Licensee shall receive the remainder; and

(b) for any suit that is initiated by the Licensee or Penn and that the other Party joins voluntarily (but only to the extent such voluntary joining is allowed under this Agreement or expressly by the other Party in a separate agreement) or involuntarily, the non-initiating Party's percentage of the total litigation costs incurred by Penn and Licensee, but in no event shall the non-initiating Party receive less than [**] percent ([**]%) of such recovery, while the initiating Party shall receive the remainder, and in no case shall Penn receive less than [**] percent ([**]%) of such recovery.

For any portion of the recovery or settlement paid as enhanced damages for willful infringement:

(c) for any suit that is initiated by Licensee or Penn and the other Party joins voluntarily (but only to the extent such voluntary joining is allowed under this Agreement or expressly by the other Party in a separate agreement) or involuntarily, Penn shall receive [**] percent ([**]%) and Licensee shall receive the remainder; and

(d) for any suit that is initiated by Licensee and in which Penn was not a party in the litigation, Penn shall receive [**] percent ([**]%) and Licensee shall receive the remainder.

For any portion of the recovery or settlement received in connection with any suit that is initiated by Penn and in which Licensee was not a party in the litigation, any recovery in excess of litigation costs will belong to Penn.

6.3.6 Each Party will reasonably cooperate and assist with the other in litigation proceedings instituted hereunder but at the expense of the Party who initiated the suit (unless such suit is being jointly prosecuted by the Parties). For clarity, [**]. If Penn is subjected to Third Party discovery related to the Penn Patent Rights or FA Gene Therapy Products licensed to Licensee hereunder, Licensee will pay Penn's documented out of pocket expenses with respect to same.

6.4 **Patent Marking**. Licensee shall place in a conspicuous location on any FA Gene Therapy Product (or its packaging where appropriate and practicable) made or sold under this Agreement a patent notice in accordance with the Laws concerning the marking of patented articles where such FA Gene Therapy Product is made or sold, as applicable.

ARTICLE 7
CONFIDENTIALITY& PUBLICATION

7.1 **Confidential Information**. Licensee shall not disclose Confidential Information to Penn unless it is necessary or useful to the performance of the Research Program or otherwise required to perform Licensee's obligations under this Agreement. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing, the Parties agree that, during the Term and for [**] thereafter, the receiving Party (the "**Receiving Party**") and its Representatives will keep confidential and will not publish or otherwise disclose or use for any purpose, other than as provided for in this Agreement, any Confidential Information or Materials, patentable or otherwise, in any form (written, oral, photographic, electronic, magnetic, or otherwise), including trade secrets, Know-How, inventions or discoveries, proprietary information, formulae, processes, techniques and information relating to the past, present and future marketing, financial, and research and development activities of any Product or potential Product or useful technology of the other Party (the "**Disclosing Party**") or its Representatives and the pricing thereof, which is disclosed to it by the Disclosing Party or its Representatives or otherwise received or accessed by a Receiving Party or its Representatives in the course of performing its obligations or exercising its rights under this Agreement.

7.2 **Exceptions to Confidentiality**. "Confidential Information" does not include information that (a) was in the lawful knowledge and possession of the Receiving Party or its Representatives prior to the time it was disclosed to, or learned by, the Receiving Party or its Representatives, or was otherwise developed independently by the Receiving Party or its Representatives, as evidenced by written records kept in the ordinary course of business, or other documentary proof of actual use by the Receiving Party or its Representatives; (b) was generally available to the public or otherwise part of the public domain at the time of

its disclosure to the Receiving Party or its Representatives, as evidenced by written records of the Receiving Party or its Representatives; (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party or its Representatives in breach of this Agreement; or (d) was disclosed to the Receiving Party or its Representatives, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party or its Representatives not to disclose such information to others. In the event a Party is required to make a disclosure under Law or regulation, the order of a court of competent jurisdiction, or the rules of the U.S. Securities and Exchange Commission (including by reason of any securities offering by Licensee), any stock exchange or listing entity, a Receiving Party shall provide prompt written notice to the Disclosing Party and take commercially reasonable steps to limit the extent of the disclosure and seek confidential treatment for any remaining required disclosure, to the extent permitted by Law.

7.3 **Penn Intellectual Property**. In order to preserve the patentability of Penn intellectual property and to preserve Penn's publication rights, Licensee shall maintain Penn Patent Rights, Research Results and information provided pursuant to the Research Program (whether oral or written) as confidential and shall not disclose such information to any Third Party (other than its Representatives) until the publication of such information by Penn or until Penn provides Licensee with written verification that all desirable patentable inventions have been protected, whichever occurs sooner.

7.4 **Publications**. Penn shall have the first right to publish, present or otherwise disclose Research Results or other information and material resulting from the Research Program for any purpose. Penn shall furnish the Licensee with a copy of any proposed publication or presentation at least [**] in advance of the date of such presentation or the submission of said proposed publication (and [**] in advance for any abstract or speaking engagement) in order for Licensee to review and comment on said proposed publication or presentation to (a) determine whether such contains any Licensee Confidential Information and (b) enable Licensee to identify any Penn intellectual property that Licensee wishes Penn to file patent applications on or to seek other intellectual property protection for. If within the [**] review period (or [**] review period) (i) Licensee notifies Penn that the Licensee requires deletion from the publication or presentation of Licensee Confidential Information, the Parties will cooperate to modify the disclosure to ensure Licensee Confidential Information is not disclosed or (ii) if Licensee requests that publication or presentation be delayed to allow for patent filings or other intellectual property protection on certain items in the proposed publication or presentation, Penn shall delay the publication or presentation for up to [**] to allow for the filing of patent applications or other intellectual property protection.

ARTICLE 8
REPRESENTATIONS, WARRANTIES AND COVENANTS

8.1 **Mutual Representations and Warranties**. Each Party represents and warrants to the other Party that, as of the Effective Date:

8.1.1 such Party is duly organized and validly existing under the Laws of the jurisdiction of its incorporation or organization;

8.1.2 such Party has taken all action necessary to authorize the execution and delivery of this Agreement and the performance of its obligations under this Agreement;

8.1.3 this Agreement is a legal and valid obligation of such Party, binding upon such Party and enforceable against such Party in accordance with the terms of this Agreement, except as enforcement may be limited by applicable bankruptcy, fraudulent conveyance, insolvency, reorganization, moratorium and other Laws relating to or affecting creditors' rights generally and by general equitable principles; and

8.1.4 such Party has all right, power and authority to enter into this Agreement, to perform its obligations under this Agreement.

8.2 **Representations of Penn**. Penn hereby represents and warrants to Licensee that, as of the Effective Date:

8.2.1 Penn Controls all Penn Patent Rights;

8.2.2 [**];

8.2.3 [**];

8.2.4 to Penn's knowledge, Penn's performance of the activities allocated to Penn under the Research Plan in furtherance of the Research Program and/or grant of rights to Licensee under this Agreement do not conflict with any agreement with a Third Party;

8.2.5 [**]; and

8.2.6 [**].

8.3 **Disclaimer of Representations and Warranties**.

8.3.1 Other than the representations and warranties provided in Sections 8.1 and 8.2 above, **PENN MAKES NO REPRESENTATIONS AND WARRANTIES, WHETHER EXPRESS OR IMPLIED, AND EXPLICITLY DISCLAIMS ANY REPRESENTATION AND WARRANTY, INCLUDING WITH RESPECT TO ANY ACCURACY, COMPLETENESS, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, COMMERCIAL UTILITY, NON-INFRINGEMENT OR TITLE FOR THE INTELLECTUAL PROPERTY, PATENT RIGHTS, LICENSE AND ANY FA GENE THERAPY PRODUCT**.

8.3.2 Furthermore, nothing in this Agreement will be construed as:

(a) a representation or warranty by Penn as to the validity or scope of any Penn Patent Right;

(b) a representation or warranty that anything made, used, sold or otherwise disposed of under the License is or will be free from infringement of patents, copyrights, trademarks or any other forms of intellectual property rights or tangible property rights of Third Parties;

(c) an obligation of Penn to bring or prosecute actions or suits against Third Parties for patent, copyright or trademark infringement; or

(d) conferring by implication, estoppel or otherwise any license or rights under any Patent Rights of Penn other than Penn Patent Rights as defined herein, regardless of whether such Patent Rights are dominant or subordinate to Penn Patent Rights.

8.4 **Covenants of Licensee**.

8.4.1 Licensee and its Affiliates will not, directly or indirectly (including where such is done by a Third Party on behalf of Licensee or its Affiliates, at the urging of Licensee or its Affiliates or with the assistance of the Licensee or its Affiliates) challenge the validity, scope, or enforceability of or otherwise oppose any Penn Patent Right, provided that if any Penn Patent Right is asserted against Licensee or its Affiliate for activities authorized under this Agreement, then such Licensee or its Affiliates is entitled to all and any defenses available to it including challenging the validity or enforceability of such Penn Patent Right. Licensee will comply with all Laws that apply to its activities or obligations under this Agreement. For example, Licensee will comply with applicable United States Export Laws and regulations. The transfer of certain technical data and commodities may require a license from the applicable agency of the United States government and/or written assurances by Licensee that Licensee will not export data or commodities to certain foreign countries without prior approval of the agency.

8.4.2 Licensee will not grant a security interest in the License or this Agreement.

ARTICLE 9
INDEMNIFICATION; INSURANCE AND LIMITATION OF LIABILITY

9.1 **Indemnification by Licensee**.

9.1.1 Licensee shall defend, indemnify and hold Penn and its respective trustees, officers, faculty, students, employees, contractors and agents (the "**Penn Indemnitees**") harmless from and against any and all liability, damage, loss, cost or expense (including reasonable attorneys' fees), including, without limitation, bodily injury, risk of bodily injury, death and property damage only to the extent arising out of Third Party claims or suits related to:

(a) the gross negligence, recklessness or wrongful intentional acts or omissions of Licensee, its Affiliates or Sublicensees and its or their respective directors, officers, employees and agents, in connection with Licensee's performance of its obligations or exercise of its rights under this Agreement;

(b) any material breach of this Agreement by Licensee;

(c) the development, manufacturing or commercialization (including commercial manufacturing, packaging and labeling of FA Gene Therapy Products, and all product liability losses) of a FA Gene Therapy Product by or on behalf of Licensee or its Affiliates or Sublicensees; or

(d) any enforcement action or suit brought by Licensee against a Third Party for infringement of Penn Patent Rights;

provided that Licensee's obligations pursuant to this Section 9.1 shall not apply to the extent such claims or suits result from the gross negligence, recklessness or willful misconduct of any of Penn Indemnitees as determined by a court of law.

9.1.2 As a condition to a Penn Indemnitee's right to receive indemnification under this Section 9.1, Penn shall: (a) promptly notify Licensee as soon as it becomes aware of a claim or suit for which indemnification may be sought pursuant hereto; (b) reasonably cooperate, and cause the individual Penn Indemnitees to reasonably cooperate, with Licensee in the defense, settlement or compromise of such claim or suit; and (c) permit the Licensee to control the defense, settlement or compromise of such claim or suit, including the right to select defense counsel. In no event, however, may Licensee compromise or settle any claim or suit in a manner which (a) admits fault or negligence on the part of Penn or any other Penn Indemnitee; (b) commits Penn or any other Penn Indemnitee to take, or forbear to take, any action, without the prior written consent of Penn; or (c) grant any rights under the Penn Patent Rights except for Sublicenses permitted under Article 3. Penn shall reasonably cooperate with Licensee and its counsel in the course of the defense of any such suit, claim or demand, such cooperation to include without limitation using reasonable efforts to provide or make available documents, information and witnesses.

9.1.3 Notwithstanding Section 9.1.2 above, in the event that Penn believes in good faith that a bona fide conflict exists between Licensee and Penn or any other Penn Indemnitee with respect to a claim or suit subject to indemnification hereunder, then Penn or any other Penn Indemnitee shall have the right to defend against any such claim or suit itself, including by selecting its own counsel, with any reasonable attorney's fees and litigation expenses being paid for by Licensee. Licensee will pay such fees and expenses either directly or will reimburse Penn within [**] of Licensee's receipt of invoices for such fees and expenses.

9.1.4 [**].

9.2 **Insurance**.

9.2.1 Licensee, at its sole cost and expense, must insure its activities in connection with the exercise of its rights under this Agreement and obtain, and keep in force and maintain Commercial Form General Liability Insurance (contractual liability included) with limits as follows:

(a) Each occurrence $[**];

(b) General aggregate $[**];

Prior to the commencement of Clinical Trials, if applicable, involving FA Gene Therapy Product:

(c) Clinical trials liability insurance $[**];

Prior to the First Commercial Sale of a FA Gene Therapy Product:

(d) Products liability insurance $[**].

Penn may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 9.2.1, and has the right to require Licensee to adjust the limits in Penn's reasonable discretion.

9.2.2 If the above insurance is written on a claims-made form, it shall continue for [**] following termination or expiration of this Agreement. The insurance shall have a retroactive date of placement prior to or coinciding with the Effective Date of this Agreement.

9.2.3 Licensee expressly understands, however, that the coverages and limits in Section 9.2.1 do not in any way limit Licensee's liability or indemnification obligations. Licensee's insurance will:

(a) be issued by an insurance carrier with an A.M. Best rating of "A" or better;

(b) provide for [**] advance written notice to Penn of any modification;

(c) state that Penn is endorsed as an additional insured with respect to the coverages in Section 9.2.1; and

(d) include a provision that the coverages will be primary and will not participate with nor will be excess over any valid and collective insurance or program of self- insurance carried or maintained by Penn.

9.2.4 Licensee must furnish to Penn with (a) valid certificate of insurance evidencing compliance with all requirements of this Agreement and (b) additional insured endorsements for Licensee's applicable policies naming "The Trustees of the University of Pennsylvania" as an additional insured. Licensee must furnish both documents within [**] of the Effective Date, once per year thereafter and at any time there is a modification in such insurance.

9.3 **LIMITATION OF LIABILITY**. IN NO EVENT SHALL EITHER PARTY OR ANY OF ITS AFFILIATES BE LIABLE TO THE OTHER PARTY OR ANY OF ITS AFFILIATES FOR SPECIAL, INDIRECT, INCIDENTAL, CONSEQUENTIAL OR PUNITIVE DAMAGES, INCLUDING LOSS OF PROFITS, WHETHER IN CONTRACT, WARRANTY, TORT, NEGLIGENCE, STRICT LIABILITY OR OTHERWISE ARISING OUT OF OR RELATING TO THIS AGREEMENT, THE TRANSACTIONS CONTEMPLATED HEREIN OR ANY BREACH HEREOF. NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS AGREEMENT SHALL LIMIT LICENSEE'S INDEMNIFICATION OBLIGATIONS UNDER SECTION 9.1 ABOVE.

ARTICLE 10
TERM AND TERMINATION

10.1 **Term**. The term of this Agreement (the "**Term**") shall commence on the Effective Date and, unless terminated sooner as provided below, shall continue in full force and effect on a country-by-country basis until the last to expire Royalty Term.

10.2 **Termination of the Agreement for Convenience**. At any time during the Term, Licensee will have the right to terminate the Agreement in its entirety for any reason or merely for convenience, upon providing ninety (90) days written notice to Penn.

10.3 **Termination For Cause**.

10.3.1 If Licensee fails to fulfill its obligations under Section 5.2 (i.e. use Commercially Reasonable Efforts to develop and commercialize a FA Gene Therapy Product in the Indication), Penn may provide written notice to Licensee of any such failure. If Licensee fails to take substantial action to remedy the failure within [**] of receiving such written notice as reasonably determined by Penn, Penn may thereafter terminate this Agreement upon written notice to Licensee; provided that, if Licensee disputes in good faith whether Licensee has failed to fulfill its obligations under Section 5.2 and provides written notice of such dispute, setting forth its dispute in reasonable details and including written documentation supporting such dispute, prior to the expiration of such [**] period, then such dispute shall be escalated to the senior officers pursuant to Section 11.10; provided further, however, [**]. In the event that Licensee fails to request to have the matter resolved within such [**] period above, Licensee shall be deemed to have accepted the determination of Penn.

10.3.2 Penn may terminate this Agreement if Licensee fails to achieve the Clinical Trial Diligence Requirement by the applicable Achievement Date and Licensee does not cure such breach within [**] of receiving written notice from Penn of such breach.

10.3.3 If either Party materially breaches any of its material obligations under this Agreement, the non-breaching Party may terminate this Agreement upon written notice if the breaching Party fails to cure such material breach within [**] after written notice from the non-breaching Party. Such written notice shall specify the

nature of the material breach, notify the breaching Party that it is required to cure such material breach, and state the non-breaching Party's intention to terminate this Agreement.

10.3.4 Penn may terminate this Agreement immediately upon notice from Penn if (a) Licensee or its Affiliate, either directly or indirectly, challenges the validity, enforceability or otherwise opposes any Penn Patent Right, (b) Licensee materially fails to comply with any Laws that apply to its activities or obligations under this Agreement, (c) Licensee grants a security interest in any Penn Patent Right, or (d) Licensee becomes insolvent or files for bankruptcy relief.

10.3.5 Either Party may terminate this Agreement, upon written notice, with immediate effect if, at any time, the other Party is (a) unable to pay its debts, including any debts related to exclusive sublicensees, when they come due, or (b) files in any court or agency pursuant to any statute or regulation of any state, country or jurisdiction, a petition in bankruptcy or insolvency or for reorganization or for an arrangement or for the appointment of a receiver or trustee of such Party or of its assets, or (c) if such Party proposes a written agreement of composition or extension of its debts, or (d) if such Party is served with an involuntary petition against it, filed in any insolvency proceeding, and such petition is not dismissed within [**] after the filing thereof, or (e) if such Party proposes or is a party to any dissolution or liquidation, or (f) if such Party makes an assignment for the benefit of its creditors of all or substantially all its assets.

10.4 **Effects of Termination**.

10.4.1 Notwithstanding the termination of this Agreement, the following provisions shall survive: Sections 2.7, 2.9 (solely to the extent that this Agreement is terminated prior to the expiration of such one (1) year period), 3.2, 3.3, 3.5, 4.5, 4.6, 4.7, 4.8, 4.9, 4.10, 4.11, 4.12, 8.3 and 10.4 and Article 1, Article 7, Article 9 and Article 11.

10.4.2 Termination of this Agreement shall not relieve the Parties of any obligation or liability that, at the time of termination, has already accrued hereunder, or which is attributable to a period prior to the effective date of such termination. Termination of this Agreement shall not preclude either Party from pursuing all rights and remedies it may have hereunder or at Law or in equity with respect to any breach of this Agreement nor prejudice either Party's right to obtain performance of any obligation.

10.4.3 If this Agreement is terminated for any reason, all outstanding Sublicenses (including all Sublicense Documents for each Sublicense) not in default shall survive, provided that each such Sublicensee agrees in writing to be bound by the applicable terms of this Agreement with respect to the activities of such Sublicensee under such Sublicense. The duties and obligations of Penn under any surviving Sublicenses will not be greater than the duties of Penn under this Agreement, and the rights of Penn under any surviving Sublicenses will not be less than the rights of Penn under this Agreement.

10.4.4 Within [**] of termination of this Agreement or the Research Program (other than termination by Licensee pursuant to Sections 10.3.3 or 10.3.5, termination by Penn prior to the DTP, or the termination of the Research Program pursuant Section 2.4), Licensee shall pay Penn all costs through the effective termination date per the Budget of the Research Plan as well as all commitments related to the performance of the Research Plan that are set forth in the Budget (i.e., all costs or non-cancellable commitments incurred prior to the receipt, or issuance, by Penn or Licensee, as applicable, of the notice of termination, and the cost of each employee, student and faculty member supported under the Research Plan through the end of such commitments; and subject to Penn's written notification to Licensee and Licensee's agreement with respect thereto, of all costs and non-cancellable commitments as they arise) incurred by Penn under this Agreement, or for the terminated Research Program, as applicable.

10.4.5 Within [**] of termination of this Agreement or the Research Program by Licensee pursuant to Sections 10.3.3 or 10.3.5, by Penn prior to the DTP, or the termination of the Research Program pursuant Section 2.4, in each case, prior to the DTP, [**], or Licensee shall pay any additional amounts due to the wind-down costs and non-cancellable costs and expenses within such [**] period.

10.4.6 Upon termination (but not expiration) of this Agreement, Licensee, its Affiliates and Sublicensees will promptly cease developing, commercializing, selling or promoting the FA Gene Therapy Product(s). Licensee will return (or destroy, as directed by Penn) all data, files, records and other Materials (in the case of Penn) containing or comprising Penn's Confidential Information with respect to this Agreement, except to the extent such Confidential Information is necessary or useful to conduct activities in connection with surviving portions of this Agreement. Notwithstanding the foregoing, Licensee will be permitted to retain one copy of such data, files, and records for archival and legal compliance purposes.

ARTICLE 11
ADDITIONAL PROVISIONS

11.1 **Relationship of the Parties**. Nothing in this Agreement is intended or shall be deemed, for financial, Tax, legal or other purposes, to constitute a partnership, agency, joint venture or employer-employee relationship between the Parties. The Parties are independent contractors and at no time will either Party make commitments or incur any charges or expenses for or on behalf of the other Party.

11.2 **Expenses**. Except as otherwise provided in this Agreement, each Party shall pay its own expenses and costs incidental to the preparation of this Agreement and to the consummation of the transactions contemplated hereby

11.3 **Third Party Beneficiary**. The Parties agree that each Sublicensee is a third party beneficiary of this Agreement with respect to Section 10.4.4.

11.4 **Use of Names**. Licensee, its Affiliates and Sublicensees may not use the name, logo, seal, trademark, or service mark (including any adaptation of them) of Penn or any Penn school, organization, employee, student or representative, without the prior written consent of Penn. Notwithstanding the foregoing, Licensee may use the name of Penn in a non-misleading and factual manner solely in (a) executive summaries, business plans, offering memoranda and other similar documents used by Licensee for the purpose of raising financing for the operations of Licensee as related to FA Gene Therapy Product, or entering into commercial contracts with Third Parties, but in such case only to the extent necessary to inform a reader that the Penn Patent Rights has been licensed by Licensee from Penn, and to inform a reader of the identity and published credentials of inventors of intellectual property, and (b) any securities reports required to be filed with the Securities and Exchange Commission.

11.5 **No Discrimination**. Neither Penn nor Licensee will discriminate against any employee or applicant for employment because of race, color, sex, sexual or affectional preference, age, religion, national or ethnic origin, handicap, or veteran status.

11.6 **Successors and Assignment**.

11.6.1 The terms and provisions hereof shall inure to the benefit of, and be binding upon, the Parties and their respective successors and permitted assigns.

11.6.2 Neither Party may assign or transfer this Agreement or any of its rights or obligations created hereunder, by operation of law or otherwise, without the prior written consent of the other Party. Notwithstanding the foregoing, without Penn's consent, Licensee shall have the right to assign or transfer this Agreement or any of Licensee's rights or obligations under this Agreement to (a) any of its Affiliates, or (b) to a Third Party in connection with a merger, acquisition of all or substantially all of the business or assets of Licensee to which this Agreement relates (whether by sale of equity, assets, operation of Law or otherwise). A Party shall promptly notify the other Party in writing, and in no event longer than [**] after, of any assignment or transfer of this Agreement pursuant to the terms set forth in this Section 11.6.2. Any permitted assignee shall assume all obligations of its assignor under this Agreement.

11.6.3 Any assignment not in accordance with this Section 11.6 shall be void.

11.7 **Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments and to do all such other acts as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

11.8 **Entire Agreement of the Parties; Amendments**. This Agreement, the Exhibits and Appendices or Schedules hereto, Equity Issuance Agreement and, to the extent entered into, the Client & Billing Agreement, constitute and contain the entire understanding and agreement of the Parties respecting the subject matter hereof and cancel and supersede any and all prior negotiations, correspondence, understandings and agreements between the Parties, whether oral or written, regarding such subject matter. No waiver, modification or

amendment of any provision of this Agreement shall be valid or effective unless made in a writing referencing this Agreement and signed by a duly authorized officer of each Party.

11.9 **Governing Law**. This Agreement shall be governed by and interpreted in accordance with the Laws of the Commonwealth of Pennsylvania, excluding application of any conflict of Laws principles that would require application of the Law of a jurisdiction outside of the Commonwealth of Pennsylvania.

11.10 **Dispute Resolution**. If a dispute arises between the Parties concerning this Agreement, then the Parties will confer, as soon as practicable, in an attempt to resolve the dispute (in accordance with Section 2.6, if applicable). Prior to the initiation of outside dispute resolution or termination of this Agreement pursuant to Section 10.3.1 or for material breach pursuant to Section 10.3.3, each Party shall escalate such issue to their respective senior officers, the [**] of Licensee and the [**] for Penn (or his or her designee), and such senior officers will engage in good faith discussions with regard to the applicable issue within [**]. If the Parties are unable to resolve such dispute amicably through good faith discussion and such escalation within [**], then either Party may submit to the exclusive jurisdiction of, and venue in, the state and Federal courts located in the Eastern District of Pennsylvania.

11.11 **Notices and Deliveries**. Any notice, request, approval or consent required or permitted to be given under this Agreement shall be in writing and directed to a Party at its address or facsimile number shown below or such other address or facsimile number as such Party shall have last given by notice to the other Party. A notice will be deemed received: if delivered personally, on the date of delivery; if mailed, five (5) days after deposit in the United States mail; if sent via courier, one (1) business day after deposit with the courier service; or if sent via facsimile, upon receipt of confirmation of transmission provided that a confirming copy of such notice is sent by certified mail, postage prepaid, return receipt requested.

For Penn	***with a copy to:***
Penn Center for Innovation University of Pennsylvania 3600 Civic Center Rd. 9th Floor Philadelphia, PA 19104 Attention: Managing Director	University of Pennsylvania Office of General Counsel 2929 Walnut St. 4th Floor Philadelphia, PA 19104 Attention: General Counsel
For Licensee:	***with a copy to:***
FA212 LLC 211 Stuyvesant Avenue Rye, NY 10580 Attention: Chief Executive Officer	Troutman Pepper Hamilton Sanders LLP 3000 Two Logan Square Philadelphia, PA 19103 Attention: Rachael Bushey, Esq.

11.12 **Waiver**. A waiver by either Party of any of the terms and conditions of this Agreement in any instance shall not be deemed or construed to be a waiver of such term or condition for

the future, or of any other term or condition hereof. All rights, remedies, undertakings, obligations and agreements contained in this Agreement shall be cumulative and none of them shall be in limitation of any other remedy, right, undertaking, obligation or agreement of either Party.

11.13 **Severability**. When possible, each provision of this Agreement will be interpreted in such manner as to be effective and valid under Law, but if any provision of this Agreement is held to be prohibited by or invalid under Law, such provision will be ineffective only to the extent of such prohibition or invalidity, without invalidating the remainder of this Agreement. The Parties shall make a good faith effort to replace the invalid or unenforceable provision with a valid one which in its economic effect is most consistent with the invalid or unenforceable provision.

11.14 **Interpretation**. The words "include", "includes" and "including" shall be deemed to be followed by the phrase "without limitation". All references herein to Articles, Sections, Schedules and Exhibits shall be deemed references to Articles and Sections of, Schedules and Exhibits to, this Agreement unless the context shall otherwise require. Except as otherwise expressly provided herein, all terms of an accounting or financial nature shall be construed in accordance with GAAP, as in effect from time to time. Unless the context otherwise requires, countries shall include territories. References to any specific Law or article, section or other division thereof, shall be deemed to include the then-current amendments or any replacement Law thereto.

11.15 **Counterparts**. This Agreement may be executed in counterparts, each of which will be deemed an original, and all of which together will be deemed to be one and the same instrument. A facsimile or a portable document format (PDF) copy of this Agreement, including the signature pages, will be deemed an original.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, duly authorized representatives of the Parties have executed this Agreement as of the Effective Date.

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA	**FA212 LLC**
By: /s/ John S. Swartley	By: /s/ Thomas E. Hamilton
Name: John S. Swartley	Name: Thomas E. Hamilton
Title: Associate Vice Provost for Research and Managing Director, Penn Center for Innovation	Title: Co-founder

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AMENDMENT No. 1 TO THE RESEARCH, COLLABORATION AND LICENSE AGREEMENT

This FIRST AMENDMENT to the Research, Collaboration and License Agreement dated November 4, 2020 is entered into as of ___, 2021 (the "First Amendment") by and between FA212, LLC a limited liability organized under the laws of Delaware ("FA212") and The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation, with offices located at Penn Center for Innovation, 3600 Civic Center Blvd., 9th Floor, Philadelphia, PA 19104 ("Penn") and amends the Research, Collaboration and License Agreement dated May 5, 2020 (the "Research Agreement") FA212 and Penn are referred to collectively as the "Parties" and individually as a "Party."

WHEREAS, the Parties wish to amend the Research Agreement; and

NOW, THEREFORE, in consideration of the promises and mutual covenants contained in the Restated Agreement and herein, and intending to be legally bound hereby, the Parties amend the Restated Agreement and otherwise agree as follows:

1. The Research Program contained in Exhibit B of the Research Agreement is hereby amended to include the additional work outlined in Schedule A to this First Amendment.

2. The budget for the additional work is included in Schedule B to this First Amendment, and this budget is intended to be in addition to those previously included under the Research Agreement.

3. The payment schedule set forth in Schedule C is incremental to the payment schedule contained in the Collaboration Agreement.

4. This First Amendment and the Research Agreement contain the entire understanding between the Parties and supersede any and all prior agreements, understandings and arrangements whether written or oral between the Parties with respect to the matters contained in the Research Agreement and this First Amendment. No amendments, changes, modifications or alterations of the terms and conditions of this First Amendment shall be binding upon any Party, unless provided in writing and signed by an authorized representative of each Party.

5. All terms and conditions of the Research Agreement not changed by this First Amendment shall remain in full force and effect.

6. Signatures on this First Amendment may be communicated by e-mail transmission and shall be binding upon the Parties upon receipt by transmitting the same by e-mail, which signatures shall be deemed originals. If executed in counterparts, the First Amendment shall be effective as if simultaneously executed.

(*Signature page follows*.)

IN WITNESS WHEREOF the Parties hereto have caused this First Amendment to be executed and delivered by their duly authorized representatives as set forth below.

AGREED ON BEHALF OF:

FA212, LLC

BY: ____
(Signature)
Name:
Title: President and CEO

AGREED ON BEHALF OF:

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

BY: /s/ John S. Swartley____
(Signature)
Name: John S. Swartley
Title: Managing Director, Penn Center for Innovation

ACKNOWLEDGED AS READ AND UNDERSTOOD
BY INSTITUTION PRINCIPAL INVESTIGATOR

____
(Signature)
Name: Dr. James Wilson

University of Pennsylvania

AMENDMENT No. 2 TO THE RESEARCH, COLLABORATION AND LICENSE AGREEMENT

This SECOND AMENDMENT ("**Second Amendment**") is entered into as of August 6, 2024 (the "**Second Amendment Effective Date**") by and between FA212, LLC, a limited liability company organized under the laws of the State of Delaware ("**Licensee**") and The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation, with offices located at Penn Center for Innovation, 3600 Civic Center Blvd., 9th Floor, Philadelphia, PA 19104 ("**Penn**") and amends that certain Research, Collaboration and License Agreement dated November 4, 2020 (the "**Original Agreement**"), as amended by that certain First Amendment to the Research, Collaboration and License Agreement dated November 1, 2021 ("**First Amendment**") (the Original Agreement as amended by the First Amendment, "**Research Agreement**"). Capitalized terms used, but not defined herein, shall have the meaning ascribed to such term in the Research Agreement.

WHEREAS, the Parties wish to amend the Research Agreement; and

NOW, THEREFORE, in consideration of the promises and mutual covenants contained in the Research Agreement and herein, and intending to be legally bound hereby, the Parties amend the Research Agreement and otherwise agree as follows:

1. Expense Reimbursement. Exhibit D attached to the Research Agreement sets forth the Research Plan and Budget for the Research Program. Section 4.2 of the Research Agreement sets forth the Milestone Payments that may become payable to Penn upon the achievement of the corresponding milestones. Following the Second Amendment Effective Date, Licensee agrees to reimburse Penn for expenses incurred by Penn in connection with the performance of the Research Program and the preparation of an IND for the Research Program in such amounts and in accordance with the reimbursement payment schedule as set forth in Schedule A attached hereto, which amounts, for the avoidance of doubt, are incremental and in addition to the amounts set forth in Exhibit D of the Research Agreement and the Milestone Payments set forth in Section 4.2 of the Research Agreement.

2. Amendments. Section 2.2 of the Research Agreement is hereby amended to add the following subsection 2.2.9 immediately following the existing subsection 2.2.8:

"2.2.9 So long as Dr. Wilson and the Wilson Lab remain at Penn, Penn, through the Wilson Lab, shall prepare an IND for the Research Program to be filed by Licensee with the FDA on or about [**] or such other date mutually agreed by the Parties at the JSC. The IND will be deemed open upon the earlier of: (a) Licensee receiving a safe to proceed notification from the FDA, (b) thirty (30) days after submission of the IND application to the FDA by Licensee with no clinical hold response from the FDA, and (c) thirty (30) days after submission of a response to a clinical hold response from the FDA in the event that such initial IND filing received a clinical hold response ("**Open IND**"). Until FPFD in FIH, and so long as Dr. Wilson and the Wilson Lab remain at Penn, Penn shall make appropriate individuals within the Wilson Lab available to respond to questions and other requests from the FDA, other Regulatory Authorities and the Licensee

(including its successors, assigns, subcontractors and commercial partners) as necessary or reasonably required with respect to the filed IND/Open IND as mutually agreed by the Parties at the JSC. In the event that Dr. Wilson leaves his current position at Penn for employment at Gemma, Penn acknowledges that Gemma [**].

3. Acknowledgement. The Parties acknowledge and agree that as of the Second Amendment Effective Date, (a) Licensee has provided the Research Support Amount to Penn in the amount of $[**], (b) all obligations of Licensee pursuant to Section 4.1 of the Research Agreement and Sections 2.1 and 2.2 of that certain Equity Issuance Agreement, dated [**], by and between the Licensee and Penn (the "**Equity Issuance Agreement**") have been satisfied, and (c) Penn holds an aggregate of [**] Common Units of Licensee ([**] Common Units that were issued pursuant to Section 2.1 of the Equity Issuance Agreement and [**] Common Units that were issued pursuant to Section 2.2 of the Equity Issuance Agreement).

4. Entire Agreement. This Second Amendment, the Research Agreement and the Equity Issuance Agreement contain the entire understanding between the Parties and supersede any and all prior agreements, understandings and arrangements whether written or oral between the Parties with respect to the matters contained in the Research Agreement, this Second Amendment and the Equity Issuance Agreement. No amendments, changes, modifications or alterations of the terms and conditions of this Second Amendment shall be binding upon any Party, unless provided in writing and signed by an authorized representative of each Party.

5. No Other Changes. All terms and conditions of the Research Agreement not changed by this Second Amendment shall remain in full force and effect.

6. Counterparts. Signatures on this Second Amendment may be communicated by e-mail transmission and shall be binding upon the Parties upon receipt by transmitting the same by e-mail, which signatures shall be deemed originals. If executed in counterparts, the Second Amendment shall be effective as if simultaneously executed.

(Signature page follows.)

IN WITNESS WHEREOF, the Parties hereto have caused this Second Amendment to be executed and delivered as of the date set forth above by their duly authorized representatives as set forth below.

AGREED ON BEHALF OF:

FA212, LLC

By: /s/ Thomas E. Hamilton
(Signature)

Name: Thomas E. Hamilton
Title: Co-Founder

AGREED ON BEHALF OF:

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ Benjamin Dibling, PhD
(Signature)

Name: Benjamin Dibling, PhD
Title: Associate Vice Provost for Research
and Managing Director, Penn Center for Innovation

ACKNOWLEDGED AS READ AND UNDERSTOOD
BY INSTITUTION PRINCIPAL INVESTIGATOR

(Signature)
Name: Dr. James Wilson

Exhibit 10.51

Execution Version

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

Dated as of September 19, 2024

by and between

SOLID BIOSCIENCES INC.

and

FA212 LLC

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ASSET PURCHASE AGREEMENT

This Asset Purchase Agreement (this "Agreement") dated as of September 19, 2024, is entered into by and between Solid Biosciences Inc., a Delaware corporation ("Buyer"), and FA212 LLC, a Delaware limited liability company ("Seller"). Buyer and Seller are sometimes individually referred to herein as a "Party" and are sometimes collectively referred to herein as the "Parties."

RECITALS

WHEREAS, Seller desires to sell to Buyer, and Buyer wishes to purchase from Seller, all of Seller's right, title and interest in, to and under the Purchased Assets upon the terms and subject to the conditions set forth herein.

NOW, THEREFORE, in consideration of the mutual benefits to be derived from this Agreement, and of the representations, warranties, conditions, agreements and promises contained herein, and other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties, intending to be legally bound, hereby agree as follows:

ARTICLE I

DEFINITIONS; INTERPRETATION

Section 1.1. Definitions. For purposes of this Agreement, the following terms will have the corresponding meanings set forth below:

"Acquisition" has the meaning set forth in Section 2.1.

"Act" has the meaning set forth in Section 2.6(d)(iii).

"Action" means any claim, action, cause of action or suit, litigation, assessment, arbitration, mediation, investigation, audit, hearing, charge, complaint, demand, notice or proceeding (in each case, whether in contract, tort or otherwise, whether at law or in equity, and whether civil or criminal or administrative) to, from, by or before any Governmental Authority.

"Affiliate" of any Person means another Person that directly or indirectly, through one or more intermediaries, Controls, is Controlled by or is under common Control with, such first Person.

"Agreement" has the meaning set forth in the preamble hereof.

"Annual Net Sales" means, with respect to a Calendar Year, the aggregate Net Sales by Buyer and its Affiliates and its or their licensees of all Products to Third Parties in the Territory during such Calendar Year.

"Assigned Agreements" has the meaning set forth in Section 2.2(a)(iii).

"Assumed Liabilities" has the meaning set forth in Section 2.3.

"Bill of Sale" means the bill of sale and assignment and assumption agreement by and between Buyer or its Affiliate, on the one hand, and Seller, on the other hand, substantially in the form of Exhibit A.

"BLA" means (a) a Biologics License Application as defined in the PHSA and the regulations promulgated thereunder, (b) a Marketing Authorization Application in the European Union or (c) any equivalent or comparable application, registration or certification in any other country or region.

"Books and Records" means all books, reports (including laboratory reports), records, files and documents in the possession or under the control of Seller related to the Business and the Purchased Assets (including research and development, Regulatory Documentation, correspondence with Governmental Authorities) other than corporate minute books. The Books and Records existing as of the Closing Date are described on Annex 1.1(a).

"Business" means all business of Seller relating to or arising out of the UPenn License, including the Exploitation of the FA Asset and Products.

"Business Day" means any day other than (a) a Saturday or Sunday or (b) a day on which banking institutions located in New York, New York are permitted or required by applicable Law to remain closed.

"Business Partner" has the meaning set forth in Section 5.5(b).

"Buyer" has the meaning set forth in the preamble hereof.

"Buyer Charter" means the Certificate of Incorporation of Buyer as in effect from time to time.

"Buyer Disclosure Schedules" means the Schedules delivered by Buyer to Seller contemporaneously with this Agreement and appended hereto, setting forth disclosures in respect of the representations and warranties contained in Article IV of this Agreement.

"Buyer Indemnified Party" has the meaning set forth in Section 6.1(a).

"Buyer Indemnity Threshold" has the meaning set forth in Section 6.1(b).

"Buyer Representation Letter" has the meaning set forth in Section 2.6(d)(iii).

"Calendar Year" means a period of twelve consecutive months beginning on January 1 and ending on December 31.

"CARES Act" means the Coronavirus Aid, Relief, and Economic Security Act (P.L. 116-136), any current federal, state or local Laws or guidance relating to the COVID-19 pandemic, any similar or successor legislation, as well as any applicable guidance issued thereunder or relating thereto (including, without limitation, IRS Notice 2020-65 and IRS Notice 2021-11), in any United States jurisdiction, and any subsequent legislation relating to the COVID-19 pandemic, including any executive memoranda or executive orders relating to the COVID-19 pandemic, the Health and

Economic Recovery Omnibus Emergency Solutions Act and the Health, Economic Assistance, Liability, and Schools Act, and the Memorandum for the Secretary of the Treasury signed on August 8, 2020.

"Clinical Trial" means a human clinical trial conducted for the purpose of gaining evidence of the safety, tolerability, pharmacokinetics, pharmacodynamics or efficacy of a Product required by applicable Law or otherwise recommended or required by a Regulatory Authority to obtain or maintain any Regulatory Approval for a Product.

"Closing" has the meaning set forth in Section 2.5(a).

"Closing Date" has the meaning set forth in Section 2.5(a).

"Closing Purchase Price" means an amount in cash equal to $1,000,000.

"Code" means the Internal Revenue Code of 1986, as amended.

"Combination Product" means a Product that is delivered with one or more additional active ingredients and/or other patentable items (which are themselves not products for a single price) incident to the administration of any such Product, including companion diagnostics, in each such case when any of the foregoing are co-formulated, copackaged or sold under one pricing scheme (whether payment of such price is paid to the same or to more than one seller).

"Commercially Reasonable Efforts" means, with respect to the efforts to be expended, or considerations to be undertaken, by Buyer with respect to any objective, activity or decision to be undertaken hereunder, reasonable, good faith efforts to accomplish such objective, activity or decision as Buyer would normally use to accomplish a similar objective, activity or decision under similar circumstances, it being understood and agreed that with respect to Buyer's obligations under Section 5.1 such efforts and resources shall be [**]. "Commercially Reasonable Efforts" will be determined on a country-by- country basis for a particular Product in the Territory and is expected to change over time, reflecting changes in the status of such Product and the markets or countries in the Territory involved. For the avoidance of doubt, Buyer's use of "Commercially Reasonable Efforts" will not require Buyer to reperform any preclinical activities that were previously performed by Seller.

"Confidential Information" has the meaning set forth in Section 5.5(a)(ii).

"Confidentiality Agreement" means the Confidential Disclosure Agreement between Seller and Buyer, dated [**].

"Contemplated Transactions" means the transactions contemplated by this Agreement and any Related Document, including the Acquisition.

"Contracts" means any loan or credit agreement, bond, debenture, note, mortgage, indenture, lease, supply agreement, license agreement, development agreement, distribution agreement or other legally binding contract, agreement, obligation, commitment, arrangement, understanding, instrument, permit, franchise or license, whether oral or written.

"Control" including its various tenses and derivatives (such as "controlled" and "controlling") means (a) when used with respect to any Person, the possession, directly or indirectly, of the power to direct or cause the direction of the management and policies of such entity, whether through the ownership of voting securities, or by Contract, (b) when used with respect to any security, the possession, directly or indirectly, of the power to vote, or to direct the voting of, such security or the power to dispose of, or to direct the disposition of, such security and (c) when used with respect to any Intellectual Property Rights, possession of the right, whether directly or indirectly, and whether by ownership, license or otherwise, to assign or grant a license, sublicense or other right to or under such Intellectual Property Rights or to compel another to do so.

"Cover" "Covers" "Covered" or "Covering" means, with respect to a Product or other subject matter at issue and a relevant Patent, that Exploitation of such Product or other subject matter by such Person would, absent a license thereto, infringe such Patent (or, in the case of a claim of a patent application that has not yet issued, would infringe such claim if it were to issue without change).

"CTA" has the meaning set forth in the definition of IND.

"Dollars" or "$" means United States dollars.

"Enforceable" means, with respect to any Contract stated to be "Enforceable" by or against any Person, that such Contract is a legal, valid and binding obligation of such Person enforceable by or against such Person in accordance with its terms, except to the extent that enforcement of the rights and remedies created thereby is subject to bankruptcy, insolvency, reorganization, moratorium and other similar Laws of general application affecting the rights and remedies of creditors and to general principles of equity (regardless of whether enforceability is considered in a proceeding in equity or at law).

"European Territory" means the United Kingdom and any country in the EU.

"European Union" or "EU" means the economic, scientific and political organization of Member States of the European Union, as its membership may be constituted from time to time.

"Exchange Act" means the United States Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

"Excluded Assets" has the meaning set forth in Section 2.2(b).

"Excluded Liabilities" has the meaning set forth in Section 2.4(a).

"Exploit" or "Exploiting" means to make, have made, import, use, sell, offer for sale, and otherwise dispose of, including to discover, research, develop, test, register, modify, enhance, improve, manufacture, have manufactured, store, formulate, optimize, export, transport, distribute, commercialize, promote, market, have sold and otherwise dispose of. "Exploitation" means the act of Exploiting a product or product candidate.

"FA Asset" means Seller's proprietary gene therapy asset having the sequence set forth on Annex 1.1(d).

"FDA" means the United States Food and Drug Administration or any successor entity thereto.

"FDA Laws" has the meaning set forth in Section 3.10.

"FDCA" means the United States Federal Food, Drug, and Cosmetic Act, as amended, and the rules and regulations promulgated by FDA thereunder.

"Field" means the treatment or prevention of Friedreich's Ataxia.

"First Commercial Sale" means, with respect to a particular Product, the first sale of such Product to a Third Party in a country or region following receipt of Regulatory Approval in such country or region.

"First Stock Consideration Issuance" has the meaning set forth in Section 2.12.

"Fraud" means, with respect to any Person, an actual and intentional common law fraud with the intent to deceive another Person (including as an element that the other Person relied thereon to its detriment).

"Fully-Diluted As-Converted Basis" means the aggregate number of Solid Shares equal to (a) the number of outstanding Solid Shares, plus (b) the number of Solid Shares issuable pursuant to (i) vested and unvested restricted stock units, (ii) vested and unvested options (calculated using the treasury method of accounting), (iii) outstanding warrants (calculated using the treasury method of accounting), and (iv) the conversion of any then outstanding shares of Solid Preferred Stock, but in all cases excluding any restricted stock units issued and outstanding as of the Closing Date, the vesting of which is subject to the achievement of certain performance conditions, until such time as vesting conditions have been satisfied in accordance with the terms of such award.

"Fundamental Representations" means the representations and warranties set forth in Section 3.1 (Organization; Standing and Power), Section 3.2(a) (Authority), Section 3.2(d)(i) (Noncontravention of Seller's Certificate of Formation and Seller's Operating Agreement), Section 3.4 (Good Title; Sufficiency of Assets), Section 3.6 (excluding subsections (d), (e) and (k) thereof) (Intellectual Property), Section 3.9 (Taxes) and Section 3.12 (Brokers and Other Advisors).

"GAAP" means the United States generally accepted accounting principles in effect from time to time.

"Governmental Authority" means any instrumentality, subdivision, court, administrative agency, commission, bureau, department, official or other authority of any country, state, province, prefect, municipality, locality or other government or political subdivision thereof, or any multinational organization or authority, or any quasi-governmental, private body, mediator, arbitrator or arbitral body exercising any executive, legislative, judicial, quasi-judicial, regulatory,

taxing, importing, administrative or other governmental or quasi-governmental authority on behalf of a government or political subdivision thereof.

"Grant-Back Intellectual Property" means, with respect to a Reversion Product, all Technology and Intellectual Property Rights generated, developed, conceived or reduced to practice by or on behalf of Buyer or its Affiliates after the Closing Date that are (a) owned by Buyer or any of its Affiliates, and (b) necessary for or used with respect to, or Covering, such Reversion Product, in each case ((a) and (b)), as of the date of Buyer's receipt of the Reversion Notice, but specifically excluding any third party Intellectual Property Rights and any background or platform Technology not exclusively used for the Reversion Product.

"Greater Liability Cap" means an amount equal to (a) prior to IND Clearance, $[**], and (b) following IND Clearance, $[**].

"IND" means (a) with respect to the US Territory, an Investigational New Drug Application, as such term is defined under the FDCA and 21 CFR Part 312, (b) with respect to the European Union, a clinical trial authorization application ("CTA"), and (c) with respect to the UK or any other regulatory jurisdiction, the equivalent application to the applicable Regulatory Authority in each case ((a), (b) and (c)), the filing of which is necessary to initiate or conduct Clinical Trials of a pharmaceutical or biological product in such jurisdiction.

"IND Clearance" means, with respect to a Product, that (a) with respect to the US Territory, a particular IND for the Product has been allowed to go into effect by the FDA, as evidenced by the passage of a thirty (30) calendar day period after the date of the IND submission (or any amended submission if such amendment restarted the applicable thirty (30)-day period) without the FDA placing the IND on clinical hold, (b) with respect to the European Union, that a Member State to which an application for clinical trial authorization has been submitted (Member States concerned) has either: (i) notified the sponsor through the EU portal (the Clinical Trials Information System) that the Clinical Trial is authorized (including an authorization subject to conditions) in accordance with Article 8(1) of Regulation (EU) No. 536/2014, or (ii) not notified the sponsor that the clinical trial is authorized within the relevant periods referred to in Article 8(1) of Regulation (EU) No. 536/2014, with the result that the clinical trial is deemed to be authorized in accordance with Article 8(6) of Regulation (EU) No. 536/2014, and (c) with respect to the United Kingdom, the Medicines and Healthcare Products Regulatory Agency has confirmed its acceptance of an application for clinical trial authorization (including an acceptance subject to conditions) pursuant to the Medicines for Human Use (Clinical Trials) Regulations 2004.

"Indemnified Party" has the meaning set forth in Section 6.3(a).

"Indemnifying Party" has the meaning set forth in Section 6.3(a).

"Indemnity Liability Cap" means as of the date that any claim for indemnification is made by an Indemnified Party pursuant to Section 6.3, an amount equal to the aggregate amount of any payments actually received by Seller pursuant to Article II. For purposes of calculating the Indemnity Liability Cap with respect to Section 6.1(b)(ii), any Solid Shares will be valued at the VWAP (with the date of measurement being the date that the applicable Milestone Shares were issued pursuant to Section 2.6, if any). For purposes of calculating the Indemnity Liability Cap

with respect to Buyer's exercise of the set-off rights set forth in Section 6.5, if applicable, any Solid Shares will be valued at the VWAP (with the date of measurement being the date that the applicable indemnification claim has been made by a Buyer Indemnified Party pursuant to Section 6.3).

"Intellectual Property Rights" means all rights, title and interests in and to all intellectual property rights and proprietary rights of every kind and nature however denominated, throughout the world, including: (a) patents, patent applications, (including in each case any provisional, continuation, continuation-in-part, division, renewal, extension, supplemental protection certificate, reexamination or reissue thereof), industrial design rights, and statutory invention registrations (collectively, "Patents"); (b) registered and unregistered trademarks, trade dress, trade names, logos, brands, design rights, service marks, together with all common law rights and the goodwill pertaining to the foregoing, and all applications, registrations and renewals therefor; (c) registered and unregistered copyrights, works of authorship, copyrightable works (published or unpublished), software, moral rights and all applications and registrations therefore; (d) domain names and social media accounts and handles; (e) software, computer programs and applications (whether in source code, object code, firmware or other form) algorithms, data and databases (including clinical data), together with all specifications, designs, documentation and Technology supporting the foregoing and all other proprietary rights in Technology; (f) trade secrets, invention disclosures, technical know-how (including processes, methods, techniques, designs, protocols, formulae, algorithms, compositions, specifications, and research and development information), and other proprietary information ("Trade Secrets") and other proprietary rights and intellectual property rights; and (g) any and all registrations, applications, recordings, licenses, common-law rights, statutory rights, administrative rights, and contractual rights relating to any of the foregoing, including the right to sue and collect for past, present and future damages.

"IRS" means the Internal Revenue Service of the United States.

"JSC" has the meaning set forth in Section 5.3(a).

"Law" means any federal, state, local or foreign constitution, treaty, law (including common law), statute, ordinance, rule, regulation, interpretation, binding guidance, advisory, published opinion, directive, policy, Order, writ, award, decree, injunction, judgment, stay or restraining order of any Governmental Authority, the terms of any permit, and any other ruling or decision of, agreement with or by, or any other requirement of, any Governmental Authority.

"Liability" or "Liabilities" means, with respect to any Person, any and all damages, debts, liabilities, obligations, losses, claims, Taxes, interest obligations, deficiencies, judgments, assessments, awards, fines, fees, penalties, costs and expenses, whether accrued or fixed, absolute or contingent, known or unknown, matured or unmatured or determined or determinable, due or to become due, whether directly incurred or consequential, whether or not required under GAAP to be accrued on the financial statements of such Person, and including those arising under any Law, Action or Order and those arising under any Contract, agreement, arrangement, commitment or undertaking.

"Lien" means any lien (statutory or otherwise), security interest, pledge, hypothecation, mortgage, assessment, lease, claim, levy, license, defect in title, charge, or any other Third Party

right, license or property interest of any kind, or any conditional sale or other title retention agreement, right of first option, right of first refusal or similar restriction, any covenant not to sue, or any restriction on use, transfer, receipt of income or exercise of any other attribute of ownership or any agreement to give any of the foregoing in the future or similar encumbrance of any kind or nature whatsoever.

"Lock-Up Period" has the meaning set forth in Section 5.10(a).

"Losses" of a Person means any and all losses, debts, obligations, Liabilities, Liens, penalties, damages, awards, judgments, Taxes, costs and expenses (including reasonable and documented attorneys' and experts' fees) of such Person.

"Member Approval" has the meaning set forth in Section 3.2(c).

"Milestone 1 Cash Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 1 Private Cash Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 1 Share Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 2 Cash Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 2 Private Cash Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 2 Share Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 3 Cash Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 3 Private Cash Consideration" has the meaning set forth in Section 2.6(a).

"Milestone 3 Share Consideration" has the meaning set forth in Section 2.6(a).

"Milestone Consideration" has the meaning set forth in Section 2.6(a).

"Milestone Event" has the meaning set forth in Section 2.6(a).

"Milestone Invoice" has the meaning set forth in Section 2.6(b).

"Milestone Shares" means the Solid Shares issuable by Buyer as Milestone 1 Share Consideration, Milestone 2 Share Consideration or Milestone 3 Share Consideration (as applicable).

"Milestones" means each of the Milestone Events set forth in Section 2.6.

"Nasdaq" means the Nasdaq Global Select Market.

"Negotiation Period" has the meaning set forth in Section 5.11(a).

"Net Sales" means with respect to a Product sold to a Third Party, the gross consideration invoiced or received by Buyer or any of its Affiliates or licensees for Sales of Products (including any cash amounts plus the fair market value of any other forms of consideration), less the following deductions (to the extent included in and not already deducted from the gross amounts invoiced or otherwise charged) to the extent reasonable and customary:

[**].

Even if there is overlap between any of deductions described above, a particular deduction shall only be deducted once in the overall Net Sales calculation.

In the case of a Combination Product, Buyer and Seller shall negotiate in good faith, but in no event later than [**] before the expected launch of such Combination Product, an allocation of Net Sales of such Combination Product to the respective Product components and other component(s) thereof, as the case may be, based on the fair market value of such components for the purposes of determining a Product specific allocation of such Net Sales. Payments related to such Combination Product under this Agreement, including Royalties and Milestone Consideration, will be calculated, due and payable based only on such allocated Net Sales.

In case of disagreement and failure by the Parties to agree upon an allocation of Net Sales of such Combination Product to the respective Product components and other component(s) thereof, an independent expert agreed upon and paid by both Parties shall determine such relative value contributions and such determination shall be final and binding upon the Parties.

"Order" means any writ, judgment, injunction, order, decree, stipulation, ruling, decision, verdict, determination or award, of or by, or any settlement under the jurisdiction of, any Governmental Authority (in each such case whether preliminary or final).

"Outstanding Solid Shares" means all Solid Capital Stock, determined on a Fully-Diluted As-Converted Basis, measured as of the date of the achievement of the applicable Milestone Event.

"Owed Damages" has the meaning set forth in Section 6.3(c).

"Owned Intellectual Property" means all Seller Intellectual Property that is owned by Seller as of the Closing Date.

"Party" or "Parties" has the meaning set forth in the preamble hereof.

"Patents" has the meaning set forth in the definition of Intellectual Property Rights.

"Permitted Liens" has the meaning set forth in Section 2.1.

"Person" means an individual, corporation, partnership, limited liability company, joint venture, association, trust, unincorporated organization or other entity or any Governmental Authority.

"PHSA" means the Public Health Service Act, as amended, and the rules and regulations promulgated by FDA thereunder.

"Pre-Closing Tax Period" means (a) any Tax period ending on or before the Closing Date, and (b) with respect to a Tax period that commences on or before but ends after the Closing Date, the portion of such period up to and including the Closing Date.

"Pricing and Reimbursement Approval" means, in a country or other jurisdiction where Governmental Authorities in such country or jurisdiction approve or determine the price that can be charged for a product in such country or jurisdiction, or that can be reimbursed by Governmental Authorities for such product in such country or jurisdiction, (a) the approval, agreement, determination, or decision by the relevant Governmental Authority(ies) establishing the price that can be legally charged to consumers for such product in such country or jurisdiction; or (b) the approval, agreement, determination, or decision by the relevant Governmental Authority(ies) establishing the level of reimbursement that will be reimbursed by Governmental Authorities for such product in such country or jurisdiction.

"Prime Rate" means, as of the date of this Agreement, the rate of interest which is identified in The Wall Street Journal as the "Prime Rate" in the United States.

"Product Inventory" means all inventory of the Products, including inventory of work-in-process, finished goods, raw materials, supplies, parts, labels and packaging, working prototypes, and samples (including rights and interests in goods in transit, consigned inventory, inventory sold on approval and rental inventory), in each case, in the possession or control of Seller or its Affiliates. The Product Inventory existing as of the Closing Date is set forth on Annex 1.1(b).

"Products" means any product (a) containing or comprising the FA Asset, whether alone or in combination with one or more additional components, in all forms, formulations and dosage forms, and (b) any product, service, article, composition, apparatus, chemical, substance or any other material made, used or sold by utilizing or practicing Seller Intellectual Property.

"Purchase Price Allocation" has the meaning set forth in Section 2.11.

"Purchased Assets" has the meaning set forth in Section 2.1.

"Registrable Securities" has the meaning set forth in Section 2.6(d)(v).

"Registration Statement" has the meaning set forth in Section 2.6(d)(v).

"Registrational Clinical Trial" means a Phase III Clinical Trial or other Clinical Trial of a Product that would satisfy the requirements of 21 C.F.R. 312.21(c) and that is designed or intended to (a) establish that the product is safe and efficacious for its intended use, (b) define warnings, precautions and adverse reactions that are associated with the product in the dosage range to be prescribed, and (c) support the submission of a BLA or similar application for Regulatory Approval for such Product in the Territory.

"Regulatory Approval" means, with respect to a country or jurisdiction in the Territory, any approval, authorization, or permit granted by the applicable Regulatory Authority that is necessary for the manufacture, marketing, importation and sale of a Product in such country or jurisdiction, which may include satisfaction of all applicable regulatory and notification requirements, including, where applicable, (a) Pricing and Reimbursement Approval(s), solely to

the extent necessary to commence marketing in such jurisdiction, (b) pre- and post-approval marketing authorizations (including any prerequisite manufacturing approval or authorization related thereto) or (c) approval of product labeling.

"Regulatory Authority" means any applicable Governmental Authority with authority over the distribution, importation, exportation, manufacture, production, use, storage, transport, clinical testing or sale of a pharmaceutical or biological product, including any Governmental Authority having the authority to grant Regulatory Approval or Pricing and Reimbursement Approval.

"Regulatory Documentation" means, with respect to a Product, all applications for and documentation relating to INDs or Regulatory Approvals for such Product, all material correspondence submitted to or received from Governmental Authorities in connection with INDs or Regulatory Approvals for such Product, and all source clinical trial data and safety data submitted to Governmental Authorities in connection with INDs or Regulatory Approvals for such Product.

"Regulatory Exclusivity" means, with respect to any country or other jurisdiction in the Territory, an additional market protection granted by a Governmental Authority in such country or other jurisdiction that confers an exclusive commercialization period during which a Person has the exclusive right to market and sell a Product in such country or other jurisdiction.

"Related Documents" means, other than this Agreement, all agreements, certificates and documents signed and delivered by either Party in connection with the signing of this Agreement or the Closing.

"Releasee" has the meaning set forth in Section 7.12.

"Releasors" has the meaning set forth in Section 7.12.

"Representatives" means, with respect to any Person, such Person's directors, officers, equity holders, members, managers, employees, counsel, consultants, accountants, financial advisors, lenders and other agents and representatives.

"Reversion Agreement" has the meaning set forth in Section 5.11(a)(i).

"Reversion Notice" has the meaning set forth in Section 5.11(a).

"Reversion Product" means any Product in the form such Product exists as of the date of Buyer's receipt of the Reversion Notice.

"Reversion Rights" has the meaning set forth in Section 5.11(a).

"Royalties" has the meaning set forth in Section 2.7(a)(i).

"Royalty Report has the meaning set forth in Section 2.7(c).

"Royalty Term" has the meaning set forth in Section 2.7(a)(ii).

"Sale" means any transaction (other than a license or sublicense) for which consideration is received or invoiced by Buyer, its Affiliates or licensees for sale, use, lease, transfer or other disposition of a Product to or for the benefit of a Third Party. For clarity, the sale, use, lease, transfer or other disposition of a Product by Buyer or any of its Affiliates or licensees to another of these entities for resale by such entity to a Third Party shall not be deemed a Sale.

"SEC" means the United States Securities and Exchange Commission.

"SEC Filings" has the meaning set forth in Section 4.10(a).

"Second UPenn Amendment" has the meaning set forth in the definition of the "UPenn License."

"Securities Act" means the Securities Act of 1933, as amended, and the rules and regulations promulgated thereunder.

"Seller" has the meaning set forth in the preamble hereof.

"Seller Disclosure Schedules" means the Schedules delivered by Seller to Buyer contemporaneously with this Agreement and appended hereto, setting forth disclosures in respect of the representations and warranties contained in Article III of this Agreement.

"Seller Indemnified Party" has the meaning set forth in Section 6.2(a).

"Seller Indemnity Threshold" has the meaning set forth in Section 6.1(b).

"Seller Intellectual Property" means (a) all Technology owned leased, licensed, or Controlled by Seller, including all such Technology that is related to the Business or the FA Asset or used, or held for use, by Seller in connection with the Exploitation of FA Asset, and (b) all Intellectual Property Rights (other than Intellectual Property Rights constituting Excluded Assets) owned, licensed by or otherwise Controlled by Seller, including all such Intellectual Property Rights that are related to the Business or used, or held for use, by Seller in connection with the Exploitation of the FA Asset, and the right to recover for past, present and future infringement of any of the foregoing. The Seller Intellectual Property Rights existing as of the Closing Date are set forth on Annex 1.1(c).

"Seller Representation Letter" has the meaning set forth in Section 2.6(d)(iii).

"Seller's Certificate of Formation" has the meaning set forth in Section 3.1.

"Seller's Knowledge" (and similar phrases) means, with respect to any matter in question, the actual knowledge of Thomas Hamilton as of the Closing Date after reasonable inquiry of all Seller's officers and managers reasonably expected to have actual knowledge of such fact or matter.

"Seller's Operating Agreement" has the meaning set forth in Section 3.1.

"Seller's Secretary's Certificate" means a certificate, dated as of the Closing Date, duly executed by the secretary of Seller, certifying that: (a) all documents to be executed by Seller and delivered at the Closing have been executed by a duly authorized officer of Seller, (b) (i) Seller's Certificate of Formation and Seller's Operating Agreement, attached to the certificate, are true, accurate and complete, (ii) such organizational documents have been in full force and effect in the form attached since the date of the adoption of the resolutions referred to in clause (iii) below and no amendment, rescission or modification to such organizational documents has occurred since the date thereof, (iii) the resolutions adopted by the board of managers of Seller, authorizing the execution, delivery and performance of this Agreement and the Related Documents to which Seller is a party, as attached to the certificate, were duly adopted by unanimous written consent of the board of managers of Seller, remain in full force and effect, and have not been amended, rescinded or modified and (iv) the resolutions adopted by the members of Seller necessary to approve and adopt this Agreement and the Related Documents to which Seller is a party, authorizing the execution, delivery and performance of this Agreement and the Related Documents to which Seller is a party, as attached to the certificate, were duly adopted by such members at a duly convened meeting of the members or by written consent of the members of Seller duly adopted, remain in full force and effect, and have not been amended, rescinded or modified and (c) Seller's officer executing this Agreement, and each of the other documents necessary for consummation of the Contemplated Transactions, is an incumbent officer, and the specimen signature on such certificate is a genuine signature.

"Share Issuance Cap" has the meaning set forth in Section 2.6(c).

"Solid Capital Stock" means, collectively, the Solid Shares and Solid Preferred Stock.

"Solid Preferred Stock" means the preferred stock, par value $0.001 per share, of Buyer.

"Solid Shares" means the common stock, par value $0.001 per share, of Buyer.

"Subsidiary" of any Person means another Person, an amount of the voting securities, other voting rights or voting partnership interests of which is sufficient to elect at least a majority of its board of directors or other governing body (or, if there are no such voting interests, 50% or more of the equity interests of which) is owned directly or indirectly by such first Person.

"Tax" or "Taxes" means (whether disputed or not) all (a) federal, state, local and foreign income, franchise, windfall or other profits, gross receipts, property, escheat or unclaimed property, ad valorem, sales, use, excise, withholding, payroll, employment, social security, unemployment compensation, disability, severance, capital gain, alternative minimum, estimated, transfer and other taxes and similar governmental charges, including any interest, penalties and additions with respect thereto, (b) Liability for the payment of any amounts of the type described in clause (a) as a result of being a member of an affiliated, consolidated, combined, unitary or aggregate group or as a transferee or successor and (c) Liability for the payment of any amounts as a result of being party to any tax sharing agreement or as a result of any express or implied obligation to indemnify any other Person with respect to the payment of any amounts of the type described in clause (a) or (b).

"Tax Return" means all returns, requests for extensions of time, claims for refund, declarations of estimated Tax payments, reports, estimates, information returns and statements, filed or to be filed with any Taxing Authority in connection with the determination, assessment, collection or administration of any Taxes, including any amendments thereto as well as any related or supporting information with respect to any of the foregoing.

"Taxing Authority" means any federal, state, local or foreign government, any subdivision, agency, commission or authority thereof, or any quasi-governmental body exercising tax regulatory authority.

"Technology" means all inventions, works, discoveries, innovations, know-how, information (including ideas, research and development, formulas, algorithms, compositions, processes and techniques, data (including clinical data), designs, drawings, lab records and notebooks, specifications, graphics, documentation and manuals), equipment, and all other forms of technology, business materials, and embodiments of Intellectual Property Rights, embodied in any form, whether or not protected by patent, copyright, trade secret Law, or otherwise, and all documents and other materials recording any of the foregoing.

"Technology Transfer" has the meaning set forth in Section 5.4.

"Territory" means worldwide.

"Third Party" means any Person other than: (a) Seller or Buyer or (b) any Affiliates of Seller or Buyer.

"Third Party Claim" has the meaning set forth in Section 6.1(a).

"Trade Secrets" has the meaning set forth in the definition of Intellectual Property Rights.

"Transfer" has the meaning set forth in Section 5.10(a).

"Transfer Taxes" has the meaning set forth in Section 5.6(a).

"UPenn" means The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation.

"UPenn IND Payments" means those certain payments set forth on Schedule A to the Second UPenn Amendments that are listed on Annex 1.1(e).

"UPenn License" means that certain Research, Collaboration and License Agreement by and between Seller and UPenn, dated as of November 4, 2020 and amended by (a) the First Amendment to the Research, Collaboration and License Agreement dated November 1, 2021 and (b) the Second Amendment to the Research, Collaboration and License Agreement dated August 6, 2024 (the "Second UPenn Amendment").

"US Territory" means the United States of America and its territories and

possessions.

"Valid Claim" means a claim of any issued and unexpired Patent within the Seller Intellectual Property whose validity, enforceability or patentability has not been affected by any of the following: (a) irretrievable lapse, abandonment, revocation, dedication to the public or disclaimer or (b) a holding, finding or decision of invalidity, unenforceability or non-patentability by a court, governmental agency, national or regional patent office or other appropriate body that has competent jurisdiction, such holding, finding or decision being final and unappealable or not appealed within the time allowed for appeal.

"Voting Rights Agreement" has the meaning set forth in Section 2.12.

"VWAP" means, as of a specific date, the volume-weighted average price per Solid Share, as reported on Nasdaq, during the consecutive [**] trading days immediately preceding the specified date, as calculated by Bloomberg Financial LP under the function "VWAP".

Section 1.2. Interpretation. When a reference is made in this Agreement to an Article, a Section, Exhibit, Schedule or Annex, such reference will be to an Article of, a Section of, or an Exhibit, Schedule or Annex to, this Agreement unless otherwise indicated. Any exception set forth in a section or subsection of any Annex or any of the Schedules will be deemed to be disclosed for purposes of, and will qualify, such section or subsection of this Agreement and any other section or subsection of this Agreement where it is reasonably apparent on the face of such exception that such exception would be applicable to such other section or subsection. The table of contents and headings contained in this Agreement, any Related Document or in any Exhibit, Schedule or Annex hereto are for reference purposes only and will not affect in any way the meaning or interpretation of this Agreement, such Related Document or such Exhibit, Schedule or Annex. Whenever the words "include", "includes" or "including" are used in this Agreement or any Related Document, they will be deemed to be followed by the words "without limitation." The word "or," when used in this Agreement, has the inclusive meaning represented by the phrase "and/or." The words "hereof", "herein" and "hereunder" and words of similar import when used in this Agreement will refer to this Agreement as a whole and not to any particular provision of this Agreement. References to the "date hereof" refer to the date of this Agreement. "Extent" in the phrase "to the extent" means the degree to which a subject or other thing extends, and such phrase does not mean simply "if". All terms defined in this Agreement will have the defined meanings when used in any certificate or other document made or delivered pursuant hereto unless otherwise defined therein. The definitions contained in this Agreement are applicable to the singular as well as the plural forms of such terms and to the masculine as well as to the feminine and neuter genders of such term. Any Contract or statute defined or referred to herein or in any Contract that is referred to herein means (a) in the case of any statute, such statute and any comparable statute that from time to time replaces such statute by succession and any rules or regulations promulgated by an applicable Governmental Authority thereunder and (b) in the case of any Contract, such Contract and all amendments, modifications and attachments thereto and instruments incorporated therein. References to a Person are also to its permitted successors and assigns.

ARTICLE II

PURCHASE AND SALE

Section 2.1. Purchase and Sale of Purchased Assets. Upon and subject to the terms and conditions hereof, at the Closing, Buyer shall purchase from Seller, and Seller shall sell, convey, assign, transfer and deliver to Buyer, all of Seller's right, title and interest in and to all assets, properties, rights, titles and interests of every kind and nature owned, licensed or leased by Seller (including indirect and other forms of beneficial ownership) as of the Closing Date that are used (or held for use) in connection with, related to, useful or otherwise necessary for the conduct of the Business, whether tangible or intangible, real or personal and wherever located (the "Purchased Assets"), free and clear of all Liens, other than the Liens set forth on Annex 2.1 ("Permitted Liens"), and agrees to assume, satisfy and discharge when due all Assumed Liabilities. The purchase and sale of the Purchased Assets hereunder is referred to herein as the "Acquisition."

Section 2.2. Purchased Assets; Excluded Assets.

(a) Without limiting the generality of Section 2.1, the Purchased Assets include all of Seller's right, title and interest in and to the following assets:

(i) all assets related to, or used in, the Business, including, but not limited to, the FA Asset;

(ii) all Seller Intellectual Property;

(iii) (A) the UPenn License and (B) all Contracts set forth on Annex 2.2(a)(iii)((A) and (B), together, the "Assigned Agreements");

(iv) all Product Inventory;

(v) all Books and Records; provided, that Seller may retain copies of the Books and Records to the extent necessary to comply with applicable Law or Seller's bona fide internal policies with respect to record-keeping obligations; and

(vi) all claims, counterclaims, credits, Actions, rights of recovery, and rights of indemnification or setoff against Third Parties and other claims arising out of or relating to the Purchased Assets and all other intangible property rights that relate to any Purchased Asset or any Assumed Liabilities.

(b) Other than the Purchased Assets, Buyer will not acquire any right, title or interest in or to any assets, properties, goodwill, and rights of Seller and its Affiliates, including the following assets (collectively, the "Excluded Assets"):

(i) any assets, properties, goodwill and rights of Seller and its Affiliates, in each case, that do not relate to the Business; and

(ii) all Contracts other than the Assigned Agreements.

Section 2.3. Assumption of Certain Liabilities. Subject to the terms and conditions set forth herein, Buyer agrees, effective at and after the Closing, to assume and to timely satisfy and discharge all Liabilities:

(a) relating to the Purchased Assets, (including Liabilities under any Assigned Agreement), but only to the extent that such Liabilities (i) arise from any act or omission at or after the Closing, (ii) do not arise from any act or omission prior to the Closing, and (iii) with respect to any Assigned Agreement, such Assigned Agreement is assigned to Buyer or Buyer otherwise receives the rights and benefits of such Assigned Agreement pursuant to Section 2.2(a)(iii), and in each case specifically excluding any Liability or obligation arising from any act or omission prior to the Closing or relating to or arising out of the Purchased Assets as a result of: (A) any breach of an Assigned Agreement occurring prior to Closing; (B) any violation of law, breach of warranty, tort or infringement occurring prior to the Closing; or (C) any charge, compliant, action, suit, proceeding, hearing, investigation, claim or demand occurring prior to the Closing or relating to the facts or circumstances occurring prior to Closing;

(b) resulting from the ownership, use, operation or maintenance of the Purchased Assets and/or the Exploitation of Products by or on behalf of Buyer, in each case, only to the extent arising at or after the Closing Date; and

(c) set forth on Annex 2.3 hereto (such Liabilities set forth in the foregoing (a), (b) and (c), collectively, the "Assumed Liabilities").

Section 2.4. Excluded Liabilities.

(a) Buyer will not be the successor to Seller or any of its Affiliates and Buyer expressly does not assume and will not become liable to pay, perform or discharge, any Liability whatsoever of Seller or any of its Affiliates other than the Assumed Liabilities (collectively, the "Excluded Liabilities"). Notwithstanding anything to the contrary in this Agreement, Excluded Liabilities will include (i) any Liabilities for Taxes relating to, or arising out of or imposed on the Business or the Purchased Assets for any Pre-Closing Tax Period, (ii) any Liabilities for Taxes of Seller or any of its Affiliates for any taxable period, (iii) any Liabilities of Seller for the payment of any amounts of Tax as a result of being a member of an affiliated, consolidated, combined or unitary group, as a result of any Tax sharing or Tax allocation agreement, arrangement or understanding, or as a result of being liable for another Person's Taxes as a transferee or successor, by Contract or otherwise, (iv) any Liabilities relating to, or arising out of the employment or engagement, or termination of the employment or engagement, of any employee or other service provider of Seller or any of its Affiliates at any time and (v) the UPenn IND Payments.

Section 2.5. Closing; Closing Deliverables.

(a) Closing. The closing of the Acquisition (the "Closing") will take place on the date hereof via an electronic exchange of documents (the "Closing Date").

(b) Seller Closing Deliverables. Prior to the Closing, Seller will deliver or cause to be delivered to Buyer or to Buyer's designee:

(i) the Bill of Sale, dated the Closing Date and duly executed by Seller;

(ii) a duly completed and executed IRS Form W-9 of Seller, or, if Seller is a disregarding entity for U.S. federal income tax purposes, a duly completed and executed IRS Form W-9 or IRS Form W-8 as appropriate of Seller's regarded owner;

(iii) Seller's Secretary's Certificate, dated the Closing Date;

(iv) the Member Approval;

(v) the assignments, consents, waivers, approvals and authorizations, in form and substance reasonably satisfactory to Buyer, relating to those items set forth on Annex 2.5(b)(v);

(vi) all bills of sale, instruments of assignment and other transfer documentation as reasonably requested by Buyer, in form and substance reasonably satisfactory to Buyer, pursuant to which all of Seller's right, title and interest in and to Seller Intellectual Property and other Purchased Assets are transferred to Buyer;

(vii) all tangible Purchased Assets in Seller's possession and control as of the Closing Date (if any); and

(viii) each of the items and materials listed on Section 2.5(b)(viii) of the Seller Disclosure Schedules.

(c) Buyer Closing Deliverables. At the Closing, Buyer will deliver or cause to be delivered to Seller the Bill of Sale.

(d) Payments by Buyer at Closing. In consideration of the sale, conveyance, delivery, transfer and assignment of the Purchased Assets to Buyer and Seller's other covenants and obligations hereunder, Buyer and its applicable Affiliates will pay, or cause to be paid, to Seller, by wire transfer of immediately available funds to the account or accounts specified by Seller to Buyer, an amount equal to the Closing Purchase Price no later than [**] following the Closing Date; provided that such [**] period shall not commence until the date that Buyer has received all required IRS Form W-9s or IRS Form W-8s in order to process the payment of the Closing Purchase Price.

Section 2.6. Developmental and Sales Milestones.

(a) Subject to adjustment pursuant to this Section 2.6 and Section 6.5, Buyer will make the non-refundable cash payments or issuances of Solid Shares (or combination thereof, as applicable) described in Table 1 below (each, "Milestone Consideration") following the first achievement by Buyer of the corresponding event (each a "Milestone Event") described in the column to the left of such payment in the table below.

Table 1		
No.	**Milestone Event**	**Milestone Consideration**
1	First IND Clearance of a Product	At the election of Buyer, (a) either (i) a number of Solid Shares representing [**]% of the Outstanding Solid Shares or (ii) a number of Solid Shares equal

Table 1		
No.	**Milestone Event**	**Milestone Consideration**
		to $5,000,000 in value, whichever is valued higher at the time of achievement of Milestone Event No. 1 (provided, that in no event will any Solid Shares paid pursuant to the foregoing (i) or (ii) exceed a value of $[**] or [**]% of the Outstanding Solid Shares, as applicable) (the "Milestone 1 Share Consideration"), (b) cash, in an amount equal to the consideration to be paid pursuant to the foregoing clause (a) in lieu of payment in Solid Shares (the "Milestone 1 Cash Consideration"), or (c) a combination of (a) and (b), in an amount equal to the consideration to be paid pursuant to the foregoing clause (a). If, as of the date Milestone Event No. 1 is achieved, Buyer is no longer a publicly traded company, Buyer will pay to Seller cash in the amount of $5,000,000 in lieu of payment as described in the foregoing (a) - (c) (the "Milestone 1 Private Cash Consideration").
2	[**]	At the election of Buyer, (a) either (i) a number of Solid Shares representing [**]% of the Outstanding Solid Shares or (ii) a number of Solid Shares equal to $[**] in value, whichever is valued higher at the time of achievement of Milestone Event No. 2 (provided, that in no event will any Solid Shares paid pursuant to the foregoing (i) or (ii) exceed a value of $[**] or [**]% of the Outstanding Solid Shares, as applicable) (the "Milestone 2 Share Consideration"), (b) cash, in an amount equal to the consideration to be paid pursuant to the foregoing clause (a), in lieu of payment in Solid Shares (the "Milestone 2 Cash Consideration"), or (c) a combination of (a) and (b), in an amount equal to the consideration to be paid pursuant to the foregoing clause (a). If, as of the date Milestone Event No. 2 is achieved, Buyer is no longer a publicly traded company, Buyer will pay to Seller cash in the amount of $[**] in lieu of payment as described in the foregoing (a) - (c) (the "Milestone 2 Private Cash Consideration").
3	[**]	At the election of Buyer, (a) either (i) a number of Registrational Clinical Trial Solid Shares representing [**]% of the Outstanding Solid Shares or (ii) a number of Solid Shares equal to $[**] in value, whichever is valued higher at the time of achievement of Milestone Event No. 3 (provided,

Table 1		
No.	**Milestone Event**	**Milestone Consideration**
		that in no event will any Solid Shares paid pursuant to the foregoing (i) or (ii) exceed a value of $[**] or [**]% of the Outstanding Solid Shares, as applicable) (the "Milestone 3 Share Consideration"), (b) cash, in an amount equal to the consideration to be paid pursuant to the foregoing clause (a), in lieu of payment in Solid Shares (the "Milestone 3 Cash Consideration"), or (c) a combination of (a) and (b), in an amount equal to the consideration to be paid pursuant to the foregoing clause (a). If, as of the date Milestone Event No. 3 is achieved, Buyer is no longer a publicly traded company, Buyer will pay to Seller cash in the amount of $[**] in lieu of payment as described in the foregoing (a) - (c) above (the "Milestone 3 Private Cash Consideration").
4	[**]	$[**] in cash.
5	[**]	$[**] in cash.
6	Annual Net Sales in the Territory are equal to or greater than $[**] and less than or equal to $[**]	$[**] in cash.
7	Annual Net Sales in the Territory are greater than $[**] and less than or equal to $[**]	$[**] in cash.
8	Annual Net Sales in the Territory are greater than $[**]	$[**] in cash.

(b) Promptly upon the achievement of a Milestone Event, and in any event within [**], Buyer will notify Seller of the achievement of such Milestone Event. Promptly following, and in any event within [**] following such notice from Buyer of the achievement of such Milestone Event, Seller will submit an invoice to Buyer for the amount of the corresponding Milestone Consideration (each, a "Milestone Invoice"), and Buyer will pay the applicable Milestone Consideration by wire transfer of immediately available funds or issuance of Solid Shares to Seller, or both, as applicable, no later than the date that is [**] after the date of such Milestone Invoice.

(c) For clarity, if any of the Milestone Event No. [**] has not been achieved at the time of the [**], then any such Milestone shall be deemed achieved and the corresponding Milestone Consideration shall become due and payable. Notwithstanding the foregoing, each Milestone Consideration payment is payable only once, upon the first achievement (or deemed achievement) of the applicable Milestone Event, regardless of any subsequent or repeated achievements of Milestone Events with respect to one or more of the same or different Products. For the avoidance of doubt, (i) the maximum aggregate value of Milestone Consideration payable

with respect to Milestone Events No. [**] through No. [**] under this Agreement will be $34,000,000 or [**]% of the Outstanding Solid Shares (which $34,000,000 or [**]% represents the aggregate value or percentage of Outstanding Solid Shares measuring each issuance of such Milestone Shares in connection with the achievement of Milestone Events No. [**] through No. [**] as of the date such Milestones were achieved), as applicable, and (ii) the maximum aggregate payments of Milestone Consideration payable with respect to Milestone Events No. [**] through No. [**] under this Agreement will be $21,000,000. In no event will the aggregate amount of Solid Shares issued as Milestone Consideration pursuant to this Agreement exceed 19.9% of all of the Solid Shares issued and outstanding as of the Closing Date (the "Share Issuance Cap"); provided, that if Buyer elects to issue an amount of Solid Shares as Milestone Consideration equal to the Share Issuance Cap, then, thereafter, any payment of Milestone Consideration or portion thereof payable to Seller will be paid in cash, subject and pursuant to this Section 2.6. For purposes of determining any Milestone Consideration payable pursuant to Section 2.6(a), so long as the Solid Shares are publicly traded on a U.S. national exchange, Solid Shares shall be valued at the VWAP for such Solid Shares as of the date such Milestone Event was achieved. For the avoidance of doubt:

(i) with respect to Milestone Event No. 1, Buyer will only be required to pay, and Seller will only be entitled to receive, one of the Milestone 1 Cash Consideration, the Milestone 1 Share Consideration or the Milestone 1 Private Cash Consideration. In no event will Buyer be required to pay, or will Seller be entitled to receive, more than one of the foregoing payments for achievement of Milestone Event No. 1;

(ii) with respect to Milestone Event No. 2, Buyer will only be required to pay, and Seller will only be entitled to receive, one of the Milestone 2 Cash Consideration, the Milestone 2 Share Consideration or the Milestone 2 Private Cash Consideration. In no event will Buyer be required to pay, or will Seller be entitled to receive, more than one of the foregoing payments for achievement of Milestone Event No. 2; and

(iii) with respect to Milestone Event No. Event No. 3, Buyer will only be required to pay, and Seller will only be entitled to receive, one of the Milestone 1 Cash Consideration, the Milestone 3 Share Consideration or the Milestone 3 Private Cash Consideration. In no event will Buyer be required to pay, or will Seller be entitled to receive, more than one of the foregoing payments for achievement of Milestone Event No. 3.

(d) In the event that Buyer elects to pay any Milestone Consideration in the form of Solid Shares pursuant to Section 2.6(a):

(i) notwithstanding anything to the contrary herein, no fractional Solid Shares will be issued as Milestone Consideration and any portion of Solid Shares representing fractional shares that would otherwise be issued as Solid Shares will instead be rounded up to the nearest whole Solid Share prior to the issuance of such Solid Shares to Seller;

(ii) Seller acknowledges and understands that any Solid Shares issued to Seller as Milestone Consideration are issued to Seller pursuant to a "private placement" exemption or exemptions from registration under Section 4(a)(2) of the Securities Act and/or Regulation D promulgated under the Securities Act and an exemption from qualification under applicable state securities Laws. Such Solid Shares will be "restricted securities" under the United States federal and state securities Laws and cannot be offered or resold except pursuant to registration under the Securities Act and applicable state securities Laws or an available exemption from registration. Seller further acknowledges that if an exemption from registration or qualification is available with respect to the resale by it of the Solid Shares, it may be conditioned on compliance with applicable Laws, which may include the time and manner of sale and the holding period for the shares of Solid Shares;

(iii) prior to receipt of any such Solid Shares, Seller will execute a representation letter in substantially the form of Exhibit B-1 (the "Seller Representation Letter") and Buyer will execute a representation letter in substantially the form of Exhibit B-2 (the "Buyer Representation Letter");

(iv) Seller acknowledges that the certificates representing the Solid Shares will bear the following legends or legends substantially similar:

> "the shares represented by this certificate have not been registered under the Securities Act of 1933 (the "Act") and may not be offered, sold or otherwise transferred, assigned, pledged or hypothecated unless registered under the Act or unless an exemption from the registration requirements of the Act is available"; and

(v) Buyer shall file within [**] of the date of the achievement of Milestone Event No. 1, Milestone Event No. 2 and Milestone Event No. 3, with respect to any Solid Shares that Buyer determines to issue as Milestone Consideration, a registration statement on Form S-3 (or any successor form to Form S-3) registering the resale of such Solid Shares (the "Registrable Securities") (or, in the event that (i) Form S- 3 is not available for the registration of the resale of the Registrable Securities, another appropriate form reasonably acceptable to Seller or (ii) if available, include the Registrable Securities in a prospectus supplement under an already effective registration statement of Buyer) by Seller (a "Registration Statement"). Buyer shall use commercially reasonable efforts (a) to cause the Registration Statement to become effective as promptly as practicable; and (b) to cause the Registration Statement to remain effective until the earlier of (i) the date on which Seller has disposed of all of the Registrable Securities and (ii) the date on which Rule 144 promulgated under the Securities Act is available for the disposition of such Registrable Securities without volume or manner-of-sale restrictions. Seller agrees to cooperate with Buyer as reasonably requested by Buyer in connection with the preparation and filing of the Registration Statement, including furnishing to Buyer such information regarding itself, the Solid Shares held by it and the intended method of disposition of the Registrable Securities as shall be reasonably required to effect the registration of such Registrable Securities. Buyer shall bear all expenses incurred in connection with the filing

of the Registration Statement; provided, however, that Buyer shall have no obligation to pay for any commissions or transfer taxes of Seller.

(e) The Parties acknowledge that it is the intention of the Parties that the development, commercialization and other Exploitation of the Products will be exercised by Buyer and its Affiliates in accordance with its or their own business judgment and, subject to the requirements of this Agreement, in its or their reasonable business judgement.

(f) To the extent permitted by applicable Law, the Parties intend that the payment of the Milestone Consideration be treated as deferred contingent purchase price eligible for installment sale treatment under Section 453 of the Code (subject to imputation of interest under Section 483 or Section 1274 of the Code).

(g) The Parties acknowledge that the achievement of the Milestone Events is uncertain and that Buyer and its Affiliates may not achieve results requiring the payment of any Milestone Consideration at all, and it is therefore not assured that Buyer will be required to pay any Milestone Consideration.

(h) As between the Parties, other than as set forth in Section 5.1 and Section 5.2, Buyer and its Affiliates have no duty to develop, commercialize or otherwise Exploit the Products, to exert any level of efforts in developing, commercializing or Exploiting the Products or to achieve the Milestone Events.

(i) Subject to Buyer's obligations under Section 5.1, Buyer and its Affiliates are not prohibited from developing, manufacturing, marketing, selling, Exploiting or acquiring assets, businesses, or other products that may compete with the Products or any other products of Buyer, Seller or its or their Affiliates.

Section 2.7. Royalties.

(a) On a Product-by-Product and country-by-country basis, during the Royalty Term for such Product, Buyer will pay a royalty to Seller on Annual Net Sales of such Product in the Territory as follows:

(i) [**]% of Annual Net Sales of such Product that are less than or equal to $[**]; and

(ii) [**]% on Annual Net Sales of such Product that are greater than $[**] (each of (Section 2.7(a)) and (Section 2.7(a)(i)), the "Royalties").

(b) Buyer's obligation to pay Royalties for a Product in a country in the Territory will begin upon the First Commercial Sale of such Product in such country and will expire upon the later of (x) the date that is [**] following the First Commercial Sale of such Product in such country, (y) the date on which the sale of such Product is not (or is no longer) Covered by a Valid Claim in such country and (z) expiration of all Regulatory Exclusivity, if any, for such Product in such country (the "Royalty Term"). Following the expiration of the Royalty Term for a Product in a given country, Annual Net Sales of such Product in such country will be excluded from Annual Net Sales for purposes of determining the Royalties due hereunder.

(c) Following the First Commercial Sale, for each calendar quarter until the expiration of the last to expire Royalty Term, Buyer will give Seller a written statement of the Royalties due with respect to a such calendar quarter within [**] after the end of such calendar quarter (each, a "Royalty Report"), which Royalty Report will include the Annual Net Sales of Products subject to Royalties sold by Buyer and its Affiliates and its and their licensees in the Territory during the reporting period and the Royalties payable on Annual Net Sales under this Agreement in accordance with this Section 2.7. Following receipt of such statement, Seller will promptly, but no more than [**] from receipt of the Royalty Report, submit an invoice to Buyer for the full amount of the corresponding Royalty payable under this Section 2.7, and Buyer will pay such amount no later than the date that is [**] after the receipt of such invoice.

Section 2.8. Late Payments. In addition to any other remedies available to Seller, any failure by Buyer to make a payment within [**] after the date when due shall obligate Buyer to pay computed interest, the interest period commencing on the due date and ending on the actual payment date, to Seller at a per annum rate equal to the lower of (i) [**] percent ([**]%) or (ii) the Prime Rate, provided that such rate shall not in any event exceed the highest rate allowed by Law..

Section 2.9. Accounting. Each Party shall calculate all amounts, and perform other accounting procedures required, under this Agreement and applicable to it in accordance with GAAP.

Section 2.10. Records and Audit Rights. Buyer will and will cause its Affiliates and its and their licensees to, keep complete and accurate financial books and records pertaining to the Exploitation of Products hereunder (including Annual Net Sales of Products) to the extent required to calculate and verify all amounts payable hereunder. Buyer will, and will cause its Affiliates and its and their licensees to, retain such books and records for [**] after the end of the period to which such books and records pertain or for such longer period as may be required by applicable Law. Seller, at its own cost, through an independent auditor reasonably acceptable to Buyer (and who has executed an appropriate confidentiality agreement reasonably acceptable to Buyer that requires the auditor to keep any information learned by it confidential except as needed to report its audit conclusions to Seller), may inspect and audit the relevant records of Buyer pertaining to the calculation of any Milestone Consideration and Royalties due to Seller under this Agreement. Buyer will provide such auditors with access to the records during reasonable business hours. Such access need not be given to any such set of records more often than [**] or more than [**] after the date of any report to be audited. Seller will provide Buyer with written notice of its election to inspect and audit the records related to the Milestone Consideration and Royalties due hereunder not less than [**] prior to the proposed date of review of Buyer's records by Seller's auditors. Should the auditor find any underpayment of Milestone Consideration or Royalties by Buyer to Seller, Buyer will (a) promptly pay Seller the amount of such underpayment; (b) if such underpayment equals or exceeds the higher of (i) [**] Dollars ($[**]) or (ii) [**] percent ([**]%) of Milestone Consideration or Royalties paid during the time period audited, reimburse Seller for the cost of the audit; and (c) provide such auditors with an audit right exercisable within [**] after Seller receives the audit report. If the auditor finds an overpayment by Buyer, then Buyer will have the right to deduct the overpayment from any future Milestone Consideration or Royalties due to Seller by Buyer or, if no such future Milestone Consideration or Royalties are payable, then Seller will refund the overpayment to Buyer within [**] after Seller receives the audit report. Buyer may designate competitively sensitive information which such auditor may see and review

but which it may not disclose to Seller; provided, however, that such designation will not restrict the auditor's investigation or conclusions.

Section 2.11. Purchase Price Allocation. In accordance with Section 1060 of the Code and the methodology set forth on Schedule Section 2.11, Buyer will prepare an allocation of the purchase price (the "Purchase Price Allocation") and provide a draft IRS Form 8594 to Seller no later than [**] after the Closing Date. Seller shall review in good faith the Purchase Price Allocation and IRS Form 8594 prepared by Buyer and shall have the opportunity to provide reasonable comments to Buyer within [**] of receipt thereof. The Parties shall cooperate in good faith to resolve any disagreements regarding the Purchase Price Allocation or IRS Form 8594. Buyer and Seller will file all Tax Returns (including IRS Form 8594) in a manner consistent with the Purchase Price Allocation, as finally determined, unless otherwise required by applicable Law or a Taxing Authority. In the event that the Purchase Price Allocation is disputed by any Governmental Authority, the Party receiving notice of the dispute will promptly notify the other Party in writing of such notice and resolution of the dispute.

Section 2.12. Voting Agreement. If, and only if, Buyer elects to pay any Milestone Consideration to Seller in the form of Solid Shares pursuant to Article II, then, prior to the first issuance of any Solid Shares as Milestone Consideration (the "First Stock Consideration Issuance"), Buyer and Seller will enter into a mutually agreeable voting rights agreement, in substantially the form attached hereto as Exhibit C (the "Voting Rights Agreement").

ARTICLE III

REPRESENTATIONS AND WARRANTIES OF SELLER

Except as set forth in the Seller Disclosure Schedules, Seller represents and warrants to Buyer as of the date hereof (or, if applicable, as of such earlier date as may be set forth in any representation and warranty in this Article III) as follows:

Section 3.1. Organization, Standing and Power. Seller is a limited liability company, duly organized, and validly existing, under the Laws of the State of Delaware, and has all requisite power and authority to own, lease or otherwise hold and operate its properties and other assets and to carry on its business as presently conducted. Seller is duly qualified or licensed to do business and is in good standing (in jurisdictions that recognize the concept of good standing) in each jurisdiction in which the nature of its business or the ownership, leasing or operation of its properties makes such qualification or licensing necessary. Seller has made available to Buyer, prior to the execution of this Agreement, complete and accurate copies of Seller's Certificate of Formation, dated September 9, 2020 ("Seller's Certificate of Formation") and its Amended and Restated Limited Liability Company Agreement, dated [**] ("Seller's Operating Agreement"), in each case as amended as of the date hereof. Seller is not in violation of any of the provisions of Seller's Certificate of Formation or Seller's Operating Agreement. Seller's Certificate of Formation and Seller's Operating Agreement have not been amended or modified. Seller does not have, and has never had, any Subsidiaries, and none of its Affiliates holds any properties, interests, assets or rights that are used or held for use in, or necessary for the conduct of the Business or the Purchased Assets, including the Exploitation of the Products.

Section 3.2. Authority; Noncontravention.

(a) Seller has all requisite power and authority to execute and deliver this Agreement and the Related Documents and to consummate the Contemplated Transactions. The execution and delivery of this Agreement and the Related Documents by Seller and the consummation by Seller of the Contemplated Transactions have been duly authorized by all necessary action on the part of Seller and no other proceedings on the part of Seller are necessary to authorize this Agreement, the Related Documents or to consummate the Contemplated Transactions. Each of this Agreement and the Related Documents has been duly executed and delivered by Seller and, assuming the due authorization, execution and delivery by Buyer, constitutes a legal, valid and binding obligation of Seller, Enforceable against Seller in accordance with its terms.

(b) The board of managers of Seller, by unanimous written consent, duly adopted resolutions: (i) approving and declaring advisable this Agreement, the other Related Documents and the Contemplated Transactions, (ii) declaring that it is in the best interests of the members of Seller that Seller enter into this Agreement and the Related Documents and consummate the Contemplated Transactions on the terms and subject to the conditions set forth in this Agreement or such Related Documents, (iii) authorizing Seller to enter into this Agreement and to consummate the Contemplated Transactions, on the terms and subject to the conditions set forth in this Agreement and the Related Documents, (iv) directing that this Agreement and the other Related Documents be submitted to the members of Seller at a meeting or by written consent in lieu of a meeting for a vote for adopting this Agreement and the Related Documents and approving the Contemplated Transactions, and (v) recommending that the members of Seller vote to approve and adopt this Agreement and the Related Documents and approve the Contemplated Transactions, which resolutions have not been subsequently rescinded, modified or withdrawn in any way.

(c) The only votes or consent of holders of any class or series of membership units necessary to approve and adopt this Agreement, the Related Documents and the Contemplated Transactions pursuant to the provisions of Seller's Certificate of Formation, Seller's Operating Agreement and applicable Law are the affirmative votes or consent of the Requisite Preferred Members (as defined in Seller's Operating Agreement) (the "Member Approval"). On the date hereof, Seller has obtained the Member Approval and delivered to Buyer a copy of such Member Approval dated on the date hereof. No other member approval is required on behalf of Seller for the execution, delivery or performance of this Agreement, the other Related Documents and the Contemplated Transactions.

(d) The execution and delivery of this Agreement and the Related Documents by Seller do not, and the consummation of the Contemplated Transactions and compliance by Seller with the provisions of this Agreement and the Related Documents will not, conflict with, or result in any violation or breach of, or default (with or without notice or lapse of time, or both) under, or give rise to a right of, or result in, termination, cancellation or acceleration of any obligation or to the loss of a benefit under, or result in the creation of any Lien (other than a Permitted Lien) in or upon the Purchased Assets under, or give rise to any payment under or any increased, additional, accelerated or guaranteed rights or entitlements under, or require any action by or notice to any Person under, (i) Seller's Certificate of Formation or Seller's Operating

Agreement, (ii) any Contract to which Seller is a party or any of its properties or other assets is subject or (iii) any Law or Order applicable to Seller, the Business or the Purchased Assets.

(e) No consent, approval, Order or authorization of, action by or in respect of, or registration, declaration or filing with, any Governmental Authority is required by or with respect to Seller, the Business or the Purchased Assets for, or in connection with, (i) the execution and delivery of this Agreement by Seller, (ii) the transfer of the Purchased Assets to Buyer or (iii) the consummation of the Contemplated Transactions.

Section 3.3. No Undisclosed Liabilities; Debt. There are no Liabilities of Seller arising out of or related to the Business or the Purchased Assets of any kind whatsoever, whether accrued, fixed, absolute, contingent, known, unknown, determined, determinable or otherwise. Seller does not have any Liability in respect of a guarantee of any debt or other Liability of any other Person or entity related to the Purchased Assets.

Section 3.4. Good Title; Sufficiency of Assets.

(a) Seller has sole and exclusive, good and marketable title to, or, in the case of property held under a lease or other Contract, an Enforceable leasehold interest in, or adequate rights to use, all of the Purchased Assets free and clear of all Liens, other than Permitted Liens. Seller has the power and right to sell, assign, transfer and deliver to Buyer, as applicable, all of Seller's right, title and interest in and to the Purchased Assets. To Seller's Knowledge, there are no adverse claims of ownership to the Owned Intellectual Property and Seller has not received written notice that any Person has asserted a claim of ownership in or to any of the Owned Intellectual Property. At the Closing, Buyer will acquire from Seller sole and exclusive, good and marketable title to, or, in the case of property held under a lease or other Contract, an Enforceable leasehold interest in, or adequate rights to use, all of the Purchased Assets, free and clear of all Liens, other than Permitted Liens. Seller does not own, nor has ever owned any, real property. This Section 3.4(a) is not, and shall not be construed as, a representation or warranty regarding non-infringement or misappropriation by the Seller of the Intellectual Property Rights of any other Persons (which representation and warranty is provided in Section 3.6(d)).

(b) The Purchased Assets constitute all properties, interests, assets and rights of Seller used or held for use in connection with, and that are necessary for the conduct of, the Business as currently conducted by Seller (other than the Excluded Assets). None of the Excluded Assets are used or held for use in connection with the Business. Without limiting the foregoing, the Assigned Agreements are all Contracts used or held for use by Seller or any Affiliate in connection with the Exploitation of any Product. This Section 3.4(b) is not, and shall not be construed as, a representation or warranty regarding non-infringement or misappropriation by Seller of the Intellectual Property Rights of any other Persons (which representation and warranty is provided in Section 3.6(d)).

Section 3.5. Books and Records. Annex 1.1(a) describes, as of the date hereof, a complete and accurate list of all material Books and Records.

Section 3.6. Intellectual Property.

(a) To Seller's Knowledge, Annex 1.1(c) sets forth, as of the date hereof, a complete and accurate list of all Seller Intellectual Property, including, where applicable, the registration number, expiration date, country or territory, registrant and owner of record in respect of each such item of Seller Intellectual Property, other than the Excluded Assets.

(b) Seller owns all rights, title and interests in and to, or has and has had a valid written license to use all Seller Intellectual Property (including the right to make, have made, sell and have sold all Products), and has the right to transfer to Buyer or its Affiliates under the terms of this Agreement, all of Seller's right, title and interest in and to the Seller Intellectual Property free and clear of all Liens, other than Permitted Liens. Other than the Excluded Assets, immediately following the Closing, Buyer will have the same rights, interest, and title in and to each item of Seller Intellectual Property as held by Seller immediately prior to the Closing, in each case, without the payment of any additional amounts or consideration other than fees, royalties or payments which Seller would otherwise have been required to pay had the Contemplated Transactions not occurred, which right, interest and title in and to any Seller Intellectual Property other than Owned Intellectual Property, is on the terms, and subject to the conditions set forth in the applicable Assigned Agreement governing such Seller Intellectual Property. To Seller's Knowledge, Seller Intellectual Property includes all of the Intellectual Property Rights and Technology, respectively, that is owned or Controlled by Seller and used in the Business.

(c) Seller is in compliance with and has not breached, violated or defaulted under, or received written notice that it has breached, violated or defaulted under, any of the terms or conditions of any license, sublicense or other Assigned Agreement to which Seller is a party or is otherwise bound relating to any of Seller Intellectual Property (including any prior or unamended versions of such Assigned Agreement), nor has there been or is there any event or occurrence that would constitute such a breach, violation or default (with or without the lapse of time, giving of notice or both).

(d) To Seller's Knowledge, Seller, in the conduct of the Business, has not infringed, diluted, misappropriated or otherwise violated and is not infringing, diluting, misappropriating or otherwise violating (including with respect to the development, marketing, license, manufacture, distribution, advertising, use, sale, maintenance, support or other Exploitation by Seller of the Products or its Intellectual Property Rights) the Intellectual Property Rights of any other Person. To Seller's Knowledge, no Person or Persons has infringed, diluted, misappropriated or otherwise violated or is or are infringing, diluting, misappropriating or otherwise violating Seller Intellectual Property. Seller has not agreed to indemnify any Person for or against any interference, infringement, misappropriation or other conflict with respect to the Intellectual Property Rights of any other Person in connection with the Products or the conduct of the Business.

(e) No claims have been asserted or threatened by any Person against Seller in writing, and nor to Seller's Knowledge, does there exist any valid basis for such a claim, with respect to: (i) challenging the ownership, licensing or use of any Seller Intellectual Property; (ii) any actual or potential infringement, dilution, misappropriation or unauthorized use of Seller Intellectual Property by Seller; (iii) any actual or potential infringement, dilution, misappropriation or unauthorized use of any Third Party's Intellectual Property Rights with respect to any Seller Intellectual Property or the Products; (iv) the validity or enforceability of any Seller Intellectual

Property; or (v) asserting that the operation of the Business, including the development, marketing, license, manufacture, distribution, advertising, use, sale, maintenance, support or other Exploitation of the Products by Seller prior to the Closing infringes, dilutes, misappropriates or otherwise violated any Intellectual Property Right of any Person.

(f) To Seller's Knowledge, the registered Seller Intellectual Property is subsisting, valid and enforceable, and have been duly registered or filed with or issued by each appropriate Governmental Authority. To Seller's Knowledge, all necessary filing, examination, registration, maintenance and renewal fees currently due in connection with such registered Seller Intellectual Property have been made, and all necessary documents, proofs of working or use, recordations, affidavits, and certificates in connection with such registered Seller Intellectual Property have been timely filed with the relevant authorities in the United States or applicable foreign jurisdictions, as the case may be, for the purposes of prosecuting, registering or maintaining such registered Seller Intellectual Property. To Seller's Knowledge, the registered Seller Intellectual Property has not expired or been declared invalid, in whole or in part, by any Governmental Authority, and is not subject to any interference, opposition, reissue, reexamination, cancellation or other similar proceeding which challenge the validity or ownership of such registered Seller Intellectual Property. There are no pending or published Patents within the Owned Intellectual Property.

(g) To Seller's Knowledge, no current or former employee or consultant of Seller owns any rights, title or interests in or to any Owned Intellectual Property or the Products. All current and former employees and consultants of Seller and its Affiliates who contributed to the discovery, creation or development of any Seller Intellectual Property or Products have executed a valid and enforceable written Contract which assigned to Seller all of his or her rights in or to the relevant Seller Intellectual Property or Products and agreed to confidentiality restrictions restricting such Person's right to use or disclose proprietary information of Seller.

(h) Seller has used commercially reasonable efforts and taken commercially necessary steps to protect its rights in Seller Intellectual Property and to maintain its Trade Secrets constituting the Owned Intellectual Property in confidence, including requiring all employees, consultants or contractors of Seller to execute confidentiality agreements with respect to intellectual property developed for or obtained from Seller. Seller has not made any of its Trade Secrets or other confidential or proprietary information available to any other Person except pursuant to written agreements requiring such Person to maintain the confidentiality of such information.

(i) Other than UPenn, no college, university or other educational or research institution or agency, Governmental Authority or other organization has sponsored research or development conducted by Seller or has any claim of right or license to, or ownership of, or other Lien, other than Permitted Liens, upon any Owned Intellectual Property.

(j) The Products have not been offered for sale or sold to any Person. To Seller's Knowledge, no inventions disclosed or claimed in any Owned Intellectual Property have been offered for sale or sold to any Person.

(k) To Seller's Knowledge, other than the UPenn License, no license or right to any Intellectual Property Right of any Third Party is necessary for to Exploit the FA Asset.

Section 3.7. Compliance with Law; Regulatory Approvals.

(a) To Seller's Knowledge, the Business and the Purchased Assets have been and are conducted, used and developed in all material respects in compliance with all applicable Law. Seller has not received any notice from any Governmental Authority or other Person in writing to the effect that Seller or any of the Purchased Assets is not, or may not be, in compliance with any Law.

(b) Seller is and has at all times been in possession of, and in material compliance with, all Regulatory Approvals required for the conduct of the Business as of the Closing Date. Each such Regulatory Approval is valid and in full force and effect, and Seller has not received any notice of and, to Seller's Knowledge, there is not pending or threatened any suspension, cancellation, modification, termination, revocation, or nonrenewal of any Regulatory Approval. There has occurred no material default (by Seller) under, or violation (by Seller) of, any Regulatory Approval.

Section 3.8. Litigation. There is no Action to which Seller (or members or managers, directors, officers, employees or agents of Seller in its, his, or her capacity as such and to the extent such Actions relate to Seller) is a party (either as plaintiff or defendant) or to which its Purchased Assets are or may be subject that is pending, or, to Seller's Knowledge, threatened, that affects or, if successful, could reasonably be expected to be adverse to Seller, the Business, the Products, the Purchased Assets or the Assumed Liabilities or that, if successful, could reasonably be expected to result in restraining, enjoining or otherwise preventing the completion by Seller of the Contemplated Transactions, nor are there, to Seller's Knowledge, any facts, circumstances or conditions on which any such Action could be brought in the future. There is no Action in respect of Seller that (a) resulted in an Order of any Governmental Authority against or settlement by Seller (whether or not such Order or settlement was paid, in whole or in part, by an insurer of Seller or other Third Party) or (b) resulted in any equitable relief or (c) relates to the Contemplated Transactions. There is no Action which Seller presently intends to initiate.

Section 3.9. Taxes.

(a) Seller has timely and properly filed all income and other material Tax Returns with respect to the Business and the Purchased Assets that are required to be filed and timely and properly paid all Taxes due and payable with respect to the Business and the Purchased Assets (whether or not shown as due and owing on such Tax Returns). All such Tax Returns are true, complete, and accurate in all material respects and prepared in accordance with applicable Law.

(b) There are no liens for Taxes upon any of the Purchased Assets other than liens for Taxes not yet due and payable.

(c) As of the date of this Agreement, Seller is not the subject of an audit, investigation, or other proceeding relating to the payment of or failure to pay any amount of Taxes with respect to the Business or the Purchased Assets, and Seller has not received written notice

from any Taxing Authority that such an audit, investigation, or other proceeding will be initiated in the future. No private letter rulings, technical advice memoranda, or similar agreement or rulings relating to Taxes have been requested, entered into or issued by any Taxing Authority with or in respect of Seller.

(d) Seller has not entered into an agreement or waiver extending any statute of limitations relating to the assessment, payment or collection of a material amount of Taxes that that is currently in effect.

(e) All Taxes required to be deducted or withheld and paid in connection with amounts paid or owing to any current or former employee, independent contractor, partner, member, manager, creditor, equity holder or other Person or by Seller have been so deducted or withheld and timely paid over to the appropriate Taxing Authority, and all associated reporting and recordkeeping requirements have been complied with in all material respects.

(f) No claim has been made to Seller by any Taxing Authority in a jurisdiction where Seller does not file Tax Returns that Seller is subject to taxation by or required to file Tax Returns in that jurisdiction.

(g) Seller has not been a member of an "affiliated group" within the meaning of Section 1504(a) of the Code filing a consolidated federal income Tax Return or any other group filing or required to file Tax Returns on a combined, consolidated or unitary basis. Seller is not party to any Contract relating to Tax sharing or Tax allocation. Seller has no Liability for the Taxes of any Person under Treasury Regulation 1.1502-6 (or any similar provision of state, local or foreign Law), as a transferee or successor, by Contract or otherwise.

(h) Seller has not received any prepaid amount that has been deferred pursuant to Section 451(c) of the Code, Revenue Procedure 2004-34, 2004-22 I.R.B. 991 or otherwise.

(i) Seller has not been a party to a transaction that is or is substantially similar to a "listed transaction" as defined in Treasury Regulation Section 1.6011-4(b).

(j) No closing agreements, private letter rulings, technical advice memoranda or similar agreements or rulings related to Taxes have been entered into, issued by or requested from any Taxing Authority with or in respect of the Business or the Purchased Assets.

(k) Neither the execution and delivery of this Agreement by Seller nor the consummation of the Contemplated Transactions (alone or together with any other event) will give rise to any payments or benefits that would be nondeductible by the payor under Section 280G of the Code.

Section 3.10. FDA Regulatory Matters.

(a) To Seller's Knowledge, Seller has conducted all Exploitation of Products in material compliance with applicable Laws, including, as applicable, the FDCA, the PHSA, good laboratory practices, good clinical practices, and good manufacturing practices, and all similar applicable Laws of any jurisdiction (collectively, "FDA Laws").

(b) To Seller's Knowledge, all ongoing and completed preclinical studies of any Product and Clinical Trials conducted by Seller have been conducted in all material respects in accordance with all FDA Laws and preclinical study and Clinical Trial protocols. No Clinical Trial conducted by or on behalf of Seller has been placed on full or partial clinical hold or terminated or suspended prior to completion. Seller has not received any notice that any Governmental Authority, investigator, or any review board has, in each case with respect to any Product: (a) refused to approve any preclinical study or Clinical Trial or any substantial amendment to a protocol for any preclinical study or Clinical Trial conducted or proposed to be conducted by Seller; (b) initiated, or threatened to initiate, any action to suspend any preclinical study or Clinical Trial conducted by or on behalf of Seller, or suspend or terminate any application for any Regulatory Approval, or otherwise restrict or delay the preclinical study; or (c) alleged that any preclinical study or Clinical Trial conducted Seller is in violation of FDA Laws.

(c) Seller has not received any notice or other correspondence from any Governmental Authority of any pending or threatened claim, suit, proceeding, hearing, enforcement, audit, investigation, or arbitration alleging potential or actual noncompliance by or liability of Seller under any FDA Laws or threatening to withdraw or suspend any Regulatory Approval or IND Clearance. Seller has not received any notices or other correspondence from any Governmental Authority or review board with respect to the Exploitation of any Product which could prevent Seller from conducting such activities substantially in the manner presently performed or contemplated by Seller and, to Seller's Knowledge, no Governmental Authority, review board or similar body has threatened any such action.

(d) Seller has filed, maintained, and furnished all material reports, documents, registrations, applications, filings, authorizations, claims, and notices required to be filed, maintained, or furnished to FDA or any other Regulatory Authority.

(e) Seller has not received from any Governmental Authority any warning letter, untitled letter, FDA Form 483, prohibition notice, recall notice or equivalent in any jurisdiction with respect to any Product, or any written notice of any pending or threatened civil, criminal, administrative or regulatory claim, suit, proceeding, hearing, enforcement, audit, investigation, arbitration, inquiry, search warrant, subpoena (other than those related to actions against third parties), and there is not pending any allegation that any operation or activity performed by of Seller is in violation of any FDA Laws.

(f) To Seller's Knowledge, Seller has not made any untrue statement of material fact or fraudulent statement to the FDA or any other Regulatory Authority with respect to any Product, failed to disclose a material fact required to be disclosed to the FDA or any other Regulatory Authority with respect to any Product, or committed an act, made a statement, or failed to make a statement with respect to any Product that could reasonably be expected to provide a basis for the FDA to invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities," set forth in 56 Fed. Reg. 46191 (September 10, 1991) and any amendments thereto or any analogous applicable Laws.

(g) Seller is not debarred, has not been convicted, and is not subject to debarment or conviction pursuant to Section 306 of the FDCA or any similar Law. Seller has not used in connection with the Exploitation of any Product, any employee, agent, or independent

contractor who has been debarred, or, to Seller's Knowledge, has been convicted or is the subject of debarment proceedings pursuant to Section 306 of the FDCA or any similar Laws.

(h) Notwithstanding the foregoing, Seller expressly disclaims, and makes no representation with respect to, any actions taken by or on behalf of UPenn.

Section 3.11. Product Inventory.

(a) Annex 1.1(b) sets forth, as of the date hereof, a list of all Product Inventory.

(b) To Seller's Knowledge, the Product Inventory was manufactured in accordance with the applicable specifications and all applicable Laws, including current good manufacturing practices.

Section 3.12. Brokers and Other Advisors. No broker, investment banker, financial advisor or other Person is entitled to any broker's, finder's, financial advisor's or other similar fee or commission in connection with the Contemplated Transactions based upon arrangements made by or on behalf of Seller.

Section 3.13. Solvency. Seller (a) is able to pay its debts as they become due, (b) is solvent and will be solvent immediately following the Closing, (c) immediately following the Closing, will possess sufficient assets to discharge its Liabilities, and (d) will, prior to any distribution to members, discharge such Liabilities. Seller is not engaged in business or a transaction, and it is not about to engage in business or a transaction, for which its remaining assets and capital are or will be insufficient to discharge such Liabilities. Seller does not intend to incur, or believe that it will incur, Liabilities that would be beyond its ability to pay as such Liabilities matured. Seller has not entered into this Agreement for the purpose of hindering, delaying or defrauding its creditors.

Section 3.14. Contracts.

(a) As to Seller, and, to Seller's Knowledge, as to any third party, as of the date of this Agreement, each Assigned Agreement is Enforceable in accordance with its terms against Seller and will be Enforceable on identical terms as of immediately following the consummation of the Contemplated Transactions.

(b) A true, correct and complete copy of each Assigned Agreement has been made available to Buyer and its Representatives. There is no violation, breach, default or delay under any Assigned Agreement by Seller or, to Seller's Knowledge, by any other party thereto, and no event has occurred or condition exists that with the lapse of time or the giving of notice or both would constitute a default thereunder by Seller or, to Seller's Knowledge, any other party thereto, and Seller has not received or given notice of any default or claimed or purported or alleged default or state of facts which, with notice or lapse of time or both, would constitute a default on the part of any party in the performance or payment of any Assigned Agreement. No notice, waiver, consent or approval is required (or the lack of which would give rise to a right of termination, cancellation or acceleration of, or entitle any party to accelerate, whether after the giving of notice or lapse of time or both, any obligation under any Assigned Agreement) under or relating to any Assigned Agreement in connection with the execution, delivery and performance

of this Agreement or the consummation of the Contemplated Transactions. Each Assigned Agreement was entered, and performance under each Assigned Agreement has been, in compliance with all applicable Law, and there is no Action with respect to the entry or performance under any Assigned Agreement, nor is there, to Seller's Knowledge, any fact, circumstance or condition on the basis of which any such Action could be brought in the future.

Section 3.15. Affiliate Transactions. No manager, member, officer, director, employee, equity holder or other Affiliate of Seller, or any individual related by blood, marriage or adoption to any such Person or entity, or to Seller's Knowledge, any entity in which any such Person or entity owns any beneficial interest, is a party to any Contract or transaction relating to the Purchased Assets.

Section 3.16. No Other Representations or Warranties. THE REPRESENTATIONS AND WARRANTIES CONTAINED IN THIS ARTICLE III (AS MODIFIED BY THE SELLER DISCLOSURE LETTER OR ANY ANNEX), IN ANY RELATED DOCUMENT TO WHICH SELLER IS A PARTY OR IN ANY CERTIFICATE DELIVERED HERETO OR THERETO, CONSTITUTE THE EXCLUSIVE REPRESENTATIONS AND WARRANTIES OF SELLER IN CONNECTION WITH, ARISING OUT OF, OR WITH RESPECT TO, THE CONTEMPLATED TRANSACTIONS.

Section 3.17. Non-Reliance. SELLER HEREBY ACKNOWLEDGES AND AGREES THAT, EXCEPT FOR THE REPRESENTATIONS AND WARRANTIES CONTAINED IN ARTICLE IV OF THIS AGREEMENT, THE OTHER RELATED DOCUMENTS TO WHICH BUYER IS A PARTY AND EACH BUYER REPRESENTATION LETTER (IN EACH CASE, OR IN ANY CERTIFICATE DELIVERED THERETO), NEITHER BUYER NOR ANY OTHER PERSON ON BEHALF OF BUYER MAKES ANY EXPRESS OR IMPLIED REPRESENTATION OR WARRANTY WITH RESPECT TO BUYER, AND (SUBJECT TO THE EXPRESS REPRESENTATIONS AND WARRANTIES OF BUYER SET FORTH IN ARTICLE IV OF THIS AGREEMENT, THE OTHER RELATED DOCUMENTS TO WHICH BUYER IS A PARTY AND EACH BUYER REPRESENTATION LETTER (IN EACH CASE, OR IN ANY CERTIFICATE DELIVERED THERETO), IN EACH CASE, AS QUALIFIED AND LIMITED BY THE BUYER DISCLOSURE SCHEDULES, SELLER HAS NOT RELIED ON ANY SUCH INFORMATION (INCLUDING THE ACCURACY OR COMPLETENESS THEREOF). NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS AGREEMENT, INCLUDING IN THIS SECTION 3.17, SHALL LIMIT THE RIGHTS OF SELLER TO BRING A CLAIM FOR FRAUD.

ARTICLE IV

REPRESENTATIONS AND WARRANTIES OF BUYER

Buyer represents and warrants to Seller as follows:

Section 4.1. Organization, Standing and Power. Buyer is a corporation duly organized and validly existing under the Laws of the State of Delaware and has all requisite corporate power and authority to carry on its business as now being conducted, except where the failure to have such power or authority or possess such governmental licenses, permits, authorizations or

approvals, individually or in the aggregate, has not been and would not reasonably be expected to be material to Buyer, taken as a whole. Buyer is duly qualified or licensed to do business and is in good standing (in jurisdictions that recognize the concept of good standing) in each jurisdiction in which the nature of its business or the ownership, leasing or operation of its properties makes such qualification or licensing necessary, other than in such jurisdictions where the failure to be so qualified or licensed or to be in good standing individually or in the aggregate has not been and would not reasonably be expected to be material to Buyer.

Section 4.2. Authority; Noncontravention.

(a) Buyer has all requisite corporate power and authority to execute and deliver this Agreement and the Related Documents and to consummate the Contemplated Transactions. The execution and delivery of this Agreement and the Related Documents by Buyer and the consummation by Buyer of the Contemplated Transactions have been duly authorized by all necessary corporate action on the part of Buyer and no other corporate proceedings on the part of Buyer are necessary to authorize this Agreement, the Related Documents or to consummate the Contemplated Transactions. Each of this Agreement and the Related Documents has been duly executed and delivered by Buyer and, assuming the due authorization, execution and delivery by Seller, constitutes an Enforceable obligation of Buyer in accordance with its terms.

(b) The execution and delivery of this Agreement and the Related Documents by Buyer do not, and the consummation of the Contemplated Transactions and compliance by Buyer with the provisions of this Agreement and the Related Documents will not, conflict with, or result in any violation or breach of, or default (with or without notice or lapse of time, or both) under, or give rise to a right of, or result in, termination, cancellation or acceleration of any obligation or to the loss of a benefit under, or result in the creation of any Lien in or upon any of the properties or other assets of Buyer under (i) the certificate of incorporation or bylaws of Buyer, (ii) any Contract to which Buyer is a party or any of its respective properties or other assets is subject or (iii) any Law or Order, except in the cases of clauses (ii) and (iii), where the conflict, violation, breach, default, termination, cancellation, acceleration or creation of a Lien, individually or in the aggregate, would prevent, materially impede or materially delay the consummation by Buyer of the Contemplated Transactions (including the payments required to be made pursuant to Article II).

(c) No consent, approval, Order or authorization of, action by or in respect of, or registration, declaration or filing with, any Governmental Authority is required by or with respect to Buyer in connection with the execution and delivery of this Agreement by Buyer or the consummation by Buyer of the Contemplated Transactions.

Section 4.3. Buyer Capitalization; Valid Issuance.

(a) The authorized capital stock of Buyer consists of 120,000,000 shares of Solid Shares and 10,000,000 shares of Solid Preferred Stock. Buyer's disclosure of its issued and outstanding capital stock in its most recent SEC Filing containing such disclosure was accurate in all material respects as of the date indicated in such SEC Filing.

(b) All of the outstanding shares of Solid Capital Stock have been duly authorized and validly issued and are fully paid, non-assessable; none of such shares were issued in violation of any preemptive rights or similar rights; and such shares were issued in compliance in all material respects with applicable state and federal securities laws and any rights of third parties. Except for (a) stock options and restricted stock units approved pursuant to Buyer stock- based compensation plans described in the SEC Filings and (b) warrants disclosed in the SEC Filings, there are no outstanding warrants, options, convertible securities or other rights, agreements or arrangements of any character under which Buyer is or may be obligated to issue any equity securities of any kind, except as contemplated by this Agreement.

Section 4.4. Financing. Buyer has, and at the Closing shall have, sufficient funds, funding options and/or existing liquidity facilities to permit Buyer to consummate the Contemplated Transactions, and with respect to the payment of Royalties and Milestone Consideration, as of the date hereof, Buyer has sufficient funds, funding options and existing liquidity facilities to permit Buyer to pay each Royalty and Milestone Consideration if, as and when payable.

Section 4.5. Litigation. As of the date of this Agreement, there is no Action pending (or, to the knowledge of Buyer, threatened in writing) against Buyer that would, or would be reasonably likely to materially delay, restrain, prevent, enjoin or otherwise prohibit the execution, delivery and performance by Buyer of this Agreement and the Contemplated Transactions.

Section 4.6. No Buyer Vote Required. No vote or action of the stockholders of Buyer is required by applicable Law, the Buyer Charter or otherwise, in order for Buyer to consummate the Contemplated Transactions, including the payment or issuance of the Milestone Consideration or payment of Royalties, if and when payable or issuable in accordance with and subject to Section 2.6 and Section 2.7, respectively.

Section 4.7. Issuance of Solid Shares. The issuance and delivery of Solid Shares in accordance with this Agreement has been, or will be prior to such issuance, duly authorized by all necessary corporate action on the part of Buyer and, when issued as contemplated hereby, such Solid Shares shall be duly and validly issued, fully paid and nonassessable. Such Solid Shares when so issued and delivered in accordance with the provisions of this Agreement, shall be free and clear of all Liens, other than restrictions on transfer created by applicable securities Laws or the terms of this Agreement and will not have been issued in violation of applicable Laws, applicable Nasdaq rules or regulations, or any preemptive rights or rights of first refusal or similar rights.

Section 4.8. Financial Statements. The financial statements included in each SEC Filing filed prior to the date hereof comply in all material respects with applicable accounting requirements and the rules and regulations of the SEC with respect thereto as in effect at the time of filing (or to the extent corrected by a subsequent restatement) and present fairly, in all material respects, the consolidated financial position of Buyer as of the dates shown and its consolidated results of operations and cash flows for the periods shown, subject in the case of unaudited financial statements to normal, immaterial year-end audit adjustments, and such consolidated financial statements have been prepared in conformity with GAAP (except as may be disclosed therein or in the notes thereto, and except that the unaudited financial statements may not contain

all footnotes required by GAAP, and, in the case of quarterly financial statements, except as permitted by Form 10-Q under the Exchange Act). Except as set forth in the financial statements of Buyer included in the SEC Filings filed prior to the date hereof, Buyer has not incurred any liabilities, contingent or otherwise, except those incurred in the ordinary course of business, consistent (as to amount and nature) with past practices since the date of such financial statements.

Section 4.9. Compliance with Nasdaq Continued Listing Requirements. Buyer is in compliance with all applicable Nasdaq continued listing requirements as of the date hereof.

Section 4.10. SEC Filings; Securities Law and Other Matters.

(a) Buyer has filed or furnished, as applicable, on a timely basis, all reports, schedules, forms, statements and other documents required to be filed by Buyer under the Securities Act and the Exchange Act, including pursuant to Section 13(a) or 15(d) thereof, for the three-year period preceding the date hereof (collectively, the "SEC Filings"). At the time of filing thereof, the SEC Filings complied as to form in all material respects with the requirements of the Securities Act or the Exchange Act, as applicable, and the rules and regulations of the SEC thereunder, and none of the SEC Filings, when filed or furnished, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(b) Buyer is eligible to register securities on Form S-3 under the Securities Act.

(c) The Solid Shares are registered pursuant to Section 12(b) of the Exchange Act and are listed on Nasdaq and Buyer has taken no action designed to, and will not take any action to, terminate the registration of the Solid Shares under the Exchange Act or delisting the Solid Shares from Nasdaq in a manner intended to deprive Seller of any consideration payable to Seller in respect of such shares, nor has Buyer received any notification that the SEC or Nasdaq is contemplating terminating such registration or listing.

Section 4.11. No Other Representations or Warranties. THE REPRESENTATIONS AND WARRANTIES CONTAINED IN THIS ARTICLE IV (AS MODIFIED BY THE BUYER DISCLOSURE LETTER OR ANY ANNEX), IN ANY RELATED DOCUMENT TO WHICH BUYER IS A PARTY OR IN ANY CERTIFICATE DELIVERED HERETO OR THERETO, CONSTITUTE THE EXCLUSIVE REPRESENTATIONS AND WARRANTIES OF BUYER IN CONNECTION WITH, ARISING OUT OF, OR WITH RESPECT TO, THE CONTEMPLATED TRANSACTIONS.

Section 4.12. Non-Reliance. BUYER HEREBY ACKNOWLEDGES AND AGREES THAT, EXCEPT FOR THE REPRESENTATIONS AND WARRANTIES CONTAINED IN ARTICLE III OF THIS AGREEMENT, THE OTHER RELATED DOCUMENTS TO WHICH SELLER IS A PARTY AND EACH SELLER REPRESENTATION LETTER (IN EACH CASE, OR IN ANY CERTIFICATE DELIVERED THERETO), NEITHER SELLER NOR ANY OTHER PERSON ON BEHALF OF SELLER MAKES ANY EXPRESS OR IMPLIED REPRESENTATION OR WARRANTY WITH RESPECT TO SELLER, AND (SUBJECT TO THE EXPRESS REPRESENTATIONS AND WARRANTIES OF SELLER SET FORTH IN

ARTICLE III OF THIS AGREEMENT, THE OTHER RELATED DOCUMENTS TO WHICH SELLER IS A PARTY AND EACH SELLER REPRESENTATION LETTER (IN EACH CASE, OR IN ANY CERTIFICATE DELIVERED THERETO), IN EACH CASE, AS QUALIFIED AND LIMITED BY THE SELLER DISCLOSURE SCHEDULES, BUYER HAS NOT RELIED ON ANY SUCH INFORMATION (INCLUDING THE ACCURACY OR COMPLETENESS THEREOF). NOTWITHSTANDING THE FOREGOING, NOTHING IN THIS AGREEMENT, INCLUDING IN THIS SECTION 4.12, SHALL LIMIT THE RIGHTS OF BUYER TO BRING A CLAIM FOR FRAUD.

ARTICLE V

ADDITIONAL AGREEMENTS

Section 5.1. Diligence Efforts. Buyer, itself or through its Affiliates or licensees, will use Commercially Reasonable Efforts to develop and seek Regulatory Approval for at least one Product in the Field in each of the US Territory and the European Territory and, to the extent that Regulatory Approval is received in the US Territory or the European Territory, to commercialize such Product in the Field in such territory. Buyer shall not knowingly take any action (or fail to take any action) for the primary purpose of avoiding (a) the achievement of any of the Milestone Event or (b) payment of any Milestone Consideration.

Section 5.2. Anti-Shelving. In the event Buyer and its Affiliates cease all research, development, and commercialization activities and other Exploitation with respect to any Product for a period of at least [**], and such cessation is not due to a fact, circumstance or requirement outside of Buyer's reasonable control, including a requirement of a Regulatory Authority, such cessation shall be deemed a material breach of Section 5.1, and Seller shall have the right to exercise the Reversion Rights provided in Section 5.11.

Section 5.3. Joint Steering Committee.

(a) Establishment and Responsibilities. The Parties will establish a joint steering committee ("JSC") within [**] following the Closing Date. The JSC will (i) serve as a forum for the exchange of information regarding the development of the FA Asset and Products, and (ii) oversee the conduct of the Technology Transfer.

(b) Membership. The JSC will consist of an equal number of representatives for each Party, with at least [**] representatives appointed by each Party. A Party may change any of its representatives on the JSC at any time with a new person (with appropriate expertise to replace the outgoing member) by giving written notice to the other Party. One member of the JSC will serve as secretary of the JSC at each JSC meeting, and the secretary will alternate from meeting to meeting between a Seller member and a Buyer JSC member.

(c) Meetings. Once established, the JSC will meet as frequently as Seller and Buyer mutually agree upon as appropriate, but no less than [**] per calendar year. Meetings of the JSC will be effective only if at least one (1) representative of each Party is present or participating. The JSC may meet either (i) in person at either Party's facilities (alternating between the facilities of Seller and Buyer) or at such locations as the Parties may otherwise agree or (ii) by

audio or video teleconference. Other representatives of each Party may attend meetings as non- voting participants, subject to the confidentiality provisions set forth in Section 5.5.

(d) Authority; Decision-Making. The JSC will serve as a forum for exchange of information relating to the development of the FA Asset and Products, and will have no decision-making authority. The JSC will not have any power to (i) modify or amend the terms and conditions of this Agreement, (ii) waive either Party's compliance with, or determine that either Party has or has not fulfilled, the terms and conditions of this Agreement, or (iii) determine any issue in a manner that would conflict with, expand, or reduce the express terms and conditions of this Agreement. Buyer shall consider any comments and recommendations from Seller's members of the JSC in good faith.

(e) Exchange of Information. Buyer will keep Seller reasonably informed as to its progress and activities in material aspects relating to the development of the Product in the Territory, by way of updates to JSC at their meetings. In connection therewith, Buyer will provide to Seller high-level progress reports in the form and containing the same substance as provided to UPenn under the UPenn License (with the same frequency as provided thereto), within [**] following the provision of such reports to UPenn.

(f) Discontinuation. The JSC will disband and will have no further authority hereunder upon the completion of the [**].

(g) Expenses. Each Party will be responsible for all of its own expenses incurred in connection with participating in the JSC meetings.

Section 5.4. Technology Transfer and Assistance.

(a) Technology Transfer. Seller will transfer or make available to (or will use commercially reasonable efforts to cause its vendors and contractors to transfer or make available to, as applicable), Buyer or its designee (i) all Books and Records described on Annex 1.1(a), (ii) all Product Inventory set forth on Annex 1.1(b), (iii) all tangible embodiments of the Seller Intellectual Property set forth on Annex 1.1(c) and (iv) the FA Asset sequence set forth on Annex 1.1(d), in each case, to the extent within Seller's possession and control, promptly following the Closing Date, and in no event more than [**] after the receipt of the consideration set forth in Section 2.5(d) (the "Technology Transfer"). Seller will effect the Technology Transfer to Buyer (or its applicable designee(s)), through the method of delivery reasonably agreed upon by Seller and Buyer in advance of the Closing, which shall be through (x) electronic delivery or (y) access to Seller's "data room" or other cloud-based storage software (in the case of (x) and (y), all electronically stored files within Seller's possession and control must be able to be downloaded by Buyer, and any documents that may require edits or modifications upon transfer to Buyer shall be provided in Microsoft Word format).

(b) Assistance. From the Closing Date until the later of (i) the date of IND Clearance for the first Product by the FDA or (ii) the date that is [**] following the Closing Date, Seller will make available to Buyer or its designees such personnel of Seller as may reasonably be requested by Buyer to assist Buyer in connection with understanding the content, or implementing or using the content, of the Technology Transfer. In addition, at the request of Buyer, Seller will

use reasonable efforts to facilitate the (i) cooperation of any Seller vendor or contractor in connection with providing such assistance and (ii) UPenn's [**], in the case of clause (ii), to the extent not completed prior to the Closing Date.

Section 5.5. Confidentiality; Non-Solicitation.

(a) Confidentiality.

(i) Each of Buyer and Seller acknowledges that the information provided to them in connection with this Agreement and the consummation of the Contemplated Transactions is subject to the terms of the Confidentiality Agreement. Effective upon, and only upon, the Closing, the Confidentiality Agreement will terminate with respect to information included in or related to the Purchased Assets, and such information will be deemed to have been provided under this Agreement and subject to this Section 5.5.

(ii) For purposes of this Agreement, "Confidential Information" will include any and all confidential or proprietary information, whether or not patentable and in any form (written, oral, photographic, electronic, magnetic, or otherwise), including information of Third Parties, that a Party (or an Affiliate or representative of such Party) discloses or otherwise makes available to the other Party (or to an Affiliate or representative of such Party) in connection with this Agreement. Notwithstanding the foregoing, any information, knowledge and data contained or included in the Purchased Assets or related to the Business, including Seller Intellectual Property, will be the Confidential Information of Buyer, regardless of which Party is the disclosing Party with respect to such information, knowledge or data. The terms and conditions of this Agreement will be the Confidential Information of both Parties.

(iii) Each Party agrees that, for a period of [**] following receipt of Confidential Information, it will keep confidential and will not publish or otherwise disclose and will not use for any purpose other than as provided for in this Agreement or any Reversion Agreement (including for the exercise of the rights granted to such Party hereunder) any Confidential Information of the other Party, except to the extent expressly agreed in writing by the other Party. The foregoing confidentiality and non-use obligations will not apply with respect to any information that the receiving Party can demonstrate by competent written proof:

(A) was in the lawful knowledge and possession of the receiving Party prior to the time it was disclosed by the disclosing Party to the receiving Party, or was otherwise developed independently by or for the receiving Party without use of or reference to the disclosing Party's Confidential Information, as evidenced by written records kept in the ordinary course of business, or other documentary proof of actual use by the receiving Party;

(B) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the receiving Party;

(C) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the receiving Party in breach of this Agreement; or

(D) was disclosed to the receiving Party, other than under an obligation of confidentiality, by a Third Party who, to the knowledge of the receiving Party, had no obligation to the disclosing Party not to disclose such information to others.

(iv) If a Party is required by applicable Law or judicial or administrative process to disclose Confidential Information that is subject to the non-disclosure provisions of this Section 5.5(a), such Party may disclose such Confidential Information as so required; provided, that such Party will to the extent reasonably practicable inform the other Party of the disclosure that is being sought in order to provide such other Party an opportunity to challenge or limit the disclosure obligations, and, if requested by the other Party, cooperate in all reasonable respects with the other Party's efforts to obtain confidential treatment or a protective order with respect to any such disclosure. Confidential Information that is disclosed as required by applicable Law or by judicial or administrative process will remain otherwise subject to the confidentiality and non-use provisions of this Section 5.5(a), and the Party so disclosing Confidential Information will (A) take all steps reasonably necessary to endeavor to ensure the continued confidential treatment of such Confidential Information and (B) disclose such Confidential Information only to the extent required by such applicable Law or judicial or administrative process.

(v) Notwithstanding anything to the contrary in this Section 5.5(a), the receiving Party may disclose the disclosing Party's Confidential Information if and to the extent such disclosure is reasonably necessary in the following instances: (A) to its actual or potential acquirors, investors, lenders or other similar sources of financing solely for the purpose of evaluating or carrying out an actual or potential investment, loan, financing or acquisition; (B) to its Affiliates, employees, consultants, agents, and, in the case of Buyer, actual or potential licensees or contractors to exercise its rights or perform its obligations in accordance with the terms of this Agreement; and (C) to its external attorneys, independent accountants or financial advisors solely for the purpose of enabling such attorneys, independent accountants or financial advisors to provide advice to it with respect to this Agreement or the activities contemplated hereunder; provided, that in each case ((A) through (C)) such Person is subject to a written agreement containing obligations of confidentiality and non-use at least as stringent as those herein (or without such agreement for recipients that are financial or legal advisors under a professional code of conduct giving rise to an expectation of confidentiality and non-use at least as restrictive as those set forth in this Agreement), and where the applicable disclosure is of the terms of this Agreement, each Party will redact confidential or commercially sensitive information or other information that is not relevant to the purpose of the applicable disclosure.

(b) Non-Solicitation. Seller agrees that for a period of [**] commencing upon the Closing Date, Seller will not, directly or indirectly, recruit or otherwise solicit any employee of Buyer or any of its Affiliates (each, a "Business Partner") to terminate or diminish its employment relationship with Buyer or any of its Affiliates after the Closing; provided, (i) nothing

herein shall prohibit Seller from hiring, retaining, employing or otherwise engaging, or attempting to hire, retain, employ or engage, any Business Partner who was terminated by Buyer or its Affiliates at least [**] prior to Seller's initial contact with such Business Partner for the purpose of attempting to hire, retain, employ or otherwise engage such Business Partner, and (ii) placing a general solicitation, advertisement or posting not targeting the employees, independent contractors or consultants of Buyer or its Affiliates.

(c) Injunctive Relief. Each Party understands that a breach of this Section 5.4 by the other Party may cause irreparable harm which may not be adequately compensated by money damages. Accordingly, in the event of a breach or threatened breach by either Party of this Section 5.4, the non-breaching Party or, as applicable, any of its Affiliates will be entitled to seek injunctive or other equitable relief to enforce the provisions hereof (without need to post bond), in addition to and not in the alternative to such other remedies to which such Party or its Affiliates may be entitled, including the recovery of money damages.

Section 5.6. Certain Tax Matters.

(a) Transfer Taxes. All recordation, transfer, documentary, sales, value added, use, stamp, conveyance or other similar Taxes, duties or governmental charges, and all recording or filing fees or similar costs, imposed or levied by reason of, in connection with or attributable to this Agreement or the Acquisition (collectively, "Transfer Taxes") will be borne equally between Seller and Buyer. Transfer Taxes will be timely paid, and all applicable filings, reports and returns will be filed, as provided by applicable Law. The paying Party (if not specified as the responsible Party therefor) will be entitled to reimbursement from the non-paying Party in accordance with this Section 5.6(a).

(b) Proration of Taxes. All real and personal property Taxes, and other *ad valorem* Taxes with respect to the Purchased Assets for a taxable period that includes (but does not end on) the Closing Date will be prorated on a *per diem* basis between Seller and Buyer on the Closing Date.

(c) Tax Withholding. Buyer and any other applicable withholding agent will be entitled to deduct and withhold, or cause to be deducted and withheld, from any amounts payable pursuant to or as contemplated by this Agreement or any Related Document any Taxes or other amounts required under the Code or any applicable Law to be deducted and withheld. To the extent that any such amounts are so deducted or withheld and properly remitted to the appropriate Taxing Authority, such amounts will be treated for all purposes of this Agreement and any Related Document as having been paid to the Person in respect of which such deduction and withholding was made. Notwithstanding anything to the contrary in this Agreement, any compensatory amounts payable pursuant to or as contemplated by this Agreement will be remitted by the applicable payer to the applicable employer for payment through such employer's payroll procedure in accordance with applicable Law.

(d) Cooperation and Exchange of Information. To the extent relevant to the Business or the Purchased Assets, each of Seller and Buyer will (i) provide the other with such assistance as may reasonably be requested by the other Party in connection with the preparation of any Tax Return, audit or other examination by any Taxing Authority or Action relating to liability

for Taxes in connection with the Business or the Purchased Assets, (ii) retain (for a period of [**] after the Closing) and provide the other with any records or other information that may be relevant to such Tax Return, audit or examination, Action or determination and (iii) provide the other with any final determination of any such audit or examination, Action or determination that affects any amount required to be shown on any Tax Return of the other for any period.

Section 5.7. Public Announcements. Seller and its Affiliates will not issue any press release or otherwise make any public statement with respect to the provisions of this Agreement or the Contemplated Transactions without the prior written consent of Buyer. Buyer or its Affiliates may issue a press release or otherwise make any public statement with respect to the provisions of this Agreement or the Contemplated Transactions without the prior written consent of Seller, as long as Buyer provides Seller with (i) a copy of such press release or public statement prior to the issuance thereof and (ii) a reasonable opportunity to comment thereon, which comments Buyer will consider in good faith. Notwithstanding anything to the contrary in this Agreement or any Related Document, either Party may issue a press release or make a public statement with respect to the Contemplated Transactions as may be required by Law or any listing agreement with any applicable securities exchange or market, in which case such Party shall use reasonable best efforts to consult with the other Party regarding the contents thereof prior to issuing any such press release or making any such public statement or public announcement and shall consider in good faith any comments proposed by such other Party. The foregoing will not limit any non-public equity holder communication of either Party or any disclosure made in connection with a court pleading.

Section 5.8. Expenses; UPenn IND Payment.

(a) Except as expressly set forth herein, each of Seller and Buyer will bear its own costs and expenses incurred in connection with this Agreement and the Contemplated Transactions.

(b) Following a UPenn IND Payment becoming due pursuant to the Second UPenn Amendment, Buyer will provide an invoice to Seller and Seller will promptly, but in any event within [**] of receipt of such invoice or notice, pay such UPenn IND Payment directly to UPenn by wire transfer of immediately available funds.

Section 5.9. Further Assurances and Approvals.

(a) Seller will at any time and from time to time after the Closing Date and for no further consideration, upon the request of Buyer, execute, acknowledge, deliver and file, or cause to be done, executed, acknowledged, delivered or filed, all such further acts, deeds, transfers, conveyances, assignments or assurances as may be reasonably required for the better transferring, conveying, assigning, assuring and vesting to and in Buyer, or for the aiding and assisting in the reducing to possession by Buyer of, Seller's right, title and interest in and to any of the Purchased Assets or Assumed Liabilities, or as may otherwise be necessary to carry out the purposes of this Agreement and the Related Documents and the consummation of the Contemplated Transactions (including transferring (or upon Buyer's request, providing Buyer or its Affiliates with access to the premises or systems of Seller to so transfer), at no additional cost to Buyer, Seller's right, title

and interest in and to any Purchased Asset or Assumed Liability contemplated by this Agreement to be transferred to Buyer at the Closing and that was not so transferred at the Closing).

(b) If, after the Closing, Buyer or any of its Affiliates possesses any Excluded Asset, Buyer will, or will cause its Affiliates to, transfer such asset to Seller at no cost to Seller. If, after the Closing, Seller or any of its Affiliates possesses any Purchased Asset, Seller will, or will cause its Affiliates to, transfer such asset to Buyer at no cost to Buyer.

(c) From and after the Closing, Seller will refer all inquiries and other communications (whether written or oral) in respect of the Business to Buyer, and will promptly inform Buyer of such inquiries and communications. At Buyer's request, Seller will reasonably cooperate with Buyer to facilitate Buyer's communication with such Person's inquiry or other communication in respect of the Business.

Section 5.10. Lock-Up.

(a) During each period commencing on the date on which Buyer has received all of the following from Seller: (i) a Milestone Invoice, (ii) all necessary documentation from Seller reasonably required by Buyer to issue the Solid Shares (including, without limitation, "live validation" and all account and other similar information required by Buyer to transfer the Solid Shares to Seller) and (iii) in the case of the First Stock Consideration Issuance, Seller's delivery of a signature page to the Voting Rights Agreement; and ending on the earlier of (A) the date that is [**] following such date or (B) the date upon which Buyer files for, or becomes subject to a proceeding for, bankruptcy, reorganization, liquidation, dissolution or similar process (each, a "Lock-Up Period"), Seller will not directly or indirectly, in any single transaction or series of related transactions, sell, assign, pledge, hypothecate or otherwise transfer (or enter into an obligation or agreement regarding the future sale, assignment, pledge or transfer of) (a "Transfer") (I) more than [**] of the aggregate Solid Shares then held by Seller during the first [**] of the Lock-Up Period, or (II) more than [**] of the remaining aggregate Solid Shares then held by Seller during any subsequent [**] period of the Lock-Up Period, in either case ((I) or (II)), other than (1) any Transfer of Solid Shares by Seller to an Affiliate of Seller, (2) any Transfer required by Law or (3) any Transfer that has ben approved in advance by the Board of Directors of Buyer or a duly authorized committee thereof. Any attempted Transfer in violation of this Section 5.10 will be of no effect and null and void, regardless of whether the purported transferee has any actual or constructive knowledge of the Transfer restrictions set forth herein. For the avoidance of doubt, a new Lock-Up Period with respect to the Solid Shares held by Seller will commence after any payment by Buyer to Seller of Milestone Shares as Milestone Consideration pursuant to Section 2.6.

(b) Following the Lock-Up Period, Seller will be entitled to Transfer any Solid Shares in its sole discretion, provided that Seller will not directly or indirectly, in any single transaction or series of related transactions, Transfer any Solid Shares:

(i) other than in accordance with all applicable Laws and the other terms and conditions of this Agreement; or

(ii) to a Person who has filed or is party of a "group" (as defined in Section 13(d) of the Exchange Act) that has filed a Schedule 13D with the SEC in respect of Solid Shares; provided, that such restriction will not apply to Transfers (A) into the public market pursuant to a bona fide, broadly distributed public offering, in each case made pursuant to the registration rights herein, (B) through a bona fide sale into the public market without registration effectuated pursuant to Rule 144 under the Securities Act or (C) in connection with a merger, tender offer or exchange offer or other business combination, acquisition of assets or similar transaction or any change of control transaction involving Buyer (which such transaction has been approved by the Board of Directors of Buyer).

Section 5.11. Reversion Rights.

(a) In the event of Buyer's (i) material breach of its diligence obligations under Section 5.1 that remains uncured [**] after Buyer's receipt of notice of such breach from Seller, (ii) public announcement that it intends to terminate all Exploitation of Products or (iii) written notice to Seller that it intends to terminate all Exploitation of Products, then Seller may in Seller's sole discretion, upon written notice to Buyer (a "Reversion Notice"), elect to exercise the reversion rights set forth in this Section 5.11 (the "Reversion Rights"), effective as of the date Seller delivers the Reversion Notice to Buyer. In such event:

(i) The Parties will promptly, for a period not to exceed [**] following Buyer's receipt of a Reversion Notice, provided that such period shall be automatically extended for an additional [**] so long as the Parties continue to negotiate in good faith (such period, the "Negotiation Period"), negotiate in good faith a written agreement (the "Reversion Agreement") pursuant to which: (A) Buyer would transfer and assign to Seller (I) all of Buyer's right, title and interest in and to the Purchased Assets (including the UPenn License and the Technology licensed pursuant thereto) and (II) all Regulatory Documentation and Regulatory Approvals relating to any Reversion Product(s); (B) at Seller's election and request, Buyer would grant to Seller and its Affiliates a license under the Grant-Back Intellectual Property solely to the extent necessary for Seller and its Affiliates to Exploit any Reversion Product in the Field in the Territory, (C) Buyer would orderly wind-down or transfer, at Seller's election, the conduct of any ongoing Clinical Trials for any Product(s), in accordance with applicable Law and taking into account patient safety matters, and (D) Buyer would transfer to Seller any Product Inventory transferred to Buyer pursuant to Section 5.4 that (I) has not been used in [**] or (II) has been used only for [**]. Without limiting the foregoing, the license grant set forth in the foregoing clause (B) will be royalty-bearing, and the Parties will negotiate in good faith a reasonable royalty on Annual Net Sales (as such term applies to the sale of Reversion Products that use or incorporate Grant-Back Intellectual Property by or on behalf of Seller or any of its Affiliates or licensees or sublicensees, *mutatis mutandis*) of such Reversion Products by or on behalf of Seller or any of its Affiliates or licensees or sublicensees in the Territory.

(ii) If Seller exercises the Reversion Right prior to the achievement of Milestone Event No. 1, Buyer will promptly pay Seller the prorated costs of any Product Inventory transferred to Buyer pursuant to clause (D) of Section 5.11(a)(i) that is not in

usable condition at the time of transfer to Seller due to Buyer's (or its Affiliates', Subsidiaries or their respective vendors') failure to use, store, handle, or transport such Product Inventory in accordance with applicable Law.

(iii) Notwithstanding anything to the contrary in this Section 5.11, at the request of any Third Party licensee of Buyer under the Purchased Assets, Seller will grant to such former licensee a direct license on the same terms granted to such licensee by Buyer, taking into account any difference in license scope, territory and duration of sublicense grant, provided, that (i) such former licensee is not then in default of its license agreement, (ii) agrees in writing to comply with the terms of such new license agreement and (iii) such license agreement with any such former licensee was in effect prior to the date of the Reversion Notice and was entered into on commercially reasonable terms and negotiated on an arm's length basis.

(iv) Subject to the Reversion Agreement (including any obligation set forth in the Reversion Agreement for Buyer to transfer, rather than wind-down, any ongoing Clinical Trials), Buyer will be responsible for the prompt wind-down of Buyer's, its Affiliates' and its and their licensees' Exploitation of any Reversion Product(s) in compliance with applicable Law; provided, that during the [**] period following the effective date of termination, Buyer and its Affiliates and its and their licensees will have the right to sell or otherwise dispose of all any Reversion Product(s) then in its or their respective inventory and any in-progress inventory, provided, further, that Buyer will continue to make payments to Seller on Annual Net Sales of such Reversion Product(s) in accordance with Section 2.6 and Section 2.7.

(v) Notwithstanding anything to the contrary in Section 5.11(a), if Buyer disputes in good faith the existence or materiality of a breach specified in a Reversion Notice, and Buyer provides Seller notice of such dispute within [**], then Seller will not have the Reversion Rights set forth in Section 5.11(a) unless and until it is determined in accordance with Section 7.8 that Buyer has materially breached it diligence obligations under Section 5.1 of this Agreement and has failed to cure such breach within [**] from the date of the Reversion Notice.

(b) If Seller delivers a Reversion Notice to Buyer, then Seller agrees that the Reversion Right shall be its sole and exclusive remedy for such material breach, announcement or notice, provided that the Parties enter into a Reversion Agreement as provided by Section 5.11(a) within the Negotiation Period. In the event Parties are unable to enter into a Reversion Agreement within the Negotiation Period, the delivery of a Reversion Notice to Buyer shall have no impact on, and the rights set forth in this **Section 5.11** shall be in addition to any other rights or remedies to which Seller is entitled at law or in equity (including the Seller's ability to receive legal damages or equitable relief, with respect to any material breach of this Agreement).

ARTICLE VI

INDEMNIFICATION

Section 6.1. Indemnification of Buyer.

(a) Subject to the limitations set forth in this Article VI, from and after the Closing, Seller will indemnify and hold harmless each of Buyer and its Affiliates and each of their respective officers, directors, employees, equity holders, agents, Affiliates and Representatives (each, a "Buyer Indemnified Party") from and against, and compensate and reimburse each Buyer Indemnified Party for, the aggregate amount of any Losses incurred by such Buyer Indemnified Party, whether or not arising from, relating to or otherwise in connection with a claim of a Third Party (each, a "Third Party Claim"), as a result of, arising out of or from, directly or indirectly:

(i) any breach of or inaccuracy in any representation or warranty set forth in Article III (other than Fundamental Representations) or in any Seller Representation Letter as such representation or warranty would read had all references to "material," "materiality," "substantial compliance" or any similar materiality qualifications, been deleted therefrom;

(ii) any breach by Seller of any Fundamental Representation;

(iii) any breach or violation of, or failure to perform by Seller of any covenant or agreement of Seller contained in this Agreement or any Related Document or Seller Representation Letter;

(iv) any claim of Fraud committed by Seller solely in respect of (A) a breach of the representations and warranties set forth in Article III or (B) the negotiation, execution, delivery, or performance of this Agreement;

(v) any Excluded Liabilities or Excluded Assets; and

(vi) the portion of any Transfer Taxes and prorated Taxes allocated to Seller pursuant to Section 5.6.

(b) Notwithstanding anything to the contrary contained herein:

(i) no Buyer Indemnified Party will be entitled to be indemnified pursuant to Section 6.1(a)(i) unless and until the aggregate of all Losses for which the Buyer Indemnified Parties would, but for this Section 6.1(b), be entitled to indemnification hereunder exceeds on a cumulative basis $[**] (the "Seller Indemnity Threshold"), at which time the Buyer Indemnified Parties will be entitled to be indemnified and held harmless by Seller for and against the full amount of such aggregate Losses, including the amount of the Seller Indemnity Threshold; provided, however, that the foregoing Seller Indemnity Threshold of this Section 6.1(b) will not apply to Losses for which the Buyer Indemnified Parties may be entitled pursuant to Section 6.1(a)(ii) or Section 6.1(a)(iv); and

(ii) other than Losses for which the Buyer Indemnified Parties may be entitled pursuant to Section 6.1(a)(iv) in no event will the aggregate amount of Losses for which the Buyer Indemnified Parties will be indemnified and held harmless and entitled to recover (i) under Section 6.1(a)(i) exceed [**] percent ([**]%) of the Indemnity Liability Cap; (ii) pursuant to Section 6.1(a)(ii), exceed the Indemnity Liability Cap; or (iii) under Section 6.1(a)(iii) exceed an amount equal to the Greater Liability Cap.

Section 6.2. Indemnification of Seller Indemnified Parties.

(a) Subject to the limitations set forth in this Article VI, from and after the Closing, Buyer will indemnify Seller and its Affiliates and each of their respective officers, managers, directors, employees, equity holders, agents, Affiliates and Representatives (each a "Seller Indemnified Party") from and against, and compensate and reimburse each Seller Indemnified Party for, any Losses incurred by such Seller Indemnified Party, whether or not arising from, relating to or otherwise in connection with a Third Party Claim, as a result of, arising out of or from, directly or indirectly:

(i) any breach of or inaccuracy in any representation or warranty set forth in Article IV or in any Buyer Representation Letter, as such representation or warranty would read had all references to "material," "materiality," "substantial compliance" or any similar materiality qualifications, been deleted therefrom;

(ii) any breach or violation of, or failure to perform by Buyer of any covenant or agreement of Buyer contained in this Agreement or any Related Document;

(iii) any claim of Fraud committed by Buyer solely in respect of (A) a breach of the representations and warranties set forth in Article IV or (B) the negotiation, execution, delivery, or performance of this Agreement;

(iv) the portion of any Transfer Taxes and prorated Taxes allocated to Buyer pursuant to Section 5.6; and

(v) any Assumed Liabilities.

(b) Notwithstanding anything to the contrary contained herein:

(i) no Seller Indemnified Party will be entitled to be indemnified pursuant to Section 6.2(a)(i) unless and until the aggregate amount of all Losses for which Seller Indemnified Parties would, but for this Section 6.2(b)(i), be entitled to indemnification hereunder exceeds on a cumulative basis $[**] (the "Buyer Indemnity Threshold"), at which point the Seller Indemnified Parties will be entitled to be indemnified and held harmless by Buyer for and against the full amount of such Losses, including the amount of the Buyer Indemnity Threshold; provided, however, that the foregoing Buyer Indemnity Threshold of this Section 6.2(b)(i) will not apply to Losses for which the Seller Indemnified Parties may be entitled pursuant to Section 6.2(a)(iii); and

(ii) other than Losses for which the Seller Indemnified Parties may be entitled pursuant to Section 6.2(a)(iii) in no event will the aggregate amount for which Seller Indemnified Parties will be indemnified and held harmless and entitled to recover (i) under Section 6.2(a)(i) exceed the Indemnity Liability Cap; or (ii) under Section 6.2(a)(ii), exceed an amount equal to the Greater Liability Cap. For clarity, the Greater Liability Cap will not be construed hereunder as a limitation on any payment obligation otherwise due and payable by Buyer pursuant to Section 2.6 (with respect to an achieved Milestone Event) or Section 2.7 (with respect to earned Royalties).

Section 6.3. Indemnification Claims.

(a) In order for a Buyer Indemnified Party or a Seller Indemnified Party (an "Indemnified Party") to be entitled to any indemnification provided for under Section 6.1 or Section 6.2 in respect of, arising out of or involving a Third Party Claim, such Indemnified Party must notify, with respect to a claim for indemnification pursuant to Section 6.1, Seller, or, with respect to a claim for indemnification pursuant to Section 6.2, Buyer (each, an "Indemnifying Party") in writing of the Third Party Claim (including in such notice a brief description of the applicable claim(s), including damages sought or estimated, to the extent actually known by such Indemnified Party) within [**] after receipt by such Indemnified Party of actual notice of the Third Party Claim; provided, however, that failure to give such notification or any deficiency in such notification will not affect the indemnification provided under Section 6.1 or Section 6.2 except to the extent that the Indemnifying Party has been actually prejudiced as a result of such failure or deficiency. The Indemnifying Party will have the right to undertake the defense or opposition to such Third Party Claim (at the Indemnifying Party's expense) with counsel selected by it and reasonably satisfactory to the Indemnified Party so long as (i) the Indemnifying Party gives written notice to the Indemnified Party within [**] after it has been notified of the Third Party Claim that it will defend the Indemnified Party against such Third Party Claim and the Indemnifying Party acknowledges its obligation to indemnify the Indemnified Party for all Losses related to such Third Party Claim, (ii) the Third Party Claim involves only money damages and does not seek an injunction or other equitable relief against the Indemnified Party, (iii) the Third Party Claim does not relate to or arise in connection with Taxes, any purported class action, or any criminal or regulatory enforcement Action, (iv) the amount claimed in such Third Party Claim, taken together with the reasonably estimated costs of defense thereof and the claimed amount with respect to any unresolved claims for indemnification under this Article VI then pending, is (A) greater than the Seller Indemnity Threshold (if Seller is the Indemnifying Party) or the Buyer Indemnity Threshold (if Buyer is the Indemnifying Party) and (B) if applicable, less than the Indemnity Liability Cap, (v) the Indemnified Party has not been advised in writing by outside counsel that a legal conflict exists between the Indemnified Party and the Indemnifying Party in connection with conducting the defense of the Third Party Claim, (vi) the Third Party Claim does not allege the infringement of the Intellectual Property Rights of any Person by the Indemnified Party, (vii) settlement of, an adverse Order with respect to, or conduct of the defense of the Third Party Claim by the Indemnifying Party is not, in the good faith judgment of the Indemnified Party, likely to be materially adverse to the Indemnified Party's or its Affiliates' reputation or continuing business interests (including its relationships with current or potential customers, licensors, distributors, suppliers or other parties material to the conduct of its business) and (viii) the Indemnifying Party diligently and vigorously and in good faith conducts the defense of the Third Party Claim. The Indemnifying Party may not settle any Third Party Claim without the prior written consent of the Indemnified Party unless (1) the claimant in such Third Party Claim provides to the Buyer Indemnified Parties or Seller Indemnified Parties, as applicable, a full, general and unqualified release from all liability in respect of such Third Party Claim, (2) such settlement does not involve any injunctive relief, (3) such settlement does not create a Lien on any of the assets of the applicable Indemnified Parties or impose any restriction or condition that would apply to or materially affect the applicable Indemnified Parties or the conduct of the applicable Indemnified Parties' businesses and (4) such settlement does not involve any finding or admission of liability or wrongdoing.

(b) In order for an Indemnified Party to be entitled to any indemnification provided for under this Agreement other than in respect of, arising out of or involving a Third Party Claim, such Indemnified Party will deliver notice of such claim to the Indemnifying Party (including in such notice a brief description of the applicable claim(s), including damages sought or estimated, to the extent actually known by such Indemnified Party); provided, however, that failure to give such notification or any deficiency in such notification will not affect the indemnification provided under Section 6.1 or Section 6.2 except to the extent that the Indemnifying Party has been actually prejudiced as a result of such failure or deficiency. If the Indemnifying Party does not notify the Indemnified Party within [**] following its receipt of such notice that the Indemnifying Party disputes the indemnity claimed by the Indemnified Party under Section 6.1 or Section 6.2, such indemnity claim specified by the Indemnified Party in such notice will be conclusively deemed a liability to be indemnified under Section 6.1 or Section 6.2 and the Indemnified Party will be indemnified for the amount of the Losses stated in such notice to the Indemnified Party on demand or, in the case of any notice in which the Losses (or any portion thereof) are estimated, on such later date when the amount of such Losses (or such portion thereof) becomes finally determined, but in all cases subject to the Seller Indemnity Threshold, the Buyer Indemnity Threshold, and the Indemnity Liability Cap, to the extent applicable. If the Indemnifying Party objects in writing to such claim, then the Indemnifying Party and Indemnified Party shall attempt in good faith to agree upon the rights of the respective parties with respect to any such claims within [**] after such objection. If the Indemnifying Party and the Indemnified Party should so agree on a claim during such [**] period, a written memorandum setting forth such agreement with respect to the Losses sought shall be prepared and signed by such parties. If no agreement can be reached after good faith negotiation between the Indemnifying Party and Indemnified Party pursuant to this Section 6.3(b), then applicable Indemnified Party shall each have the right to submit such dispute to a court of competent jurisdiction in accordance with the provisions of Section 7.8.

(c) The agreed or finally determined amount of Losses for which an Indemnified Party is responsible pursuant to this Section 6.3 shall be deemed the "Owed Damages."

Section 6.4. Termination of Indemnification Obligations.

(a) The obligations to indemnify and hold harmless an Indemnified Party hereto (i) pursuant to Section 6.1(a)(i) (excluding with respect to the representations and warranties set forth in Section 3.6 (Intellectual Property)) and Section 6.2(a)(i) will terminate when the applicable representation or warranty terminates pursuant to Section 6.4(b), (ii) pursuant to Section 6.1(a)(i) with respect to the representations and warranties set forth in Section 3.6 (Intellectual Property), will terminate when the applicable representation or warranty terminates pursuant to Section 6.4(b), (iii) pursuant to Section 6.1(a)(ii) will terminate when the applicable representation or warranty terminates pursuant to Section 6.4(b), and (iv) pursuant to the other clauses of Section 6.1(a) and Section 6.2(a) will not terminate; provided, however, that as to clauses (i)-(iv) above such obligation to indemnify and hold harmless will not terminate with respect to any claims as to which the Indemnified Party will have, before the expiration of the applicable period, previously delivered a notice of such claim to the Indemnifying Party.

(b) All representations and warranties contained in this Agreement will survive the Closing; provided, however, that except in the case of Fraud (to the extent a Party may claim indemnification pursuant to Section 6.1(a)(iv) or Section 6.2(a)(iii), as applicable, with respect to such Fraud), (i) the Fundamental Representations will terminate on the date that is [**] after the expiration of the applicable statute of limitation (taking into account any tolling periods and other extensions) and (ii) the representations and warranties contained in this Agreement (other than the Fundamental Representations) will terminate on the date that is [**] following the Closing Date. Notwithstanding the foregoing, all representations and warranties contained in a Buyer Representation Letter or a Seller Representation Letter, as applicable, will terminate on the date that is [**] following the date thereof.

(c) The Parties acknowledge and agree that the time periods set forth in this Section 6.4 for the assertion of claims under this Agreement are the result of arms'-length negotiation among the Parties and that they intend for the time periods to be enforced as agreed by the Parties.

Section 6.5. Damages; Remedies.

(a) Except for any claims involving Fraud against any Person who has committed such Fraud, from and after the Closing Date, the rights set forth in this Article VI and Section 5.11 (Reversion Rights) shall be the sole and exclusive remedy of the Indemnified Parties with respect to a breach of a representation or warranty, covenant or obligation set forth in this Agreement. For clarity, the Greater Liability Cap (as applied under Article VI) will not be construed hereunder as a limitation on any payment obligation otherwise due and payable by Buyer pursuant to Section 2.6 (with respect to an achieved Milestone Event) or Section 2.7 (with respect to earned Royalties).

(b) For purposes of this Agreement, all Losses shall be calculated after giving effect to any amounts actually recovered from third parties, including amounts recovered under insurance policies (for the avoidance of doubt, excluding any self-insurance program or similar arrangement) with respect to such Losses, and net of any costs to recover such amounts. Any Losses for which indemnification is sought under this Agreement shall be determined without duplication of recovery due to the facts giving rise to such Losses forming a basis for a claim for recovery under multiple provisions of this Article VI or a breach of more than one representation, warranty, covenant or agreement.

(c) Except as otherwise set forth herein, the rights of each Indemnified Party under this Article VI are cumulative, and each Indemnified Party will have the right in any particular circumstance, in its sole discretion, to enforce any provision of this Article VI without regard to the availability of a remedy under any other provision of this Article VI.

(d) The right of any Indemnified Party to indemnification pursuant to this Article VI will not be affected by any investigation conducted or knowledge acquired (or capable of being acquired) at any time, whether before or after the execution and delivery of this Agreement or the Closing, with respect to the accuracy of any representation or warranty, or performance of or compliance with any covenant or agreement. The waiver of any condition contained in this Agreement based on the breach of any such representation or warranty, or on the

performance of or compliance with any such covenant or agreement, will not affect the right of any Indemnified Party to indemnification pursuant to this Article VI based on such representation, warranty, covenant or agreement.

Section 6.6. Right of Set-off.

(a) If Buyer is entitled to indemnification for Owed Damages pursuant to Section 6.3, subject to the limitations contained in Section 6.1(b) and this Section 6.6, Buyer shall be and hereby is expressly entitled to set off and withhold the amount of any outstanding or unsatisfied indemnity claim made by a Buyer Indemnified Party or payable by Seller hereunder pursuant to this Article VI, against any payments that are or may become payable by Buyer either at the time of such setoff or at any future time to Seller pursuant to this Agreement, including any Milestone Consideration or Royalties, subject to the requirements of this Section 6.6 (which set off shall be allocated and applied against the next Milestone Consideration or the next Royalty, that is or becomes payable to Seller hereunder (if any), and if such Milestone Consideration or Royalty is insufficient to cover the full amount of Owed Damages, then Buyer shall offset such amount against each subsequent Milestone Consideration, that is or becomes payable to Seller hereunder, if any, until such Owed Damages are satisfied in full and if such Milestone Consideration is insufficient to cover the full amount of Owed Damages, then Buyer shall offset such amount against any Royalties that is or become available in the order in which such Milestone Consideration or Royalty becomes payable to Seller pursuant to the terms hereof); provided that the foregoing shall not in any manner limit the right to recover or method of recovery by Buyer of Losses hereunder.

(b) Notwithstanding anything to the contrary, Buyer must seek to recover Owed Damages as follows: (i) first, the Seller shall pay to an account designated by Buyer or the applicable Buyer Indemnified Party, an amount in cash and/or a number of Solid Shares (which Solid Shares will be valued at the VWAP (with the date of measurement being the date that the applicable Milestone Shares were issued pursuant to Section 2.6), equal to the Owed Damages, and (ii) second, if Seller fails to pay, in the aggregate, an amount in cash and/or a number of Solid Shares (which Solid Shares will be valued at the VWAP (with the date of measurement being the date that the applicable Milestone Shares were issued pursuant to Section 2.6), equal to the Owed Damages within [**], then Buyer may offset the Owed Damages from any Milestone Consideration or Royalties that become due and payable to Seller pursuant to this Agreement for the unpaid portion of the Owed Damages.

(c) If Buyer exercises its right of setoff under this Section 6.6, then Buyer will provide written notice to Seller thereof (including in such notice a summary of the applicable claim(s), including Owed Damages subject to such offset to the extent actually known by Buyer); provided, however, that failure to give such notification or any deficiency in such notification will not affect Buyer's rights under this Section 6.6. Seller must notify Buyer within [**] following its receipt of such notice that Seller disputes Buyer's right of set-off under this Section 6.6, and such amount claimed by Buyer will be held by Buyer, to be released to Buyer or Seller, as applicable, after such dispute is finally determined in accordance with the dispute resolution procedures set forth herein. In the case of the exercise of the right to setoff against the payment of any Milestone Shares hereunder, the amount of Owed Damages which may be offset against such payment of Milestone Shares shall be determined as such number of Solid Shares equal to the applicable

amount of Owed Damages divided by the VWAP for the Solid Shares (with the date of measurement being the date that the applicable claim for indemnification was made).

Section 6.7. Purchase Price Adjustments. To the extent permitted by applicable Law, any amounts payable under Article VI will be treated by Buyer and Seller as an adjustment to the purchase price for United States federal income Tax purposes.

Section 6.8. Indemnity Payments. In the event that an Indemnifying Party agrees to or is determined to have an obligation to reimburse an Indemnified Party for Losses as provided in this Article VI, except as otherwise set forth in Section 6.6, the Indemnifying Party will promptly pay such amount to the Indemnified Party in Dollars by wire transfer of immediately available funds to the accounts specified in writing by the Indemnified Party.

ARTICLE VII

GENERAL PROVISIONS

Section 7.1. Rules of Construction. The Parties agree that they have been represented by counsel during the negotiation and execution of this Agreement and have together drafted this Agreement and, therefore, waive the application of any Law, regulation, holding or rule of construction providing that ambiguities in an agreement or other document will be construed against the Party drafting such agreement or document.

Section 7.2. Notices. All notices, requests, claims, demands and other communications hereunder will be given (and will be deemed to have been duly given upon receipt) (a) by hand delivery, (b) by prepaid overnight courier (providing written proof of delivery), (c) by certified or registered mail (return receipt requested and first class postage prepaid), addressed as follows (or at such other address for a Party as will be specified by like notice), or (d) by electronic mail (followed by confirmation via one of the methods set forth in the foregoing (a) - (c)):

if to Buyer, to:

Solid Biosciences Inc.
500 Rutherford Ave, 3rd Floor
Charlestown, MA 02129
Attention: General Counsel
Email: [**]

and

Ropes & Gray LLP
Prudential Tower
800 Boylston Street
Boston, Massachusetts 02199
Attention: Abigail Gregor
Email: Abigail.Gregor@ropesgray.com

if to Seller, to:

FA212 LLC
Attn: Thomas E. Hamilton
[**]

with a copy (which will not constitute notice) to:

Goodwin Procter LLP
One Commerce Square
2005 Market Street, 32nd Floor
Philadelphia, PA 19103
Attention: Rachael M. Bushey; Allison P. Nicklin
Email: RBushey@goodwinlaw.com; ANicklin@goodwinlaw.com

provided that any notice received at the addressee's location (or, if sent by electronic mail, to the appropriate email addresses set forth in this Section 7.2) on any Business Day after 5:00 p.m. (addressee's local time) will be deemed to have been received at 9:00 a.m. (addressee's local time) on the next Business Day.

Section 7.3. Consents and Approvals. For any matter under this Agreement requiring the consent or approval of either Party to be valid and binding on the Party, such consent or approval must be in writing.

Section 7.4. Counterparts. This Agreement may be executed in one or more counterparts (including by facsimile .pdf or other electronically transmitted signatures), all of which will be considered one and the same agreement and will become effective when one or more counterparts have been signed by each of the Parties and delivered to the other Party.

Section 7.5. Entire Agreement; No Third-Party Beneficiaries. This Agreement, the Confidentiality Agreement and the other Related Documents (a) constitute the entire agreement, and supersede all prior agreements and understandings, both written and oral, among the Parties with respect to the subject matter of this Agreement, the Related Documents, and the Confidentiality Agreement and (b) other than in respect of any Indemnified Party pursuant to the terms of Article VI, are not intended to and do not confer upon any Person other than the Parties any legal or equitable rights or remedies.

Section 7.6. Assignment. Neither this Agreement nor any of the rights, interests or obligations hereunder will be assigned, in whole or in part, by operation of Law or otherwise by either of the Parties without the prior written consent of the other Party, and any assignment without such consent will be null and void, except that (a) either Party may assigns any of or all its rights, interests and obligations under this Agreement to one or more of its Affiliates or to a successor in interest in connection with a merger, acquisition, sale of all or substantially all of the assets of such party or other similar transaction, and (b) Seller may assign may assign to a Third Party its right to receive the Milestone Consideration under Section 2.6 or the Royalties under Section 2.7, provided, that the Third Party to which the right to receive payments is assigned is subject to confidentiality obligations and non-use restrictions that are substantially similar to the obligations of confidentiality and non-use set forth in Section 5.5. Subject to the preceding

sentences, this Agreement will be binding upon, inure to the benefit of, and be Enforceable by, the Parties and their respective successors and assigns.

Section 7.7. GOVERNING LAW. THIS AGREEMENT WILL BE GOVERNED BY, AND CONSTRUED IN ACCORDANCE WITH, THE LAWS OF THE STATE OF NEW YORK, REGARDLESS OF THE LAWS THAT MIGHT OTHERWISE GOVERN UNDER APPLICABLE PRINCIPLES OF CONFLICTS OF LAWS THEREOF.

Section 7.8. Dispute Resolution; Enforcement; Waiver of Jury Trial.

(a) Each Party irrevocably submits to the exclusive jurisdiction of (i) the courts of the State of New York located in New York, NY, and (ii) the United States District Court for the Southern District of New York, for the purposes of any suit, Action or other proceeding arising out of this Agreement or the Contemplated Transactions. Each Party agrees to commence any such Action, suit or proceeding either in the United States District Court for the Southern District of New York or if such Action may not be brought in such court for jurisdictional reasons, in the courts of the State of New York located in New York, NY. Each Party further agrees that service of any process, summons, notice or document by the United States registered mail to such Party's respective address set forth above in Section 7.2 will be effective service of process for any Action, suit or proceeding in New York with respect to any matters to which it has submitted to jurisdiction in this Section 7.8(a). Each Party irrevocably and unconditionally waives any objection to the laying of venue of any Action, suit or proceeding arising out of this Agreement or the Contemplated Transactions in (x) the courts of the State of New York located in New York, NY, and (y) the United States District Court for the Southern District of New York, and hereby and thereby further irrevocably and unconditionally waives and agrees not to plead or claim in any such court that any such Action, suit or proceeding brought in any such court has been brought in an inconvenient forum.

(b) EACH PARTY WAIVES ITS RIGHT TO TRIAL OF ANY ISSUE BY JURY. Each Party (i) certifies that no Representative, agent or attorney of the other Party has represented, expressly or otherwise, that such Party would not, in the event of any Action, suit or proceeding, seek to enforce the foregoing waiver and (ii) acknowledges that it and the other Party has been induced to enter into this Agreement, by, among other things, the mutual waiver and certifications in this Section 7.8(b).

Section 7.9. Specific Enforcement. The Parties agree that irreparable damage may occur and that the Parties may not have any adequate remedy at law if any of the provisions of this Agreement were not performed in accordance with their specific terms or were otherwise breached. Notwithstanding anything to the contrary herein, it is accordingly agreed that the Parties will be entitled to an injunction or injunctions to prevent breaches of this Agreement and to enforce specifically the terms and provisions of this Agreement in the state courts of New York located in New York, NY, and the United States District Court for the Southern District of New York, this being in addition to any other remedy to which they are entitled at law or in equity. Each Party hereby covenants and agrees not to raise, and irrevocably waives, any objections to the availability of such relief that a remedy at law would be adequate and that a bond or other security will be required.

Section 7.10. Severability. If any term or other provision of this Agreement or any Related Document is invalid, illegal or incapable of being enforced by any rule of Law or public policy, all other conditions and provisions of this Agreement or such Related Document will nevertheless remain in full force and effect. Upon such determination that any term or other provision is invalid, illegal or incapable of being enforced, the Parties will negotiate in good faith to modify this Agreement or such Related Document so as to effect the original intent of the Parties as closely as possible to the fullest extent permitted by applicable Law in an acceptable manner to the end that the Contemplated Transactions are fulfilled to the extent possible.

Section 7.11. Amendment. This Agreement may be amended by the Parties at any time by an instrument in writing signed on behalf of each of the Parties.

Section 7.12. Release. Effective as of the Closing, each of Seller and Buyer, on behalf of itself and their respective Affiliates, successors and assigns (collectively, as applicable to Seller and Buyer, the "Releasors"), hereby release, acquit and forever discharge, to the fullest extent permitted by Law, the other Party and its Affiliates (each, a "Releasee") of, from and against any and all Actions, causes of Action, claims, demands, damages, judgments, debts, dues and suits of every kind, nature and description whatsoever, in law or in equity, which such Releasor ever had, now has or may in the future have on or by reason of any matter, cause or thing whatsoever related to the Business and the Purchased Assets. Each Releasor covenants and agrees not to assert any claim released hereby against any Releasee. Notwithstanding anything contained in this Section 7.12 to the contrary, the release set forth in this Section 7.12 will not affect or release the obligations of the Parties under this Agreement or the Related Documents.

[Remainder of page intentionally left blank]

IN WITNESS WHEREOF, the Parties have caused this Asset Purchase Agreement to be signed by their respective officers hereunto duly authorized, all as of the date first written above.

SELLER:

FA212 LLC

By: /s/ Thomas E. Hamilton
Name: Thomas E. Hamilton
Title: Manager

[Signature Page to Asset Purchase Agreement]

IN WITNESS WHEREOF, the Parties have caused this Asset Purchase Agreement to be signed by their respective officers hereunto duly authorized, all as of the date first written above.

BUYER:

SOLID BIOSCIENCES INC.

By: /s/ Bo Cumbo
Name: Bo Cumbo
Title: President and CEO

[Signature Page to Asset Purchase Agreement]

Exhibit 19.1

SOLID BIOSCIENCES INC.

INSIDER TRADING POLICY

1. BACKGROUND AND PURPOSE

1.1 ***Why Have We Adopted This Policy?***

The federal securities laws prohibit any employee, officer (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934 (the "Exchange Act"), an "executive officer") or member of the Board of Directors (a "Director") of Solid Biosciences Inc. (together with its subsidiaries, the "Company") from purchasing or selling Company securities on the basis of material nonpublic information concerning the Company, or from tipping material nonpublic information to others. These laws impose severe sanctions on individuals who violate them. In addition, the Securities and Exchange Commission (the "SEC") has the authority to impose large fines on the Company and on the Company's Directors, executive officers and controlling stockholders if the Company's employees engage in insider trading and the Company has failed to take appropriate steps to prevent it (so-called "controlling person" liability).

This Insider Trading Policy (this "Policy") is being adopted in light of these legal requirements, and with the goal of helping:

- prevent inadvertent violations of the insider trading laws;
- avoid embarrassing proxy disclosure of reporting violations by persons subject to Section 16 of the Exchange Act;
- promote compliance with the Company's obligation to publicly disclose information related to its insider trading policies and procedures and the use of certain trading arrangements by Company insiders;
- avoid the appearance of impropriety on the part of those employed by, or associated with, the Company;
- protect the Company from controlling person liability; and
- protect the reputation of the Company, its employees and its Directors.

As detailed below, this Policy applies to family members and certain other individuals and entities with whom employees, executive officers or Directors have relationships. While the provisions in Sections 2 and 3 of this Policy are not applicable to transactions by the Company itself, transactions by the Company will only be made in accordance with applicable U.S. federal securities laws, including those relating to insider trading.

1.2 ***What Type of Information is "Material"?***

Information concerning the Company is considered material if there is a substantial likelihood that a reasonable stockholder would consider the information important in making an

investment decision with respect to the Company's securities. Stated another way, there must be a substantial likelihood that a reasonable stockholder would view the information as having significantly altered the "total mix" of information available about the Company. Material information can include positive or negative information about the Company. Information concerning any of the following subjects, or the Company's plans with respect to any of these subjects, would often be considered material:

- the Company's liquidity, cash burn rate, revenues, earnings or losses (including forecasts);
- information concerning the Company's ongoing and planned clinical trials and preclinical studies, including the timing of and findings and data from such trials and studies;
- the results of clinical trials and certain preclinical studies;
- information concerning Food and Drug Administration actions or other significant regulatory developments, including a significant product recall;
- a change in control of the Company;
- a significant merger or acquisition involving the Company;
- a significant licensing or collaboration agreement or serious discussions regarding such an agreement;
- a significant change in the management or the Board of Directors of the Company;
- changes in dividend policy, declaration of a stock split, establishment of a repurchase program, or the offering or sale of a significant amount of securities of the Company;
- a default on outstanding debt of the Company or a bankruptcy filing;
- a new product release or a significant development, invention or discovery;
- the loss, delay or gain of a significant contract, sale or order or other important development regarding customers, collaborators or suppliers;
- any litigation or disputes to which the Company may be a party;
- a significant operational issue or investigation of a potential such issue, including cybersecurity incidents and product defects; or
- changes in, or disputes with, the Company's auditors or a notification from its auditors that the Company may no longer rely on the auditor's audit report.

This list is illustrative only and is not intended to provide a comprehensive list of circumstances that could give rise to material information; many other types of information may

be considered "material," depending on the circumstances. The materiality of particular information is subject to reassessment on a regular basis.

1.3***When is Information "Nonpublic"?***

Information concerning the Company is considered nonpublic if it has not been disseminated in a manner making it available to investors generally. Information will generally be considered nonpublic unless (1) the information has been disclosed in a press release, in a public filing made with the SEC (such as a Report on Form 10-K, Form 10-Q or Form 8-K), or through a news wire service or daily newspaper of wide circulation, and (2) a sufficient amount of time has passed so that the information has had an opportunity to be digested by the marketplace.

Whether a particular item is "material" or "nonpublic" will be judged with hindsight. Accordingly, when in doubt as to a particular item of information, you should presume it is material and has not been disclosed to the public. Chances are, if you learn something that leads *you* to want to buy or sell securities, that information may be considered material. It is important to keep in mind that material information need not be *certain* information - information that something is likely to happen, or even just that it may happen, can affect the market price of the securities and therefore, in hindsight, may be determined to be material. Do not hesitate to contact the Company's General Counsel with any questions you may have. However, in all cases, the responsibility for determining whether an individual is in possession of material nonpublic information rests with the individual, and any action on the part of the Company, members of the Company's legal or finance departments or any other employee pursuant to this Policy does not in any way constitute legal advice or insulate an individual from liability under securities laws.

2. PROHIBITIONS RELATING TO TRANSACTIONS IN THE COMPANY'S SECURITIES

2.1As used in Section 2:

(a) "Covered Persons" means all Company Parties and their Related Persons.

(b) "Company Parties" means all employees, all executive officers and all Directors.

(c) "Related Persons" means any family member of a Company Party who shares the same address as, or is financially dependent on, the Company Party and any other person who shares the same address as the Company Party (other than (x) an employee or tenant of the Company Party or (y) another unrelated person whom the General Counsel determines should not be covered by this Policy); and all corporations, limited liability companies, partnerships, trusts or other entities controlled by any Covered Person, unless the entity (i) has implemented policies or procedures designed to ensure that such Covered Person cannot influence transactions by the entity involving Company securities or (ii) is a professional investment organization (e.g. venture capital fund, investment fund, registered investment company or registered investment advisor) and has established policies and procedures designed to ensure its compliance with federal securities laws and regulations prohibiting trading in the securities of a company on the basis of material, nonpublic information.

2.2Prohibition on Trading While Aware of Material Nonpublic Information.

(a) Prohibited Activities. Except as provided in Section 4, no Covered Person may:

- purchase, sell or gift (which term, as used in this policy, includes charitable donations) any securities of the Company while such Covered Person is aware of any material nonpublic information concerning the Company or recommend doing so to someone else; or

- tip or otherwise disclose to someone else any material nonpublic information concerning the Company if the recipient may use that information to purchase, sell or gift Company securities or tip that information to others.

In addition, no Covered Person who, in the course of service to the Company, learns of material nonpublic information about another company (1) with which the Company does business, such as the Company's collaborators, vendors, customers and suppliers, or (2) that is involved in a potential transaction or business relationship with the Company, may purchase, sell or gift that other company's securities until the information becomes public or is no longer material, or tip or otherwise disclose to someone else such information if the recipient may use that information to purchase, sell or gift that other company's securities or tip that information to others.

(b) Application of Policy After Cessation of Service. If an individual or entity ceases to be a Covered Person at a time when such individual or entity is aware of material nonpublic information concerning the Company, the prohibitions on purchasing, selling and gifting of securities in Section 2.2(a) shall continue to apply until that information has become public or is no longer material.

2.3Prohibition on Pledges. No Covered Person may purchase Company securities on margin, borrow against Company securities held in a margin account, or pledge Company securities as collateral for a loan. However, an exception may be granted in extraordinary situations where a Covered Person wishes to pledge Company securities as collateral for a loan (other than a margin loan) and clearly demonstrates the financial capacity to repay the loan without resort to the pledged securities. Any Covered Person who wishes to pledge Company securities as collateral for a loan must submit a request for approval to the Chief Financial Officer and the General Counsel. In addition, any such request by a Director or executive officer must also be reviewed and approved by the Audit Committee of the Board of Directors.

2.4Prohibition on Short Sales, Derivative Transactions and Hedging Transactions. No Covered Person may engage in any of the following types of transactions with respect to Company securities:

(a) short sales, including short sales "against the box"; or

(b) purchases or sales of puts, calls or other derivative securities; or

(c) purchases of financial instruments (including prepaid variable forward contracts, equity swaps, collars and exchange funds) or other transactions that hedge or offset, or are designed to hedge or offset, any decrease in the market value of Company securities.

3. ADDITIONAL PROHIBITIONS APPLICABLE TO DIRECTORS, EXECUTIVE OFFICERS AND DESIGNATED EMPLOYEES

3.1 As used in Section 3:

(a) "Designated Covered Persons" means Designated Corporate Persons and their Designated Related Persons.

(b) "Designated Corporate Persons" means all Directors and executive officers and such other Company Parties holding positions identified by the Board of Directors, the Chief Executive Officer, the Chief Financial Officer or the General Counsel as Designated Corporate Persons from time to time.

(c) "Designated Related Persons" means any family member of a Designated Corporate Person who shares the same address as, or is financially dependent on, the Designated Corporate Person and any other person who shares the same address as the Designated Corporate Person (other than (x) an employee or tenant of the Designated Corporate Person or (y) another unrelated person whom the General Counsel determines should not be covered by this Policy); and all corporations, limited liability companies, partnerships, trusts or other entities controlled by any Designated Covered Person, unless the entity (i) has implemented policies or procedures designed to ensure that such Designated Covered Person cannot influence transactions by the entity involving Company securities or (ii) is a professional investment organization (e.g. venture capital fund, investment fund, registered investment company or registered investment advisor) and has established policies and procedures designed to ensure its compliance with federal securities laws and regulations prohibiting trading in the securities of a company on the basis of material, nonpublic information.

3.2 Blackout Periods.

(a) Regular Blackout Periods. Except as provided in Section 4, no Designated Covered Person may purchase, sell or gift any securities of the Company during the period beginning on the last day of each fiscal quarter and ending upon the completion of the first full trading day after the public announcement of earnings for such quarter (a "regular blackout period").

(b) Corporate News Blackout Periods. The Company may from time to time notify the Designated Corporate Persons that an additional blackout period (a "corporate news blackout period") is in effect in view of significant events or developments involving the Company. In such event, except as provided in Section 4, no person who is notified of a corporate news blackout period may purchase, sell or gift any securities of the Company during such corporate news blackout period or inform anyone else that a corporate news blackout period is in effect. (In this Policy, regular blackout periods and corporate news blackout periods are each referred to as a "blackout period").

(c) Awareness of Material Nonpublic Information when a Blackout Period is Not in Effect. Even if no blackout period is then in effect, if a Designated Covered Person is aware of material nonpublic information the prohibitions contained in Section 2.2(a) apply.

3.3Notice and Pre-Clearance of Transactions.

(a) Pre-Transaction Clearance. No Designated Covered Person may purchase, sell, gift, transfer, or otherwise acquire or dispose of securities of the Company, either directly or indirectly, other than in a transaction permitted under Section 4, unless such Designated Covered Person pre-clears the transaction with either the Chief Financial Officer or General Counsel. A request for pre-clearance shall be made in accordance with the procedures established by the General Counsel or, if there is no General Counsel, the Chief Financial Officer. The Chief Financial Officer and the General Counsel (or either of them acting singly) shall have sole discretion to decide whether to clear any contemplated transaction. The General Counsel (or, if none, the Chief Executive Officer) shall have sole discretion to decide whether to clear transactions by the Chief Financial Officer or by Designated Covered Persons subject to this Section 3 as a result of their relationship with the Chief Financial Officer, and the Chief Financial Officer (or, if none, the Chief Executive Officer) shall have sole discretion to decide whether to clear transactions by the General Counsel or by Designated Covered Persons subject to this Section 3 as a result of their relationship with the General Counsel. All transactions that are pre-cleared must be effected within three business days of receipt of the pre-clearance unless a longer or shorter period has been specified by the General Counsel or the Chief Financial Officer, as applicable. A pre-cleared transaction (or any portion of a pre-cleared transaction) that has not been effected during the three business day period must be pre-cleared again prior to execution. **Notwithstanding receipt of pre-clearance, if the Designated Covered Person becomes aware of material nonpublic information or becomes subject to a blackout period before the transaction is effected, the transaction may not be completed.**

(b) Post-Transaction Notice. Certain Designated Covered Persons are subject to reporting obligations, requirements, trading restrictions and "short swing" profit recovery provisions under Section 16 of the Exchange Act (the "Section 16 Officers"). In order to facilitate compliance with Section 16 reporting requirements, each Section 16 Officer must promptly report to the General Counsel or Chief Financial Officer, or their respective designees, the occurrence of any purchase, sale, gift, transfer, or other acquisition or disposition of securities of the Company as soon as possible following the transaction, but in any event within one business day after the transaction. Such notification should be in writing (including by e-mail) and should include the identity of the Designated Covered Person, the type of transaction, the date of the transaction, the number of shares involved, the purchase or sale price, and whether the transaction was effected pursuant to a contract, instruction or written plan that is intended either to satisfy the affirmative defense conditions of Rule 10b5-1(c) (and if so, the date of adoption of such contract, instruction or written plan) or to constitute a non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K).

(c) Deemed Time of a Transaction. For purposes of this Section 3.3, a purchase, sale, gift, transfer, or other acquisition or disposition shall be deemed to occur at the time the person becomes irrevocably committed to it (for example, in the case of an open market purchase or sale, this occurs when the trade is executed, not when it settles).

4. **EXCEPTIONS.**

4.1The prohibitions in Sections 2.2(a) (Prohibited Activities) and 3.2 (Blackout Periods) on purchases, sales and gifts of Company securities do not apply in the case of the following transactions:

(a) Stock Option Exercises. Exercises of stock options or vesting of other equity awards or the surrender of shares to the Company in payment of the exercise price or in satisfaction of any tax withholding obligations, in each case in a manner permitted by the applicable equity award agreement; provided, however, that the securities so acquired may not be sold (either outright or in connection with a "cashless" exercise transaction through a broker) while the Covered Person is aware of material nonpublic information or during an applicable blackout period (as defined in Section 3.2(b));

(b) 401(k) Plan. Acquisitions or dispositions of Company common stock under the Company's 401(k) or other individual account plan that are made pursuant to standing instructions, in a form approved by the Company, not entered into or modified while the Covered Person is aware of material nonpublic information or during an applicable blackout period;

(c) Purchases from the Company. Other purchases of securities from the Company (including purchases under the Company's employee stock purchase plan pursuant to standing instructions, in a form approved by the Company) or sales of securities to the Company; provided, however, that if the transaction involves the exercise of stock options or other equity awards, the transaction must be permitted by Section 4.1(a) above;

(d) Bona Fide Gifts. Bona fide gifts that are approved in advance by the Company;

(e) 10b5-1 Plans. Purchases, sales or gifts made pursuant to a binding contract, written plan or specific instruction which satisfies the applicable affirmative defense conditions of Rule 10b5-1(c), including as applicable the requirements applicable to an eligible sell-to-cover transaction as defined in Rule 10b5-1(c)(1)(ii)(D)(3), or for which the affirmative defense is available under Rule 10b5-1(c) because such plan was adopted prior to February 27, 2023, met the affirmative defense conditions in effect at the time of adoption, and was not modified or changed on or after February 27, 2023 (a "trading plan"); provided such trading plan: (1) is in writing and (2) was submitted to the Company for review prior to its adoption; and

(f) Non-Rule 10b5-1 Trading Arrangements. Purchases, sales or gifts made pursuant to a binding contract, written plan or specific instruction which satisfies the definition of a "non-Rule 10b5-1 trading arrangement" as such term is defined in Item 408(c) of Regulation S-K, provided such non-Rule 10b5-1 trading arrangement: (1) is in writing and (2) was submitted to the Company for review prior to its adoption.

4.2Partnership Distributions. Nothing in this Policy is intended to limit the ability of a venture capital partnership or other similar entity with which a Director is affiliated to distribute Company securities to its partners, members or other similar persons. It is the responsibility of each affected Director and the affiliated entity, in consultation with their own counsel (as

appropriate), to determine the timing of any distributions, based on all relevant facts and circumstances and applicable securities laws.

4.3Underwritten Public Offering. Nothing in this Policy is intended to limit the ability of any Covered Person to sell Company securities as a selling stockholder in an underwritten public offering pursuant to an effective registration statement in accordance with applicable securities law.

5. REGULATION BTR

If the Company is required to impose a "pension fund blackout period" under Regulation BTR, each Director and executive officer shall not, directly or indirectly sell, purchase or otherwise transfer during such blackout period any equity securities of the Company acquired in connection with the service of such person as a Director or officer of the Company, except as permitted by Regulation BTR.

6. PENALTIES FOR VIOLATION OF POLICY

Violation of any of the foregoing rules is grounds for disciplinary action by the Company, including termination of employment. In addition to any disciplinary actions the Company may take, insider trading can also result in administrative, civil or criminal proceedings which can result in significant fines and civil penalties, being barred from service as an officer or director of a public company, or imprisonment.

7. COMPANY ASSISTANCE AND EDUCATION

7.1Education. The Company shall take reasonable steps designed to ensure that all Directors and employees of the Company are educated about, and periodically reminded of, the federal securities law restrictions and Company policies regarding insider trading.

7.2Assistance. The Company shall provide reasonable assistance to all Directors and executive officers, as requested by such Directors and executive officers, in connection with the filing of Forms 3, 4 and 5 under Section 16 of the Exchange Act. However, the ultimate responsibility, and liability, for timely filing remains with the Directors and executive officers.

7.3Limitation on Liability. None of the Company, the Chief Financial Officer, the General Counsel or the Company's other employees will have any liability for any delay in reviewing, or refusal of, a request to allow a pledge submitted pursuant to Section 2.3, a request for pre-clearance submitted pursuant to Section 3.3(a) or a trading plan submitted pursuant to Section 4.1. Notwithstanding any pre-clearance of a transaction pursuant to Section 3.3(a) or review of a trading plan pursuant to Section 4.1, none of the Company, the Chief Financial Officer, the General Counsel or the Company's other employees assumes any liability for the legality or consequences of such transaction or trading plan to the person engaging in or adopting such transaction or trading plan.

Amended and restated effective as of March 6, 2025.

Exhibit 21.1

Subsidiaries of Solid Biosciences Inc.

Name of Subsidiary	Jurisdiction of Organization
AavantiBio, Inc.	Delaware
Solid Biosciences Securities Corporation	Massachusetts

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the incorporation by reference in the Registration Statements on Form S-3 (Nos. 333-277871, 333-276764, 333-269424, 333-254310 and 333-233594) and Form S-8 (Nos. 333-280116, 333-277869, 333-272456, 333-270765, 333-269162, 333-268643, 333-265351, 333-258856, 333-241370 and 333-222763) of Solid Biosciences Inc. of our report dated March 6, 2025 relating to the financial statements, which appears in this Form 10-K.

/s/ PricewaterhouseCoopers LLP
Boston, Massachusetts
March 6, 2025

Exhibit 31.1

CERTIFICATION OF PERIODIC REPORT UNDER SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Alexander Cumbo, certify that:

1. I have reviewed this Annual Report on Form 10-K of Solid Biosciences Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 6, 2025

By: /s/ Alexander Cumbo
Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION OF PERIODIC REPORT UNDER SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Kevin Tan, certify that:

1. I have reviewed this Annual Report on Form 10-K of Solid Biosciences Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 6, 2025

By: /s/ Kevin Tan
Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Solid Biosciences Inc. (the "Company") on Form 10-K for the year ended December 31, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Alexander Cumbo, Chief Executive Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: March 6, 2025

By: /s/ Alexander Cumbo
Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report of Solid Biosciences Inc. (the "Company") on Form 10-K for the year ended December 31, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Kevin Tan, Chief Financial Officer of the Company, certify, pursuant to 18 U.S.C. § 1350, as adopted pursuant to § 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and result of operations of the Company.

Date: March 6, 2025

By: /s/ Kevin Tan
Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)