2023 ANNUAL REPORT

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, 2023

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 30, 2023, 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 $51.5 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 5, 2024 was 37,756,877.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant intends to file a definitive proxy statement pursuant to Regulation 14A within 120 days of the end of the fiscal year ended December 31, 2023. Portions of such definitive proxy statement are incorporated by reference into Part III of this Annual Report on Form 10-K.

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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 and results of ongoing and planned preclinical studies and clinical trials for our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates;
- our ability to establish or maintain collaborations or strategic relationships, including our collaboration with Ultragenyx Pharmaceutical Inc., or Ultragenyx;
- our ability to obtain and maintain U.S. and foreign regulatory approval of our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, and the timing and scope thereof;
- the size of the patient populations and potential market opportunity for our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our plans to develop and commercialize our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, if approved;
- the pricing and reimbursement of our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) 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, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) or other future candidates, if approved;
- the rate and degree of market acceptance and clinical utility of our neuromuscular (e.g., SGT-003, AVB-202-TT), cardiac (e.g., AVB-401, SGT-501) 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 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 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 in the "Risk Factors" section of this Annual Report on Form 10-K. These risks include the following:

- We may fail to realize the anticipated benefits of our acquisition of AavantiBio, those benefits may take longer to realize than expected, and we may encounter significant integration difficulties.
- Our stockholders may not realize a benefit from the Acquisition and the related private placement commensurate with the ownership dilution they experienced in connection with the Acquisition and the related private placement.
- 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 gene transfer candidates are based on 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 gene transfer candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- One of our clinical trials has been placed on clinical hold by the FDA in the past, and we cannot guarantee that similar events will not happen in future clinical trials for our Candidates.
- We have never completed a clinical trial and may be unable to do so for any product candidate, including SGT-003 and other Candidates.
- 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 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 more narrow 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-501, or other current and future candidates. In addition, changes to manufacturing sites or processes, or formulations for our product 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 product 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-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia, or CPVT, and additional assets for the treatment of 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 patients with neuromuscular and cardiac diseases;
- 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.

On December 2, 2022, we completed our acquisition of AavantiBio, Inc., or AavantiBio, a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia, or FA, and rare cardiomyopathies, or the Acquisition. Upon the consummation of the Acquisition, we acquired AavantiBio's gene therapy programs, AVB-202-TT for the treatment of FA and AVB-401 for the treatment of BAG3-mediated dilated cardiomyopathy, or DCM, additional assets for the treatment of cardiac diseases, platform technologies and know-how related thereto.

Our Pipeline

We are focused on developing transformative treatments to improve the lives of patients with rare neuromuscular and cardiac diseases. Our current programs are all 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 are absent or not functional prior to treatment, potentially offering long-lasting clinical benefit. 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. 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.

Lead Neuromuscular Program

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 results 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, and for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments. 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 certain pediatric patients with Duchenne, Duchenne represents a significant societal and economic burden. The economic burden, estimated at $1.2 billion annually in the United States (excluding costly mortality and end-of-life care expenses), includes costs associated with hospital admissions, medication, frequent doctor visits and investment in assistive devices, as well as indirect costs related to productivity losses for the caregivers and costs due to pain, anxiety and social handicap. Of this amount, approximately 45% is represented by indirect costs. 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 multistakeholder 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

Our lead neuromuscular program is directed to Duchenne. Our efforts are focused on our gene transfer candidate, SGT-003, which is designed to address the underlying genetic cause of Duchenne by delivering a synthetic transgene that produces dystrophin-like protein that is only expressed in muscles of the body, including skeletal, cardiac and respiratory muscles and is agnostic to specific mutations in the gene. Our Duchenne candidate capsid is derived from a naturally occurring, non-pathogenic virus called AAV, which was selected for its ability to efficiently enter skeletal, diaphragm and cardiac muscle tissues. The capsid is designed to carry a synthetic dystrophin transgene construct, called microdystrophin, that retains the most critical components of the full-size dystrophin gene yet is small enough to fit within AAV packaging constraints and a muscle specific promoter.

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 subsequent published studies, Drs. Duan and 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 prompt gene expression specifically in muscle tissues. In comparison to other regulatory cassettes, we chose CK8 due to its small size and its ability 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.

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.

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. In addition, international clinical trial application submissions are planned to initiate beginning in the first half of 2024. Our INSPIRE Duchenne trial is a Phase 1/2 first in human, open-label, multicenter trial to determine the safety and tolerability of SGT-003 in pediatric patients with Duchenne at a dose of 1E14vg/kg. We anticipate dosing the first patient in the second quarter of 2024. SGT-003 will be administered as a one-time intravenous infusion to patients in two cohorts with a minimum of three patients each, with the potential for cohort expansion. Cohort 1 will study patients aged four to less than six years of age with Duchenne. Cohort 2 will study patients aged six to less than eight years of age. We plan to evaluate long-term safety and efficacy for a total of five years following treatment.

We anticipate providing an initial safety update of the INSPIRE Duchenne trial, in mid-2024, subject to initiating patient dosing in the second quarter of 2024, and we anticipate providing initial data from the INSPIRE Duchenne trial in the second half of 2024.

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

Lead Cardiac Program

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 twelve years. CPVT is an inherited cardiac arrhythmia syndrome characterized by adrenergically induced polymorphic arrythmias in the presence of a normal resting sinus rhythm and a structurally normal heart. It is estimated that the prevalence of CPVT is 1 per 10,000 persons.

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 (up to 50%). 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 the underlying causes 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 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 caused by gain of function mutation in the ryanodine receptor 2 (coded for by the RYR2 gene), which is referred to as CPVT-1, as well as loss of function mutations in the calsequestrin 2, or CASQ2, gene, which is referred to as CPVT-2. Mutations in RYR2 or CASQ2 genes disrupt cardiac calcium, or Ca^{++}, release into the cytoplasm triggering abnormal contraction and relaxation leading to arrythmias.

Our approach focuses on AAV-mediated therapeutic overexpression 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 CPVT-1 and CPVT-2.

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.

Two nonclinical mouse studies have demonstrated proof of concept for CASQ2 gene replacement using a recombinant AAV8 serotype capsid encoding CASQ2 to mitigate effects associated with CPVT.

We believe that the SGT-501 construct design and emerging preclinical data supports further development of SGT-501 for the treatment of CPVT. We are conducting preclinical studies of SGT-501 to identify the minimally active dose and to define our good laboratory practices toxicology study plans. We anticipate submitting an IND to the FDA for SGT-501 for the treatment of patients with CPVT-1 in early 2025. The FDA and the European Medicines Agency, or EMA, have granted orphan drug designation for SGT-501 for the treatment of CPVT.

Other Candidates

Cardiac

We are currently developing a preclinical stage candidate, AVB-401, for the treatment of BAG3-mediated DCM utilizing the AAVrh74 capsid and a muscle-specific promoter. 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. Preclinical data in wild type mice indicate that the AAVrh74 capsid and muscle-specific promoter combination demonstrated enhanced cardiac biodistribution and expression and decreased liver expression relative to an AAV9 capsid and constitutive promoter combination at doses of 5E13 vg/kg or less.

We also have two cardiac pipeline gene transfer programs, SGT-601 (formerly AVB-501) for TNNT2 DCM and SGT-701 (formerly AVB-601) for RBM20 DCM, that are both currently in early preclinical development. 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 leads to heart failure. RBM20 DCM is a rare inherited 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 and is correlated with high rates of heart failure, arrhythmias, and sudden cardiac death.

Neuromuscular

We also have a neuromuscular gene transfer program for the treatment of FA, a rare, inherited, multisystem genetic disease having both neurological and cardiac manifestations caused by loss of functional frataxin protein, or FXN, due to defects in both copies of the frataxin gene. Proof of concept data in animal models suggest that gene therapy may be a viable treatment for FA. Our approach for the treatment of FA is to target both the neurological and cardiac impairments experienced by patients through dual route of administration (intravenous and intrathecal) of gene transfer therapy to more comprehensively target disease pathology. Preclinical data in cardiac-specific FXN knockout mice supported enhanced survival and cardiac function. We continue to evaluate construct design and potential preclinical testing with a focus on the neurodegenerative portions of the disease.

Platform Technologies

In addition to our gene transfer candidates, we have development programs focusing on platform technologies, including novel capsid libraries 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 and cardiac capsid libraries using two approaches designed to enhance skeletal 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 novel AAV capsids, including SLB-101, 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 supports 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. We are continuing to develop this rationally designed novel capsid library. Using both directed evolution and rational design, we are also developing a library 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.

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 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 and SGT-501. The transient transfection process was selected in order to efficiently advance SGT-003 and SGT-501 along their respective development timelines.

We currently rely on third-party manufacturers for SGT-003 and SGT-501 and plan to rely on third-party manufacturers for our 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.

We are supplying, and expect to continue to supply, our ongoing and future 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.

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 29, 2024, 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. 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 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 two issued U.S. patents, two pending U.S. non-provisional patent applications, and sixteen granted patents and eight 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.

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, 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 one patent family 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.

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, 2023, 2022, and 2021. 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 with Ultragenyx, or the Collaboration Agreement, 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, 2023, 2022, and 2021. 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, have entered into several license agreements with the University of Florida Research Foundation, Inc., or UFRF. Broadly, the agreements relate to FA and our early stage cardiac candidates, including AVB-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 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.

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 €1,500, 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 €15,000, cumulative sales milestone payments of up to €15,000, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits.

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.

Ultragenyx Collaboration Agreement

On October 22, 2020, or the Effective Date, we entered into a collaboration and license agreement with Ultragenyx, to focus on the development and commercialization of new gene therapies for Duchenne. We granted Ultragenyx an exclusive worldwide license for any pharmaceutical product that expresses our proprietary microdystrophin construct from AAV8 and variants thereof in clade E for the treatment of Duchenne and other diseases resulting from the lack of functional dystrophin. We retain exclusive rights to all other uses of our microdystrophin proteins, including under our SGT-003 program.

We have conducted and may conduct in the future certain activities agreed to by the parties with respect to the development of licensed products. Ultragenyx is obligated to reimburse us for personnel and out-of-pocket costs that we incur in conducting such development activities. Otherwise, Ultragenyx has decision-making authority with respect to the development, manufacturing and commercialization of licensed products. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement and an investor agreement with Ultragenyx, pursuant to which we issued and sold 521,719 shares of our common stock to Ultragenyx at a price of $76.669 per share for an aggregate purchase price of approximately $40.0 million. The shares purchased by Ultragenyx were subject to a lock-up period until the earliest to occur of (i) 18 months from the closing date, (ii) the termination of the Collaboration Agreement or (iii) other specified events. Pursuant to the terms of the investor agreement, Ultragenyx agreed that, so long as it holds at least 10% of our outstanding common stock, the shares will be subject to a voting agreement, such that until the earliest to occur of certain specified events, and subject to specified conditions, Ultragenyx will, and will cause its permitted transferees to, vote in accordance with the recommendation of our Board of Directors with respect to specified matters.

Ultragenyx also agreed to pay up to $255.0 million in cumulative milestone payments per product upon achievement of specified milestone events, and tiered royalties on worldwide net sales at low double digit to mid-teens percentages. Upon achievement of proof-of-concept, we have the right to opt-in to co-fund collaboration programs in return for participation in a profit share or increased royalty payments. None of the payments under the Collaboration Agreement are refundable.

For each licensed product for which Ultragenyx decides to initiate a registrational trial in humans, we have the option to fund 30% of the development costs in the United States and European Union for such licensed product and forgo the development milestones and regulatory milestones, or the Development Option, and receive tiered royalties on a licensed product-by-licensed product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's, and any of its affiliates' and sublicensees' annual worldwide net sales of each such licensed product.

For each licensed product for which we exercise the Development Option, we may also elect to share 30% of the net income and net losses on net sales of such licensed product in the United States and European Union, or the Income Share Option. For licensed products for which we have exercised the Income Share Option, we will not be entitled to milestone payments and Ultragenyx will pay us tiered royalties on a licensed product-by-licensed product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's, and any of its affiliates' and sublicensees', annual net sales of each such licensed product outside of the United States and European Union.

We and Ultragenyx established a Joint Steering Committee, or the JSC. The JSC will, among other responsibilities, review and oversee certain development activities performed under the Collaboration Agreement, including reviewing the development plan and budget for the development activities to be performed by us.

The term of the Collaboration Agreement began on the Effective Date and expires upon the expiration of all payment obligations from Ultragenyx to us under the Collaboration Agreement. Ultragenyx also has the ability to terminate for convenience with prior written notice to us, and either party may terminate for an uncured material breach.

As described in Note 3, we entered into the Collaboration Agreement with Ultragenyx for the research, development and commercialization of other pharmaceutical products that express our MD5 nNOS binding domain form of microdystrophin protein. During the year ended December 31, 2023, we recognized no revenue associated with the Collaboration Agreement. As of December 31, 2023, there was no deferred revenue related to the Collaboration Agreement. There are no amounts due from Ultragenyx as of December 31, 2023.

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, cardiac diseases 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 December 2023, Sarepta announced that it submitted a supplemental biologics license application, or BLA, to broaden the approved indication for ELEVIDYS to all patients (all ages and ambulation status) with Duchenne, and on February 16, 2024 Sarepta announced that the FDA accepted the supplemental BLA for priority review and set a PDUFA date of June 21, 2024. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including Pfizer Inc. with a product candidate currently in Phase 3 clinical development, 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 clinical development.

We are also aware of several companies and research institutions conducting clinicals 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, record keeping, distribution, 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 good laboratory practices, or 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 nonclinical and clinical trial sites that generated the data in support of the BLA;
- payment of user fees;
- FDA review and licensure of the BLA; 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 nonclinical 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 nonclinical 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. 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 board or committee, or DSMB, or 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.

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 NIH's Final Rule on registration and reporting requirements for clinical trials became effective in 2017. Although the FDA has historically not enforced these reporting requirements due to the Department of Health and Human Services', or HHS's, long delay in issuing final implementing regulations, the FDA has issued several pre-notices for voluntary corrective action and several notices of non-compliance during the past two years. 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 FD&C Act with violations subject to potential civil monetary penalties of up to $10,000 for each day the violation continues.

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.

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 study is conducted under an IND, all IND requirements must be met unless waived. When a foreign clinical study 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 studies. The FDA's regulations are intended to help ensure the protection of human subjects enrolled in non-IND foreign clinical studies, as well as the quality and integrity of the resulting data. They further help ensure that non-IND foreign studies 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 investigations 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 2024 is approximately $4.05 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 2024 is currently $416,734 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.

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 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 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 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.

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. 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 after the confirmatory trial fails to verify the product's clinical benefit. Further, FDORA requires the FDA to publish on its website "the rationale for why a post-approval study is not appropriate or necessary" whenever it decides not to require such a study upon granting accelerated approval. In March 2023, the FDA issued draft guidance that outlines its current thinking and approach to 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 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. 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. Further, in October 2023, the FDA published draft guidance outlining the FDA's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use..

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 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 before the end of 2026. 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 during the 2024 legislative sessions that will go into effect in 2025 and beyond. 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 in 2024. 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.

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 remain governed by said directive until January 31, 2025. After this date, all clinical trials (including those which are ongoing) will become subject to the provisions of the CTR.

Parties conducting certain clinical studies must post clinical trial information in the European Union at the EU Clinical Trials Register.

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 close 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. Except conditional marketing authorizations, marketing authorizations have an initial duration of five years. After these five years, the authorization may be renewed on the basis of a reevaluation of the risk-benefit balance.

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.

On February 27, 2023, the UK government and the European Commission announced a political agreement in principle to replace the Northern Ireland Protocol with a new set of arrangements, known as the "Windsor Framework". This new framework fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the UK In particular, the MHRA will be 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. A single UK-wide marketing authorization will be granted by the MHRA for all medicinal products to be sold in the UK, enabling products to be sold in a single pack and under a single authorization throughout the UK The Windsor Framework was approved by the EU-UK Joint Committee on March 24, 2023, so the UK government and the European Union will enact legislative measures to bring it into law. On June 9, 2023, the MHRA announced that the medicines aspects of the Windsor Framework will apply from January 1, 2025. The Human Medicines Regulations 2012 (SI 2012/1916) (as amended), or HMR, is the primary legal instrument for the regulation of medicines in the UK. 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.

EU laws which have been transposed into UK law through secondary legislation continue to be applicable as "retained EU law." However, new legislation such as the (EU) Clinical Trials Regulation will not be applicable in Great Britain. Since a significant proportion of the regulatory framework for pharmaceutical products in the UK covering the quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorizations, commercial sales, and distribution of pharmaceutical products is derived from EU directives and regulations, Brexit may have a material impact upon the regulatory regime with respect to the development, manufacture, importation, approval, and commercialization of our product candidates in the UK. For example, the UK is no longer covered by the centralized procedures for obtaining EU-wide marketing authorizations from the EMA, and a separate marketing authorization will be required to market our product candidates in the UK. A new international recognition framework has been in place since January 1, 2024, whereby the MHRA will have regard to decisions on the approval of marketing authorizations made by the EMA and certain other regulators when determining an application for a new Great Britian marketing authorization.

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.

Although the previous administration took executive actions to undermine or delay implementation of the Health Care Reform Law, President Biden rescinded those actions with the issuance of an Executive Order on January 28, 2021, which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the Health Care Reform Law that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the Health Care Reform Law; and policies that reduce affordability of coverage or financial assistance, including for dependents.

Pharmaceutical Prices

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. There have been several recent 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 2020, the prior administration issued several executive orders intended to lower the costs of prescription products and certain provisions in these orders have been incorporated into regulations. These regulations include an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

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. Nine 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. In January 2024, the FDA approved Florida's plan for Canadian drug importation.

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 (first due in 2023); 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. 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 $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

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 the U.S. Chamber of Commerce and pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. 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. 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 diverse group of employees. Our efforts to recruit and retain a diverse and passionate workforce 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, 2023, we employed 88 full-time employees, including 65 in research and development and 23 in general and administrative positions, and of which 19 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 the Acquisition

We may fail to realize the anticipated benefits of our acquisition of AavantiBio, those benefits may take longer to realize than expected, and we may encounter significant integration difficulties.

On December 2, 2022, we completed our acquisition, or the Acquisition, of AavantiBio, Inc., or AavantiBio, and acquired AavantiBio's pipeline programs which included cardiac and neuromuscular programs. Our ability to realize the anticipated benefits of the Acquisition will depend, to a large extent, on our ongoing ability to integrate AavantiBio and these cardiac and neuromuscular programs into our business and business strategy and realize anticipated growth opportunities and synergies. We may fail to realize some or all of the anticipated benefits of the Acquisition. Potential difficulties we may encounter in the integration process include the following:

- the inability to successfully combine the businesses of Solid and AavantiBio in a manner that permits us to achieve the anticipated benefits from the Acquisition, which would result in the anticipated benefits of the Acquisition not being realized partly or wholly in the time frame currently anticipated or at all;
- difficulties in managing the expanded operations of a more complex company following the Acquisition;
- creation of uniform standards, controls, procedures, policies and information systems;
- difficulties in assimilating AavantiBio employees in our business, in maintaining employee morale and in attracting and retaining key personnel; and
- potential unknown liabilities, adverse consequences, or unforeseen increased expenses, delays or regulatory conditions associated with the Acquisition.

Also, we now possess certain liabilities and obligations, including contractual liabilities and obligations, that were assumed by us upon closing of the Acquisition. Further, it is possible that undisclosed, contingent or other liabilities, problems or obligations may arise in the future of which we were previously unaware. These disclosed and undisclosed liabilities could have an adverse effect on our business, financial condition and results of operations.

Any or all of these factors could decrease or delay the expected accretive effect of the Acquisition and negatively impact our stock price. As a result, it cannot be assured that we will be successful in the integration of AavantiBio with our business or that we will realize the benefits anticipated from the Acquisition or in the anticipated time frames or at all.

Our stockholders may not realize a benefit from the Acquisition and the related private placement commensurate with the ownership dilution they experienced in connection with the Acquisition and the related private placement.

If we are unable to realize the full strategic and financial benefits anticipated from the Acquisition, our stockholders will have experienced substantial dilution of their ownership interests without receiving any commensurate benefit, or only receiving part of the commensurate benefit to the extent the combined company is able to realize only part of the benefits anticipated from the Acquisition and the related private placement.

We may be exposed to increased litigation, including stockholder litigation, which could have an adverse effect on our business and operations.

We may be exposed to increased litigation from stockholders, customers, suppliers, consumers and other third parties due to the combination of Solid's and AavantiBio's businesses following the Acquisition. Such litigation may have an

adverse impact on our business and results of operations or may cause disruptions to our operations. In addition, in the past, stockholders have initiated class action lawsuits against biotechnology companies following periods of volatility in the market prices of these companies' stock. Such litigation, if instituted against us, could cause us to incur substantial costs and divert management's attention and resources, which could have a material adverse effect on our business, financial condition and results of 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 $96.0 million, $86.0 million and $72.2 million for the years ended December 31, 2023, 2022 and 2021, respectively. As of December 31, 2023, we had an accumulated deficit of $658.8 million. Prior to the Acquisition, we had 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. 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:

- enroll patients in our INSPIRE Duchenne trial and advance clinical development of SGT-003;
- advance our other Candidates into clinical trials;
- 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;
- 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;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to our collaboration and license agreement with Ultragenyx; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more product 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 enroll patients in and conduct the INSPIRE Duchenne trial 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 and otherwise integrate the operations of AavantiBio into our business. 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, 2023, together with the net proceeds from our private placement that closed in January 2024, will be sufficient to fund our operating expenses and capital requirements into 2026, 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 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;
- the success of our collaboration with Ultragenyx;
- 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, such as the recent COVID-19 pandemic, and collateral consequences related thereto.

Identifying potential product 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 biologic license application, or BLA, or obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenue, if any, will be derived from or based on sales of product 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, such as the recent COVID-19 pandemic, 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, with respect to the development of SGT-001, which we are no longer developing, and SGT-003, 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, establishing arrangements for the manufacture of SGT-001 and SGT-003, identifying SGT-001 and SGT-003 as potential gene transfer candidates and undertaking preclinical studies and clinical trials of SGT-001 and SGT-003. Following the Acquisition, we have expanded our operations to include the development of additional Candidates. As a company, we have limited experience in clinical development and we have not yet demonstrated the ability to complete clinical trials of any product 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.

Public health emergencies or pandemics, including the recent COVID-19 pandemic, may affect our ability to initiate and complete current or future preclinical studies or clinical trials, disrupt regulatory activities, disrupt our manufacturing and supply chain or have other adverse effects on our business and operations.

Public health emergencies or pandemics, including the recent COVID-19 pandemic, could adversely affect our business, financial condition, results of operations, and prospects.

We and our third-party manufacturers for supply of drug product for our candidates, and prospective contract research organizations, or CROs, may face disruptions as a result of such pandemics that may affect our ability to initiate and complete preclinical studies or clinical trials, including disruptions in procuring items that are essential for our research and development activities, including, for example, raw materials used in the manufacturing of drug product for our candidates, and laboratory supplies for our current and future preclinical studies and clinical trials, in each case, for which there may be shortages because of ongoing efforts to address the outbreak. We and our third-party manufacturers, and prospective CROs, may face disruptions related to future clinical trials arising from delays in IND-enabling studies, manufacturing disruptions, and the ability to obtain necessary institutional review board or other necessary site approvals, as well as other delays at clinical trial sites.

We may also face difficulties recruiting or enrolling patients for our clinical trials if patients are affected by the recent COVID-19 pandemic or other public health emergencies or are fearful of visiting or traveling to, or unable to travel to, clinical trial sites. For example, we experienced a few missed or postponed patient visits in our IGNITE DMD trial for SGT-001, which we are no longer developing, due to site closures early in the COVID-19 pandemic.

The response to public health emergencies or pandemics may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

While the public health emergency declarations related to COVID-19 ended on May 11, 2023 the FDA retained a number of COVID-19 related policies. It is unclear how, if at all, these policies will impact our efforts to develop and commercialize our product candidates.

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 product 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 product candidates

Our gene transfer candidates are based on 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.

We are evaluating SGT-003 for the treatment of Duchenne and are advancing a portfolio of programs for the treatment of other rare genetic diseases, and our future success depends on our successful development of these 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 product 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 product 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 European Medicines Agency, or 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 regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

Our 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 product 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 included 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. The FDA convened the Cellular, Tissue, and Gene Therapies Advisory Committee in September 2021 to discuss toxicity risks of AAV based gene therapy products. Discussed risks included oncogenicity risks due to capsid genome integration, hepatotoxicity, thrombotic microangiopathy, and neurotoxicity (especially related to dorsal root ganglion toxicity). If any such 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 product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product 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 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 future clinical trials.

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 product 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 product candidate, including SGT-003 and other Candidates.

We are early in our development efforts and we have never completed a clinical trial. We anticipate dosing our first patient in our INSPIRE Duchenne trial in the second quarter of 2024. All of our other 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 and complete our ongoing clinical trials in order to obtain FDA approval to market SGT-003 and other Candidates. We have limited experience in preparing, submitting and prosecuting regulatory submissions, and have not previously submitted a BLA for any product 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 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 or other Candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product 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 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 future clinical trial results and are not necessarily indicative of final results. Our preclinical studies for certain Candidates in animals have been limited. We have not dosed human subjects with SGT-003 or any of our other Candidates. 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 and clinical activities 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 product 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 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 product 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 IRB 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 product 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, such as the recent COVID-19 pandemic or from the outbreak of another pandemic or contagious disease or other global instability could delay the initiation or rate of completion of any clinical trial; 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. For example, 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 drug or biological product. Similarly, the regulatory landscape related to clinical trials in the EU recently evolved. If we are not able to adapt to these and other 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 product 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 product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product 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 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 or other Candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-003 and 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 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, such as the recent COVID-19 virus 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 product 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 product 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. Similarly, the regulatory landscape related to clinical trials in the EU recently evolved. 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 studies 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.

Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of gene therapy protocols;
- difficulty in identifying and partnering with qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology research and products.

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 more narrow indication than we seek.

We cannot commercialize our Candidates until the appropriate regulatory authorities have reviewed and approved the product 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 product 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 product 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.

Even if we receive regulatory approval, regulatory authorities may approve a product 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 product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates.

Even if we obtain regulatory approval for a product candidate, our product 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 product 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 ongoing litigation challenging the FDA's approval of mifepristone. Specifically, in April 2023, the U.S. District Court for the Northern District of Texas stayed the approval by the FDA of mifepristone, a drug product which was originally approved in 2000 and whose distribution is governed by various conditions adopted under a REMS. In reaching that decision, the district court made a number of findings that may negatively impact the development, approval and distribution of drug products in the U.S. Among other determinations, the district court held that plaintiffs were likely to prevail in their claim that FDA had acted arbitrarily and capriciously in approving mifepristone without sufficiently considering evidence bearing on whether the drug was safe to use under the conditions identified in its labeling. Further, the district court read the standing requirements governing litigation in federal court as permitting a plaintiff to bring a lawsuit against the FDA in connection with its decision to approve an new drug application or establish requirements under a REMS based on a showing that the plaintiff or its members would be harmed to the extent that FDA's drug approval decision effectively compelled the plaintiffs to provide care for patients suffering adverse events caused by a given drug.

On April 12, 2023, the district court decision was stayed, in part, by the U.S. Court of Appeals for the Fifth Circuit. Thereafter, on April 21, 2023, the U.S. Supreme Court entered a stay of the district court's decision, in its entirety, pending disposition of the appeal of the district court decision in the Court of Appeals for the Fifth Circuit and the disposition of any petition for a writ of certiorari to or the Supreme Court. The Court of Appeals for the Fifth Circuit held oral argument in the case on May 17, 2023 and, on August 16, 2023, issued its decision. The court declined to order the removal of mifepristone from the market, finding that a challenge to the FDA's initial approval in 2000 is barred by the statute of limitations. But the Appeals Court did hold that plaintiffs were likely to prevail in their claim that changes allowing for expanded access of mifepristone that FDA authorized in 2016 and 2021 were arbitrary and capricious. On September 8, 2023, the Justice Department and a manufacturer of mifepristone filed petitions for a writ of certiorari, requesting that asked the U.S. Supreme Court to review the Appeals Court decision. On December 13, 2023, the Supreme Court granted these petitions for writ of certiorari for the appeals court decision.

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 product 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 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 October 2023, the FDA published draft guidance outlining the agency's non-binding policies governing the distribution of scientific information on unapproved uses to healthcare providers. This draft guidance calls for such communications to be truthful, non-misleading, factual, and unbiased and include all information necessary for healthcare providers to interpret the strengths and weaknesses and validity and utility of the information about the unapproved use. In addition, under some relatively recent guidance from the FDA and the Pre-Approval Information Exchange Act, or PIE Act, signed into law as part of the Consolidated Appropriations Act of 2023, companies may also promote information that is consistent with the prescribing information and proactively speak to formulary committee members of payors regarding data for an unapproved drug or unapproved uses of an approved drug. We may engage in these discussions and communicate with healthcare providers, payors and other constituencies in compliance with all applicable laws, regulatory guidance and industry best practices. We will need to carefully navigate the FDA's various regulations, guidance and policies, along with recently enacted legislation, to ensure compliance with restrictions governing promotion of our products.

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 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 FD&C 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 product 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 product candidate in the United States by the FDA does not ensure approval of such product 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 product 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 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 product 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, 2021, the MHRA became responsible for supervising medicines and medical devices in Great Britain, comprising England, Scotland and Wales under domestic law, whereas under the terms of the Northern Ireland Protocol, Northern Ireland is currently subject to European Union rules. The United Kingdom and European Union have however agreed to the Windsor Framework which fundamentally changes the existing system under the Northern Ireland Protocol, including with respect to the regulation of medicinal products in the United Kingdom. Once implemented, the changes introduced by the Windsor Framework will see the MHRA be 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 product 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 (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. 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 product 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 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.

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 product 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 product 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 product 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 product 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 product 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 product 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 product candidates; however, we cannot assure our stockholders that one or more of our product 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 biologics license application 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 product 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 product 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 product candidates qualifies as a breakthrough therapy, the FDA may later decide that the product 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 product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek approval of one or more of our product 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 product candidates, or withdrawal of a product candidate, would result in a longer time period until commercialization of such product candidate, could increase the cost of development of such product candidate and could harm our competitive position in the marketplace.

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

We may seek a regenerative medicine advanced therapy designation for some of our product 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 studies, 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 product 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 product 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 product 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 product 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 product 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 product 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 product candidates. However, a BLA for such product 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, 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, 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.

We may seek a fast track designation for one or more of our product 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 product candidates.

If a therapy is intended for the treatment of a serious condition and nonclinical 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 product 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.

We may seek priority review designation for one or more of our product 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 product candidates, however, we cannot assume that one or more of our product 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 product 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.

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 product 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 December 2023, Sarepta announced that it submitted a supplemental BLA to broaden the approved indication for ELEVIDYS to all patients (all ages and ambulation status) with Duchenne, and on February 16, 2024 Sarepta announced that the FDA accepted the supplemental BLA for priority review and set a PDUFA date of June 21, 2024. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including Pfizer Inc. with a product candidate currently in Phase 3 clinical development, 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 clinical development. We are also aware of several companies and research institutions conducting clinicals 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.

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 product candidate that we may develop. Changes within the competitive landscape could lead us to alter 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 product 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 product 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.

In addition, in October 2020, we entered into a collaboration and license agreement with Ultragenyx, pursuant to which we granted Ultragenyx an exclusive worldwide license under certain intellectual property rights controlled by us to develop AAV8 or other clade E AAV variant pharmaceutical products that express our MD5 nNOS binding domain form of microdystrophin protein for the treatment of Duchenne and other disease indications resulting from a lack of functional dystrophin, which we refer to as the Licensed Products.

Our spending on current and future research and development programs may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product 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 product candidate. Alternatively, we may allocate internal resources to a product 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 product candidate or fail to develop a potentially successful product candidate.

Risks related to the manufacturing and commercialization of our product candidates

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

In October 2020, we entered into a collaboration and license agreement with Ultragenyx, pursuant to which we granted Ultragenyx an exclusive worldwide license under certain intellectual property rights controlled by us to develop the Licensed Products.

While we have retained all rights to and are developing on our own SGT-003, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to SGT-003 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, including our collaboration with Ultragenyx, 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 product 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 product 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 product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates on a discretionary basis;
- collaborators, including Ultragenyx, could develop products that compete directly or indirectly with our product candidates and products pursuant to the collaboration;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product 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;
- product 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 product candidates;
- a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;
- a collaborator with marketing and distribution rights to one or more of our product 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 product candidates, might lead to additional responsibilities for us with respect to product 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 product candidates.

Collaboration agreements may not lead to development or commercialization of product 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 product candidates could be delayed and we may need additional resources to develop our product 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 product 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 product 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 product 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-501, or other current and future candidates. In addition, changes to manufacturing sites or processes, or formulations for our product candidates may result in additional cost or delay.

We have limited experience manufacturing SGT-003, SGT-501 and our other current or future 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 optimize 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. While we have observed positive results in preclinical studies 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 preclinical studies to date. Similarly, in the future we may further optimize 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 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-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-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 third-party manufacturers for SGT-003 and SGT-501 and plan to rely on third-party manufacturers for our Candidates. In order to produce sufficient quantities of product 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 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. For example, we have not released a manufacturing lot for clinical supply utilizing the transient transfection-based manufacturing process and 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-501 and other Candidates could be delayed, and we could incur additional expense. Any such failure could delay or prevent our IND or commercialization of SGT-003, SGT-501 or other Candidates.

If supply from a manufacturing facility is interrupted, including as a result of 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 product candidates as we transfer our manufacturing technology to these manufacturers and as they gain experience manufacturing our product 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 product 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 product 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 product 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 product 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 product candidates.

In addition, we do not currently have long-term supply or manufacturing arrangements in place for the production of our product 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 product 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 product 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

product 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 product candidates, which will expose us to risks including:

- reduced control of manufacturing activities;
- the inability of certain CMOs to produce our product 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 product 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 product 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 product 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 product 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 product 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 product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which our product 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 product 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 product 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 product 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 product candidates. If our product 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 product 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 product 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 product 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 product 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 product 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 product 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 product candidates. Accordingly, in markets outside the United States, the reimbursement for our product 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 product 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 product 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.

Our strategic plan and associated workforce reductions may not result in anticipated savings, could result in total costs and expenses that are greater than expected and could disrupt our business.

In April 2022 and December 2022, we announced a reduction in workforce by approximately 35% and 18%, respectively, as part of a strategic plan designed to streamline our operating structure. We may not realize, in full or in part, the anticipated benefits, savings and improvements in our cost structure from our restructuring efforts due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected operational efficiencies and cost savings from the restructuring, our operating results and financial condition would be adversely affected. We also cannot guarantee that we will not have to undertake additional workforce reductions or restructuring activities in the future. Furthermore, our strategic restructuring plan and the Acquisition may be disruptive to our operations. For example, our workforce reductions and integration of AavantiBio's business and operations into ours could yield unanticipated consequences, such as attrition beyond planned staff reductions, or increase difficulties in our day-to-day operations. Our workforce reductions and the Acquisition could also harm our ability to attract and retain qualified management, scientific, clinical, manufacturing and sales and marketing personnel who are critical to our business. Any failure to attract or retain qualified personnel could prevent us from successfully developing and commercializing our product candidates in the future.

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 product 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 product 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 for 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 product candidates for which we may obtain regulatory approval or the frequency with which any such product 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 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.

Although the previous administration took actions to undermine or delay implementation of the Health Care Reform Law, President Biden rescinded those actions with the issuance of an Executive Order on January 28, 2021 which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the Health Care Reform Law that may reduce coverage or undermine the programs,

including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the Health Care Reform Law; and policies that reduce affordability of coverage or financial assistance, including for dependents. This Executive Order also directs the U.S. Department of Health and Human Services to create a special enrollment period for the Health Insurance Marketplace in response to the recent COVID-19 pandemic.

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 prices of prescription pharmaceuticals have been the subject of considerable discussion in the United States. To date, there have been several recent 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 products. In 2020, CMS issued an interim final rule implementing a most favored nation model for prices that would tie Medicare Part B payments for certain physician-administered pharmaceuticals to the lowest price paid in other economically advanced countries, effective January 1, 2021. That rule, however, has been subject to a nationwide preliminary injunction and, on December 29, 2021, CMS issued a final rule to rescind it. With issuance of this rule, CMS stated that it will explore all options to incorporate value into payments for Medicare Part B pharmaceuticals and improve beneficiaries' access to evidence-based care.

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, 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. Nine 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. In January 2024, the FDA approved Florida's plan for Canadian drug importation. 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 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 (first due in 2023); 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. 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 $4,000 a year in 2024 and, thereafter beginning in 2025, at $2,000 a year.

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 the U.S. Chamber of Commerce and pharmaceutical companies, also filed lawsuits in various courts with similar constitutional claims against HHS and CMS. 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. 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 product 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 product 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 product 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 product candidates, restrict or regulate post-approval activities and affect our ability to commercialize our product 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 product 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, a number of 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 before the end of 2026. 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 in 2025 and beyond. 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.

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 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 product 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 product 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 product candidate that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product 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 product 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 anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product 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 product 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. 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 product 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 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 product 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 product 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 our 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 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 product 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 product candidates or will effectively prevent others from commercializing competitive products.

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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product candidates. Such challenges may also result in our inability to manufacture or commercialize our product 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 product 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 product 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 product 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 product candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our product 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 product 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, product 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.

In each of our existing license agreements, and we expect in our future agreements, patent prosecution of our licensed technology is controlled solely by the licensor, and we may be required to reimburse the licensor for their costs of patent prosecution. 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 product 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 product 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 product 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 product candidates infringes its patent, the patent holder may sue us even if we have licensed other patent protection for our product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 product 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 data 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 product 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 product 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 product 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 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 achieve the anticipated benefits of the Acquisition and 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, such as the recent COVID-19 pandemic, 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 product candidates, or our clinical development programs and our commercialization efforts;
- the results of our efforts to discover, develop, acquire or in-license additional product 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 product 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"; not being required to furnish a contractual obligations table in "Management's Discussion and Analysis of Financial Condition and Results of Operations"; and not being required to furnish a stock performance graph in our annual report.

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, 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 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's 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 sixteen years of experience in information technology, ten 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 and Florida.

Item 3. Legal Proceedings.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

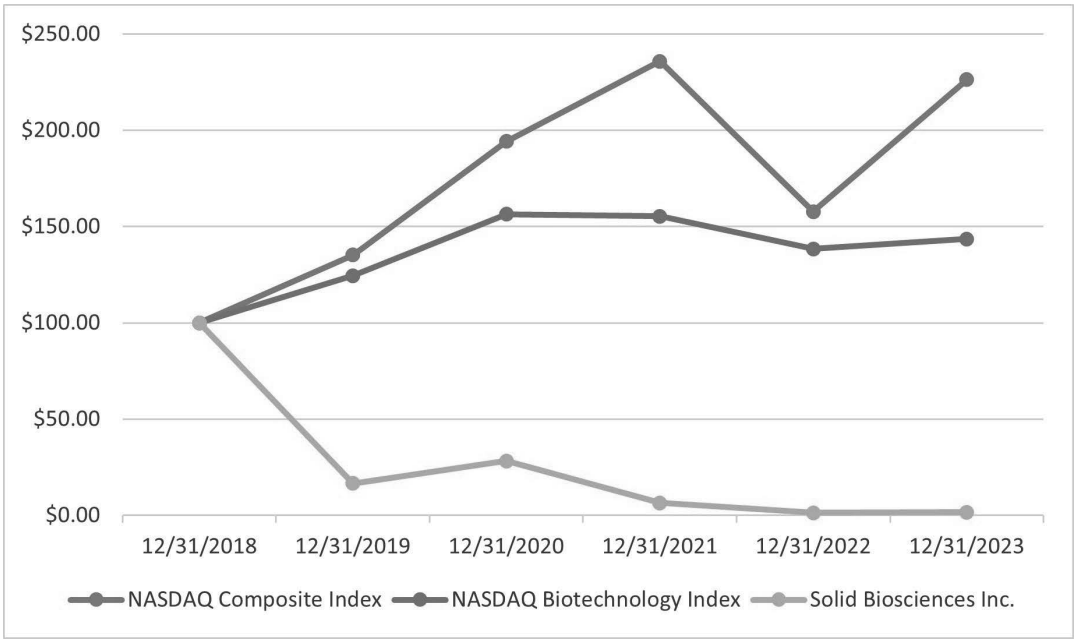
Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

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, 2018 through December 31, 2023. The comparison assumes $100 was invested after the market closed on December 31, 2018 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 February 13, 2024, we had approximately 57 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

We did not sell any securities, during the year ended December 31, 2023 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.

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.

Unless otherwise indicated, all information in this Annual Report on Form 10-K gives effect to a 1-for-15 reverse stock split of our common stock that became effective on October 27, 2022, and all references to historical share and per share amounts give effect to the reverse stock split.

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-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia, or CPVT, and additional assets for the treatment of 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 Solid expands 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 patients with neuromuscular and cardiac diseases;
- 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.

On December 2, 2022, we completed our acquisition of AavantiBio, Inc., or AavantiBio, a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia, or FA, and rare cardiomyopathies, or the Acquisition. Upon the consummation of the Acquisition, we acquired AavantiBio's gene therapy programs, AVB-202-TT for the treatment of FA and AVB-401 for the treatment of BAG3-mediated dilated cardiomyopathy ("DCM"), additional assets for the treatment of cardiac diseases, platform technologies and know-how related thereto.

Our Operations

We are focused on developing transformative treatments to improve the lives of patients with rare neuromuscular and cardiac diseases. Our current programs are all 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 are absent or not functional prior to treatment, potentially offering long-lasting clinical benefit. 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. 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.

Due to our significant research and development expenditure, licensing and patent investment, and general administrative costs associated with our operations, we have generated substantial operating losses in each period since our inception. Our net losses were $96.0 million, $86.0 million and $72.2 million for the years ended December 31, 2023, 2022

and 2021, respectively. As of December 31, 2023, we had an accumulated deficit of $658.8 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, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

As of December 31, 2023, we had cash, cash equivalents, and available-for-sale securities of $123.6 million, excluding restricted cash of $1.8 million. In January 2024, we sold to investors in a private placement, or the January 2024 Private Placement, 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 approximately $104.0 million of aggregate net proceeds, after deducting offering costs. We believe that our cash, cash equivalents, and available-for-sale securities as of December 31, 2023, together with the net proceeds from the January 2024 Private Placement, will enable us to fund our operating expenses and capital expenditure requirements into 2026. 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

Collaboration revenue

There was no collaboration revenue for the year ended December 31, 2023. Collaboration revenue was $8.1 million for the year ended December 31, 2022. We recognized this revenue related to research services and cost reimbursement from the collaboration and license agreement, or the Collaboration Agreement, with Ultragenyx Pharmaceutical Inc., or Ultragenyx. No other research and development has commenced under the Collaboration Agreement.

Product revenue

We have not generated any product revenue to date and do not expect to generate any product revenue from the sale of our products, if approved, for the foreseeable future, if ever. If our development efforts for our Candidates are successful and result in marketing approval, we may generate 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 each of these 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 the development of SGT-003 and other Candidates and include:

- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct research and preclinical activities on our behalf, as well as contract manufacturing organizations, or CMOs, that manufacture SGT-003 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 SGT-003 and other 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. Product 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 and continue to identify and develop additional candidates.

We typically use our employee and infrastructure resources across our product candidates. We track outsourced development costs and milestone payments made under our licensing arrangements by product candidates, but we do not allocate personnel costs, license payments made under our licensing arrangements or other internal costs to product 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 product candidates for the respective periods:

	For the Year Ended December 31,	
(in thousands)	**2023**	**2022**
SGT-001	$ 3,432	$ 24,844
SGT-003	20,856	9,995
SGT-501	3,200	-
Other development programs	7,439	3,450
Unallocated research and development expenses		
Personnel related expenses	24,968	24,883
External expenses	16,668	15,248
Total unallocated research and development expenses	41,636	40,131
Total research and development expenses	$ 76,563	$ 78,420

We cannot determine with certainty the duration, costs and timing of clinical trials of SGT-003 or other Candidates, or if, when or to what extent we will generate revenue from the commercialization and sale of any of our product 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 product 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, acquisition costs, and facility-related expenses, which include direct depreciation costs and allocated expenses for rent and maintenance of office facilities and other operating costs.

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 and any planned or future clinical trials for and potential commercialization of our Candidates.

Restructuring charges

In April 2022, we implemented changes to our corporate strategy. In connection with the changes to corporate operations, we reduced headcount by approximately 35 percent.

In November 2022, we approved a plan designed to streamline our operating structure in connection with the Acquisition. In connection with the plan, we reduced headcount by approximately 18 percent in December 2022.

Other income (expense), net

Other income (expense), net consists of interest income earned on our cash, cash equivalents, available-for-sale securities, amortization of investment premium or accretion of investment discount.

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 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.

Revenue recognition

As discussed in Note 2 to our consolidated audited financial statements, under Accounting Standards Codification, or ASC 606, Revenue from Contracts with Customers, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services.

To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, we perform the following five steps: (i) identification of the contract(s) with the customer, (ii) identification of the promised goods or services in the contract and determination of whether the promised goods or services are performance obligations, (iii) measurement of the transaction price, (iv) allocation of the transaction price to the performance obligations, and (v) recognition of revenue when (or as) we satisfy each performance obligation. We only apply the five-step model to contracts when it is probable that the entity will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer.

When optional goods or services are offered, we assess the options to determine whether the options grant the customer a material right. This determination includes whether the option is priced at an amount that the customer would not have received without entering into the contract. If we conclude the option conveys a material right, it is accounted for as a separate performance obligation. In identifying performance obligations in a contract, we identify those promises that are distinct. Promised goods or services are considered distinct when the customer can benefit from the goods or services on their own, or together with readily available resources, and the goods or services are separately identifiable from other promises in the contract. If a promise is not distinct, it is combined with other promises in the contract until the combined group of promises is capable of being distinct.

We estimate the transaction price based on the amount of consideration we expect to receive for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of each arrangement that includes variable consideration, we evaluate the amount of the potential payments and the likelihood that the payments will be received. If it is probable that a significant revenue reversal would not occur, the variable consideration is included in the transaction price. For contracts that include sales-based royalties for licensed compounds, we recognize revenue at the date when the related sales occur. Finally, we determine whether the contract contains a significant financing component by analyzing the promised consideration relative to the standalone selling price of the promised goods and services and the timing of payment relative to the transfer of the promised goods and services. At each reporting date, we reassess the transaction price and probability of achievement of the performance obligations and the associated constraints on transaction price. If necessary, we adjust the transaction price, recording a cumulative catch-up based on progress for the amount that was previously constrained.

Revenue is recognized when (or as) control of a performance obligation is transferred to the customer. When combined performance obligations contain a promised license and related services or other promises, management judgment is required to determine the appropriate timing of revenue recognition. In doing so, we must identify the predominant promise or promises in the contract to determine whether revenue is recognized at a point in time or over time. If over time, we must determine the appropriate measure of progress. If a license is deemed to be the predominant promise in a performance obligation, we must determine the nature of the license, whether functional or symbolic intellectual property, to conclude whether point-in-time or over-time revenue recognition is most appropriate. The determination of functional or symbolic intellectual property requires an assessment of whether the customer is able to exploit and benefit from the license in its

current condition, or if the utility of the license is dependent on or influenced by our ongoing activities or being associated with us.

At each reporting date, we calculate the measure of progress for the performance obligations transferred over time. The calculation generally uses an input measure based on costs incurred to-date relative to estimated total costs to complete the transfer of the performance obligation. The measurement of progress is then used to calculate the total revenue earned, including any cumulative catch-up adjustment.

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 on our behalf and conducting clinical trials and preclinical studies on our behalf;
- vendors in connection with preclinical development 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 received 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. We have not issued any awards with performance-based vesting conditions. 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. We historically have been a private company and lack company-specific historical and implied volatility information. Therefore, we estimate our expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded stock price. For options with service-based vesting conditions and options granted to non-employees, the expected term of our stock options 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. Expected dividend yield is 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.

Results of operations

Comparison of the years ended December 31, 2023 and 2022

The following table summarizes our results of operations for the years ended December 31, 2023 and 2022:

	For the Year Ended December 31,		Increase	%
	2023	2022	(decrease)	Change
(in thousands)				
Collaboration revenue - related party	$ —	$ 8,094	$ (8,094)	(100)%
Operating expenses:				
Research and development	76,563	78,420	(1,857)	(2)%
General and administrative	27,752	28,948	(1,196)	(4)%
Restructuring expense	(63)	7,178	(7,241)	(101)%
Total operating expenses	104,252	114,546	(10,294)	(9)%
Loss from operations	(104,252)	(106,452)	2,200	2%
Other income, net:				
Interest income, net	7,142	2,616	4,526	173%
Gain on acquisition	—	18,236	(18,236)	(100)%
Other income (expense)	1,095	(381)	1,476	(387)%
Total other income, net	8,237	20,471	(12,234)	(60)%
Net loss	$ (96,015)	$ (85,981)	$ (10,034)	(12)%

Collaboration revenue

There was no collaboration revenue for the year ended December 31, 2023, compared to $8.1 million of collaboration revenue for the year ended December 31, 2022. The decrease in collaboration revenue of $8.1 million was related to the completion of research and development services contemplated under the Collaboration Agreement during the second quarter of fiscal year 2022, resulting in the recognition of the remaining deferred revenue, which was recorded at the time the Collaboration Agreement was executed. No other research and development has commenced under the Collaboration Agreement.

Research and development expenses

(in thousands)	For the Year Ended December 31, 2023	2022	Increase (decrease)	% Change
SGT-001	$ 3,432	$ 24,844	$ (21,412)	(86)%
SGT-003	20,856	9,995	10,861	109%
SGT-501	3,200	-	3,200	100%
Other development programs	7,439	3,450	3,989	116%
Unallocated research and development expenses				
Personnel related expenses	24,968	24,883	85	0%
External expenses	16,668	15,248	1,420	9%
Total unallocated research and development expenses	41,636	40,131	1,505	4%
Total research and development expenses	$ 76,563	$ 78,420	$ (1,857)	(2)%

Research and development expenses for the year ended December 31, 2023 were $76.6 million, compared to $78.4 million for the year ended December 31, 2022. The decrease of $1.8 million in research and development expenses was primarily related to a $21.4 million decrease in costs for SGT-001 due to our decision to prioritize development of SGT-003, offset by an $11.0 million increase in manufacturing, clinical, and study related costs for SGT-003, a $4.0 million increase in costs for other development programs, a $3.2 million increase in costs for SGT-501, and a $1.4 million increase in external expenses.

General and administrative expenses

General and administrative expenses were $27.8 million for the year ended December 31, 2023, compared to $28.9 million for the year ended December 31, 2022. The decrease of $1.1 million was primarily related to a $1.6 million decrease in legal fees due to acquisition related costs occurring in 2022, a $1.0 million decrease in insurance premiums, a $0.4 million decrease in business development research, and a $0.1 million decrease in charitable contributions, offset by a $1.0 million increase in personnel related costs, a $0.4 million increase in IT related costs, a $0.3 million increase in recruiting costs, and a $0.3 million increase in facilities costs.

Restructuring charges

During the year ended December 31, 2023, restructuring charges were $(0.1) million compared to $7.2 million for the year ended December 31, 2022. The restructuring charges were related to severance and other employee-related costs in connection with the restructurings that occurred in April 2022 and December 2022. We paid $3.7 million during the year ended December 31, 2023 and $3.3 million during the year ended December 31, 2022.

Other income (expense)

Other income (expense) was $8.2 million and $20.5 million for the years ended December 31, 2023 and 2022, respectively. The decrease in other income was primarily related to the gain recorded in connection with the Acquisition of $18.2 million during the year ended December 31, 2022, partially offset by a $4.5 million increase in interest income primarily related to available-for-sale securities included within our portfolio, and a $1.5 million increase in other income primarily related to our sublease of space at our Massachusetts facility.

Results of operations—Years ended December 31, 2022 and 2021

Discussion and analysis of the year ended December 31, 2022 compared to the year ended December 31, 2021 is included in Part II, Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our Annual Report on Form 10-K for the year ended December 31, 2022 as filed with the SEC on March 23, 2023 (the “2022 Form 10-K”).

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 common stock and prefunded warrants to purchase shares of our common stock in private placements and the sale of common stock in our initial public offering and a follow-on public offering, and sales of common stock under our "at-the-market offering" sales agreement, dated March 13, 2019 and as amended on August 16, 2021, by and between us and Jefferies LLC, or Jefferies, or the ATM Sales Agreement. Through December 31, 2023, 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 $546.8 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 agreement with Ultragenyx, as detailed in the following paragraphs.

On March 13, 2019, we entered into the ATM Sales Agreement, which was amended in August 2021, under which we may offer and sell, from time to time, shares of our common stock having aggregate gross proceeds of up to $75.0 million 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 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, 2023 and 2022, we sold 602,030 and 0 shares of common stock, respectively, pursuant to the ATM Sales Agreement resulting in net proceeds of $3.0 million and $0, respectively. During the three months ended December 31, 2023, we sold 182,030 shares pursuant to the ATM Sales Agreement.

On March 23, 2021, we issued and sold in a public offering 1,666,666 shares of our common stock at a price per share of $86.25, including the full exercise by the underwriters of an option to purchase additional shares of common stock. We received net proceeds of approximately $134.9 million after deducting underwriting discounts and commissions and offering expenses.

On December 2, 2022, we issued and sold 10,638,290 shares of our common stock at a price per share of $7.05 in a private placement, or the December 2022 Private Placement, which closed immediately following the Acquisition. We received $72.6 million of net proceeds from the December 2022 Private Placement after deducting placement agent fees.

As of December 31, 2023, we had cash, cash equivalents and available-for-sale securities of $123.6 million, excluding restricted cash of $1.8 million, and had no debt outstanding.

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 the January 2024 Private Placement. We received approximately $104.0 million of net proceeds from the January 2024 Private Placement after deducting offering costs.

Cash flows

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

	For the Year Ended December 31,	
	2023	2022
(in thousands)		
Net cash used in operating activities	$ (94,180)	$ (97,977)
Net cash provided by investing activities	9,689	59,157
Net cash provided by financing activities	3,122	74,831
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (81,369)	$ 36,011

Operating activities

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 $8.2 million due primarily to equity-based compensation of $7.6 million and depreciation and impairment expense of $3.0 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 $6.3 million which included a decrease of $9.0 million in accrued other current and non-current liabilities, and a decrease in accounts payable of $0.7 million, partially offset by a decrease in prepaid and other non-current assets of $3.4 million.

During the year ended December 31, 2022, operating activities used $98.0 million of cash, primarily resulting from our net loss of $86.0 million and non-cash charges of $8.3 million due primarily to the gain on acquisition of $18.2 million, offset by equity-based compensation of $7.5 million and depreciation expense of $2.4 million. Net cash used by changes in our operating assets and liabilities was $3.7 million which included a decrease in deferred revenue of $8.1 million, a decrease in accounts receivable of $0.1 million as a result of the Ultragenyx Collaboration Agreement, and a decrease in accounts payable of $5.2 million due to the timing of payments, partially offset by an increase in prepaid and other non-current assets of $3.7 million and a decrease in accrued other liabilities and non-current liabilities of $5.8 million.

Investing activities

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.

During the year ended December 31, 2022, investing activities provided cash of $59.2 million, consisting primarily of the sale of available-for-sale securities of $212.8 million, net cash received upon the closing of the Acquisition of $31.5 million, and cash proceeds from the sale of property and equipment of $0.6 million, partially offset by net purchases of available-for-sale securities of $182.8 million and the purchase of property and equipment of $3.0 million primarily related to our corporate headquarters lease.

Financing activities

During the year ended December 31, 2023, net cash provided by financing activities was $3.1 million as a result of proceeds from the issuance of common stock of $3.0 million and from the issuance of shares of $0.1 million under the Company's employee stock purchase plan.

During the year ended December 31, 2022, net cash provided by financing activities was $74.8 million as a result of the December 2022 Private Placement.

A discussion of changes in our cash flow from the year ended December 31, 2021 to the year ended December 31, 2022 can be found in Part II, Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” of the 2022 Form 10-K.

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:

- enroll patients in our INSPIRE Duchenne trial and advance clinical development of SGT-003;
- advance our other Candidates into clinical trials;
- 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;
- 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;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to the Collaboration Agreement; and
- add operational, financial and management information systems and personnel.

As of December 31, 2023, we had cash, cash equivalents and available-for-sale securities of $123.6 million, excluding restricted cash of $1.8 million. Based on our current operating plan, we believe that our cash, cash equivalents and available-for-sale securities as of December 31, 2023, together with the net proceeds from our January 2024 Private Placement, will be sufficient to fund our operating expenses and capital requirements into 2026. 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.

Because of 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 product 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 product candidates. Our future capital requirements will depend on many factors, including:

- the progress, costs and results of the INSPIRE Duchenne trial 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;
- the success of our collaboration with Ultragenyx;
- 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, such as the recent COVID-19 pandemic, and collateral consequences related thereto.

We are supplying, and expect to continue to supply, our ongoing and future 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 product candidates or generate revenue from the sale of any products for which we may obtain marketing approval. In addition, our product 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 product 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 product candidates or any future commercialization efforts or grant rights to develop and market product 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, North Carolina and Florida. These leases are used for our continuing operations. For a description of our lease obligations, refer to Note 11 to our consolidated financial statements appearing at the end of 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 "Business—Strategic partnerships and collaborations/licenses" and see Note 13 of our consolidated financial statements appearing at the end of 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, 2023, our cash equivalents consisted of money market accounts that have contractual maturities of less than 90 days from the date of acquisition. As of December 31, 2023, our investments consisted of treasury bills 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 three 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 officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2023. 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 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, 2023, our disclosure controls and procedures were effective at the reasonable assurance level.

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, 2023 based on the framework in *Internal Control-Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Based on that evaluation, management has concluded that the Company's internal control over financial reporting was effective as of December 31, 2023.

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 the 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, 2023 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 2023.

Item 9C. Disclosure Regarding Foreign Jurisdictions that Prevent Inspections.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Except to the extent provided below, the information required under this Item 10 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

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 Investor Relations portion of our website, www.solidbio.com. The nominating and corporate governance committee of our Board of Directors is responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. In addition, 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.

Item 11. Executive Compensation.

The information required under this Item 11 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters.

The information required under this Item 12 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

Item 13. Certain Relationships and Related Transactions, and Director Independence.

The information required under this Item 13 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

Item 14. Principal Accountant Fees and Services.

The information required under this Item 14 is incorporated by reference to our definitive proxy statement for our 2024 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission not later than 120 days after the end of the fiscal year ended December 31, 2023.

PART IV

Item 15. Exhibits and Financial Statement Schedules.

(1) Financial Statements:

(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 by reference to Exhibit 4.1 to the Registration Statement on Form S-8 filed on December 2, 2022).
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.
10.8*#	License Agreement, dated as of October 15, 2015, by and between Solid GT, LLC and The Curators of the University of Missouri.
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 (incorporated by reference to Exhibit 10.12 to the Annual Report on Form 10-K filed on March 23, 2023).
10.11	Securities Purchase Agreement, dated July 25, 2019, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on July 26, 2019).
10.12	Form of Pre-Funded Warrant to Purchase Common Stock to be issued pursuant to the Securities Purchase Agreement (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on July 26, 2019).
10.13	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).
10.14†	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).

10.15*†	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, and Second Amendment to the Executive Chair Agreement, effective January 1, 2024
10.16†	Amended and Restated 2020 Equity Incentive Plan (incorporated by reference to Exhibit 99.1 to the Current Report on Form 8-K filed on December 1, 2022).
10.17†	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).
10.18†	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).
10.19#	Collaboration and License Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
10.20	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).
10.21#	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).
10.22#	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).
10.23	Securities Purchase Agreement, dated December 10, 2020, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on December 11, 2020).
10.24	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).
10.25#	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).
10.26	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).
10.27†*	Amended and Restated 2021 Employee Stock Purchase Plan.
10.28†	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).
10.29†	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).
10.30*	Amended and Restated Sales Agreement, dated March 13, 2024, by and between the Company and Jefferies LLC.
10.31	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).
10.32	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).
10.33	Securities Purchase Agreement, dated September 29, 2022, by and among Solid Biosciences Inc. and the persons party thereto (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed on September 30, 2022).
10.34	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).

10.35†	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).
10.36†	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).
10.37†	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).
10.38†	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).
10.39†	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).
10.40	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).
10.41	Executive Transition and Separation Agreement, dated as of May 22, 2023, between Solid Biosciences Inc. and Carl Morris (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on August 14, 2023).
10.42	Consulting Agreement dated as of July 14, 2023, between Solid Biosciences Inc. and PHDL Consulting LLC (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on August 14, 2023)
10.43	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).
10.44#	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).
10.45#	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).
10.46	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).
10.47	Form of Securities Purchase Agreement, dated January 8, 2024, by and among Solid Biosciences Inc. and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on January 8, 2024).
10.48	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).
10.49*†	2024 Inducement Stock Incentive Plan.
10.50*†	Form of Non-Statutory Stock Option Agreement under the 2024 Inducement Stock Incentive Plan.
10.51*†	Form of Restricted Stock Unit Agreement under the 2024 Inducement Stock Incentive Plan.
10.52*†	Form of Restricted Stock Unit Agreement under the 2020 Equity Incentive Plan.
10.53*##	Patent License Agreement, dated as of March 10, 2016, by and between Solid GT, LLC and the Regents of the University of Michigan.
10.54*##	Life Technologies Cell Line License Agreement, dated as of November 20, 2016, by and between Solid Biosciences, LLC and Life Technologies Corporation.
10.55*##	License Agreement, dated as of June 23, 2016, by and between Solid GT, LLC and President and Fellows of Harvard College.
10.56*##	License Agreement, dated as of August 3, 2017, by and between Solid Biosciences, LLC and President and Fellows of Harvard College.
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
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 13, 2024

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 13, 2024
/s/ Kevin Tan Kevin Tan	Chief Financial Officer *(Principal Financial and Accounting Officer)*	March 13, 2024
/s/ Ian F. Smith Ian F. Smith	Chairman of the Board	March 13, 2024
/s/ Martin Freed Martin Freed	Director	March 13, 2024
/s/ Ilan Ganot Ilan Ganot	Director	March 13, 2024
/s/ Clare Kahn Clare Kahn	Director	March 13, 2024
/s/ Georgia Keresty Georgia Keresty	Director	March 13, 2024
/s/ Adam Koppel Adam Koppel	Director	March 13, 2024
/s/ Sukumar Nagendran Sukumar Nagendran	Director	March 13, 2024
/s/ Rajeev Shah Rajeev Shah	Director	March 13, 2024
/s/ Adam Stone Adam Stone	Director	March 13, 2024
/s/ Lynne Sullivan Lynne Sullivan	Director	March 13, 2024

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, 2023 and 2022, and the related consolidated statements of operations, of comprehensive loss, of stockholders' equity and of cash flows for each of the three years in the period ended December 31, 2023, 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, 2023 and 2022, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2023 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 audit 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 (i) relates to accounts or disclosures that are material to the consolidated financial statements and (ii) involved our especially challenging, subjective, or complex judgments. The 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 cancelable, 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, 2023 was $76.6 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 13, 2024

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,	
	2023	**2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 74,015	$ 155,384
Available-for-sale securities	49,625	58,338
Prepaid expenses and other current assets	6,094	5,916
Total current assets	129,734	219,638
Operating lease, right-of-use asset	26,539	28,949
Property and equipment, net	6,624	9,657
Other non-current assets	209	175
Restricted cash	1,833	1,833
Total assets	$ 164,939	$ 260,252
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 2,032	$ 3,238
Accrued expenses	10,137	16,691
Operating lease liabilities	1,855	1,897
Finance lease liabilities	469	668
Other current liabilities	24	14
Total current liabilities	14,517	22,508
Operating lease liabilities, excluding current portion	22,707	24,279
Finance lease obligations, excluding current portion	1,234	1,703
Other non-current liabilities	-	96
Total liabilities	38,458	48,586
Commitments and Contingencies (Note 12)		
Stockholders' Equity:		
Preferred stock, $0.001 par value; 10,000,000 shares authorized at December 31, 2023 and December 31, 2022; no shares issued and outstanding at December 31, 2023 and December 31, 2022	—	—
Common stock, $0.001 par value; 60,000,000 shares authorized at December 31, 2023 and December 31, 2022; 20,386,606 shares issued and outstanding at December 31, 2023 and 19,556,732 shares issued and outstanding at December 31, 2022	20	20
Additional paid-in capital	785,199	774,452
Accumulated other comprehensive income (loss)	15	(68)
Accumulated deficit	(658,753)	(562,738)
Total stockholders' equity	126,481	211,666
Total liabilities and stockholders' equity	$ 164,939	$ 260,252

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,		
	2023	**2022**	**2021**
Collaboration revenue - related party	$ —	$ 8,094	$ 13,620
Operating expenses:			
Research and development	76,563	78,420	58,739
General and administrative	27,752	28,948	27,135
Restructuring expense	(63)	7,178	—
Total operating expenses	104,252	114,546	85,874
Loss from operations	(104,252)	(106,452)	(72,254)
Other income, net:			
Interest income, net	7,142	2,616	64
Gain on acquisition	—	18,236	—
Other income (expense)	1,095	(381)	2
Total other income, net	8,237	20,471	66
Net loss	$ (96,015)	$ (85,981)	$ (72,188)
Net loss per share, basic and diluted	$ (4.83)	$ (10.10)	$ (10.14)
Weighted average common stock outstanding, basic and diluted	19,884,007	8,512,089	7,118,024

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,		
	2023	**2022**	**2021**
Net loss	$ (96,015)	$ (85,981)	$ (72,188)
Other comprehensive income (loss):			
Unrealized gain (loss) on available-for-sale securities	83	(23)	(45)
Comprehensive loss	$ (95,932)	$ (86,004)	$ (72,233)

The accompanying notes are an integral part of these consolidated financial statements.

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS STOCKHOLDERS' EQUITY
(In thousands except for share data)

	Common Stock	Amount	Additional paid in capital	Accumulated other comprehensive income (loss)	Accumulated Deficit	Total Equity
Balance at December 31, 2020	5,803,487	$ 6	$ 536,649	$ —	$ (404,569)	$ 132,086
Equity-based compensation	—	—	13,373	—	—	13,373
Sale of common stock, net of issuance costs of $8,872	1,666,666	1	134,877	—	—	134,878
Exercise of common stock options	775	—	41	—	—	41
Issuance of common stock in connection with employee stock purchase plan	2,978	—	66	—	—	66
Vesting of restricted stock units	27,946	—	—	—	—	—
Forfeiture of restricted stock awards	(1,947)	—	—	—	—	—
Unrealized gain on available-for-sale securities	—	—	—	(45)	—	(45)
Net loss	—	—	—	—	(72,188)	(72,188)
Balance at December 31, 2021	7,499,905	7	685,006	(45)	(476,757)	208,211
Equity-based compensation	—	—	7,537	—	—	7,537
Sale of common stock, net of issuance costs of $2,449	10,638,290	11	72,540	—	—	72,551
Exercise of pre-funded warrants	—	—	22	—	—	22
Issuance of common stock in connection with employee stock purchase plan	29,130	1	180	—	—	181
Vesting of restricted stock units	35,149	—	—	—	—	—
Issuance of shares in connection with acquisition	1,354,258	1	9,167	—	—	9,168
Unrealized loss on available-for-sale securities	-	—	—	(23)	—	(23)
Net loss	-	—	—	—	(85,981)	(85,981)
Balance at December 31, 2022	19,556,732	20	774,452	(68)	(562,738)	211,666
Equity-based compensation	—	—	7,625	—	—	7,625
Vesting of restricted stock units	183,951	—	—	—	—	—
Sale of common stock net of issuance costs of $76	602,030	—	2,974	—	—	2,974
Issuance of common stock in connection with employee stock purchase plan	43,893	—	148	—	—	148
Unrealized gain on available-for-sale securities	—	—	—	83	—	83
Net loss	—	—	—	—	(96,015)	(96,015)
Balance at December 31, 2023	20,386,606	$ 20	$ 785,199	$ 15	$ (658,753)	$ 126,481

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,		
	2023	**2022**	**2021**
Operating activities:			
Net loss	$ (96,015)	$ (85,981)	$ (72,188)
Adjustments to reconcile net loss to net cash used in operating activities:			
Net amortization of (discount)/premium on available-for-sale securities	(2,408)	233	1,117
Equity-based compensation expense	7,625	7,537	13,373
Depreciation and amortization expense	2,581	2,408	2,964
Loss on sale of property and equipment	374	—	92
Gain on lease termination	—	(249)	(81)
Gain on acquisition	—	(18,236)	—
Changes in operating assets and liabilities:			
Prepaid expenses and other current and non-current assets	3,436	3,695	(9,247)
Accounts receivable - related party	—	110	(110)
Accounts payable	(764)	(5,246)	1,209
Accrued expenses and other current and non-current liabilities	(9,009)	5,832	(2,255)
Deferred revenue - related party, current and non-current	-	(8,080)	(12,638)
Net cash used in operating activities	(94,180)	(97,977)	(77,764)
Investing activities:			
Purchases of property and equipment	(1,515)	(3,015)	(1,281)
Acquisition of business, net of cash received	—	31,523	—
Proceeds from sale of property and equipment	—	600	—
Proceeds from sales and maturities of available-for-sale securities	128,632	212,811	51,444
Purchases of available-for-sale securities	(117,428)	(182,762)	(141,249)
Net cash provided by (used in) investing activities	9,689	59,157	(91,086)
Financing activities:			
Proceeds from issuance of common stock, net of issuance costs	2,974	72,551	134,878
Proceeds from financing liabilities	—	2,143	—
Proceeds from exercise of stock options	—	—	41
Proceeds from exercise of pre-funded warrants	—	22	—
Employee stock purchases and withholdings	148	181	66
Repayment of financing liabilities	—	(66)	—
Net cash provided by financing activities	3,122	74,831	134,985
Net (decrease) increase in cash, cash equivalents and restricted cash	(81,369)	36,011	(33,865)
Cash, cash equivalents, and restricted cash at beginning of period	157,217	121,206	155,071
Cash, cash equivalents, and restricted cash at end of period	$ 75,848	$ 157,217	$ 121,206
Supplemental disclosure of non-cash investing and financing activities:			
Issuance of common stock in acquisition	$ —	$ 9,168	$ —
Right-of-use assets obtained in exchange for operating lease liabilities	$ 410	$ 29,126	$ —
Decrease in right-of-use asset and lease liability due to lease termination	$ (252)	$ (464)	$ (1,233)
Decrease in property, plant and equipment due to asset exchange	$ (950)	$ —	$ —
Property and equipment included in accounts payable and accruals	$ 76	$ 527	$ 104

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" or "Solid"). 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-501 for the treatment of Catecholaminergic polymorphic ventricular tachycardia ("CPVT"), and additional assets for the treatment of 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, Solid'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. Product 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.

On October 27, 2022, the Company effected a reverse stock split of its outstanding shares of common stock at a ratio of one-for-15 pursuant to a certificate of amendment to its certificate of incorporation filed with the Secretary of State of the State of Delaware. The reverse stock split was reflected on the Nasdaq Stock Market ("Nasdaq") beginning with the opening of trading on October 28, 2022. Pursuant to the reverse stock split, every 15 shares of the Company's issued and outstanding shares of common stock were automatically combined into one issued and outstanding share of common stock, without any change in the par value per share of the common stock. The reverse stock split reduced the authorized number of shares of common stock from 300,000,000 to 20,000,000 and, pursuant to the certificate of amendment, such reduced authorized number of shares of common stock was subsequently multiplied by three, such that following the reverse stock split the Company has 60,000,000 shares of common stock authorized. The reverse stock split affected all issued and outstanding shares of the Company's common stock, and the respective numbers of shares of common stock underlying the Company's outstanding stock options, outstanding restricted stock units, outstanding warrants and the Company's equity incentive plans were proportionately adjusted. All share and per share amounts of the common stock included in the accompanying consolidated financial statements have been retrospectively adjusted to give effect to the reverse stock split for all periods presented, including reclassifying an amount equal to the reduction in par value to additional paid-in capital.

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, 2023, 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 prefunded 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 September 29, 2022, the Company entered into the securities purchase agreement, pursuant to which, on December 2, 2022, the Company issued an aggregate of 10,638,290 shares of the Company's common stock in a private placement. The private placement closed immediately following the closing of the Acquisition on December 2, 2022. The Company received net proceeds from the private placement of $72,551.

During the year ended December 31, 2023, the Company issued 602,030 shares of its common stock, pursuant to the Company's "at-the-market offering" sales agreement, dated March 13, 2019, as amended on August 16, 2021, between the Company and Jefferies LLC (the "ATM Sales Agreement"). During the year ended December 31, 2023, the Company received net proceeds of $2,974 from the sale of shares pursuant to the ATM 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 approximately $104,034 of net proceeds from the January 2024 Private Placement after deducting 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, 2023, the Company had an accumulated deficit of $658,753. During the years ended December 31, 2023, 2022 and 2021, the Company incurred a net loss of $96,015, $85,981 and $72,188, respectively. The Company used $94,180 of cash in operations for the year ended December 31, 2023. 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 $123,640, excluding restricted cash of $1,833, as of December 31, 2023, together with the net proceeds from the January 2024 Private Placement, 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.

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 revenues and expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, estimates related to revenue recognition, the recognition of research and development expenses and equity-based compensation. 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.

Restricted Cash

The Company held restricted cash of $1,833 in a restricted bank account as a security deposit for lease of the Company's facilities as of December 31, 2023 and December 31, 2022. The Company has included restricted cash of $1,833 as a non-current asset as of December 31, 2023 and December 31, 2022. A reconciliation of the amounts of cash and cash equivalents and restricted cash from the cash flow statement to the balance sheet is as follows:

	December 31, 2023	December 31, 2022	December 31, 2021
Cash and cash equivalents	$ 74,015	$ 155,384	$ 119,136
Restricted cash, non-current	1,833	1,833	2,070
Cash and cash equivalents and restricted cash	$ 75,848	$ 157,217	$ 121,206

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 (expense) within the consolidated statement of operations. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the consolidated statement of operations. 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. See Note 5, *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 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 cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from 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.

Revenue Recognition

The Company recognizes revenue in accordance with ASC Topic 606, Revenue from Contracts with Customers ("ASC 606"). Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, the Company performs the following five steps: (i) identification of the contract(s) with the customer; (ii) identification of the promised goods or services in the contract and determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

As part of the accounting for these arrangements, the Company must use significant judgment to determine: a) the number of performance obligations based on the determination under step (ii) above and whether those performance obligations are distinct from other performance obligations in the contract; b) the transaction price under step (iii) above; and c) the standalone selling price for each performance obligation identified in the contract for the allocation of transaction price in step (iv) above. The Company uses judgment to determine whether milestones or other variable consideration, except for royalties, should be included in the transaction price as described further below. The transaction price is allocated to each performance obligation on a relative stand-alone selling price basis, for which the Company recognizes revenue as or when the performance obligations under the contract are satisfied. In determining the stand-alone selling price of a license to the Company's proprietary technology or a material right provided by a customer option, the Company considers market conditions as well as entity-specific factors, including those factors contemplated in negotiating the agreements as well as internally developed estimates that include assumptions related to the market opportunity, estimated development costs, probability of success and the time needed to commercialize a product candidate pursuant to the license. In validating its estimated stand-alone selling prices, the Company evaluates whether changes in the key assumptions used to determine its estimated stand-alone selling prices will have a significant effect on the allocation of arrangement consideration between performance obligations.

The Company estimates the transaction price based on the amount of consideration the Company expects to be received for transferring the promised goods or services in the contract. The consideration may include both fixed consideration and variable consideration. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of the potential payments and the likelihood that the payments will be received. The Company utilizes either the most likely amount method or expected value method to estimate the transaction price based on which method better predicts the amount of consideration expected to be received. If it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur, the variable consideration is included in the transaction price.

Performance obligations are promised goods or services in a contract to transfer a distinct good or service to the customer. Promised goods or services are considered distinct when: (i) the customer can benefit from the good or service on its own or together with other readily available resources and (ii) the promised good or service is separately identifiable from other promises in the contract. In assessing whether promised goods or services are distinct, the Company considers factors such as the stage of development of the underlying intellectual property, the capabilities of the customer to develop the intellectual property on their own and whether the required expertise is readily available.

For performance obligations which consist of licenses and other promises, the Company utilizes judgment to assess the nature of the combined performance obligation in order to determine whether the combined performance obligation is satisfied over time or at a point in time. Up-front payments and fees are recorded as deferred revenue upon receipt or when due until the Company performs its obligations under these arrangements. Amounts expected to be recognized as revenue within the 12 months following the balance sheet date are classified as current portion of deferred revenue in the accompanying consolidated balance sheets. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion. Amounts are recorded as accounts receivable when the Company's right to consideration is unconditional. Amounts recognized as revenue, but not yet received or invoiced are generally recognized as contract assets.

Exclusive Licenses

If the license granted in the 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. 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. 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. For those elements of the arrangement that are accounted for pursuant to ASC 606, the Company applies the five-step model described above.

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 up-front 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 Company has not issued any awards with performance-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. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions and options granted to non-employees, the expected term of the Company's stock options 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. Expected dividend yield is 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.

The fair value for restricted stock units ("RSU") was calculated using the closing price of the Company's common stock on the date of grant.

The Company classifies stock-based compensation expense in its consolidated statement 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.

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.

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 (loss) in all periods presented was unrealized gains (losses) 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.

The Company's preferred stock 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 the years ended December 31, 2023, 2022, and 2021, 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.

Recently Issued Accounting Pronouncements

In December 2023, the Financial Accounting Standards Board 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.

3. Collaborations

Ultragenyx Collaboration

Collaboration Agreement

On October 22, 2020, the Company entered into the Collaboration Agreement with Ultragenyx Pharmaceutical Inc. ("Ultragenyx ") to focus on the development and commercialization of new gene therapies for Duchenne. The Company granted Ultragenyx an exclusive worldwide license for any pharmaceutical product that expresses the Company's proprietary microdystrophin construct from AAV8 and variants thereof in clade E for the treatment of Duchenne and other diseases resulting from the lack of functional dystrophin (the "Licensed Products"). The Company retains exclusive rights to all other uses of its microdystrophin proteins, including under its SGT-003 program.

The Company has conducted certain research and development activities with respect to the development of the Licensed Products, and concluded such activities as were contemplated under the Collaboration Agreement during the second quarter of 2022, resulting in the recognition of the remaining deferred revenue recorded at the time the Collaboration Agreement was executed, related to the upfront payment received from Ultragenyx. The Company may conduct additional research and development activities in collaboration with Ultragenyx from time to time in the future. Ultragenyx reimbursed the Company for personnel and out-of-pocket costs that the Company incurred in conducting such activities.

In addition, Ultragenyx granted to the Company an exclusive Development Option or Income Share Option (each as defined and described below) exercisable in the Company's sole discretion one time per Licensed Product. After the date of first achievement of clinical proof of concept, Ultragenyx will provide to the Company a data package with respect to the relevant

Licensed Product. The Company will use the data package to determine whether to exercise the corresponding Development Option or Income Share Option with respect to such Licensed Product.

With respect to each Licensed Product for which the Company has not exercised the Development Option or Income Share Option the Company will be entitled to milestone payments of up to $25,000 in the aggregate for each such Licensed Product that achieves specified development milestones and $65,000 in the aggregate for each such Licensed Product that achieves specified regulatory milestones. With respect to each Licensed Product for which the Company has not exercised the Income Share Option, the Company will also be entitled to milestone payments of up to $165,000 in the aggregate for each Licensed Product that achieves specified annual worldwide net sales milestones. For Licensed Products for which the Company has not exercised the Development Option or Income Share Option, Ultragenyx will pay the Company tiered royalties on a Licensed Product-by-Licensed Product and country-by-country basis ranging from a low double-digit percentage to a mid-teens percentage based on Ultragenyx's annual worldwide net sales of such Licensed Products.

For each Licensed Product for which Ultragenyx decides to initiate a registrational trial in humans, the Company will have the option to fund 30% of the development costs in the United States and European Union for such Licensed Product and forgo the development and regulatory milestones (the "Development Option") and receive tiered royalties on a Licensed Product-by-Licensed Product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's annual worldwide net sales of each such Licensed Product.

For each Licensed Product for which the Company exercises the Development Option, the Company may also elect to share 30% of the net income and net losses on net sales of such Licensed Product in the United States and European Union (the "Income Share Option"). For Licensed Products for which the Company has exercised the Income Share Option, the Company will not be entitled to milestone payments and Ultragenyx will pay the Company tiered royalties on a Licensed Product-by-Licensed Product and country-by-country basis ranging from a mid-teens percentage to a low twenties percentage based on Ultragenyx's annual net sales of each such Licensed Product outside of the United States and European Union.

The Company may only exercise an Income Share Option if neither the Company nor any of its affiliates is then developing or commercializing a product that is competitive with the Licensed Product that is subject to such option. If the Company or any of its affiliates subsequently develops or commercializes a product that is competitive with a Licensed Product for which the Company has exercised an Income Share Option, then the Company and Ultragenyx will no longer share the net income and net losses on net sales of such Licensed Product and such Licensed Product will be treated as if the Company had exercised the Development Option with respect to such Licensed Product.

Following the Company's exercise of the Development Option or Income Share Option with respect to a Licensed Product, the Company also has the right to cease participation in the sharing of development costs and sharing in net income and net losses on net sales, as applicable, for such Licensed Product by written notice to Ultragenyx. Upon such notice, the Company will no longer share in the development costs and net income and net losses on net sales of such Licensed Product, as applicable, and will be eligible to receive payments on milestones achieved after the opt-out for such Licensed Product and royalties at the rates applicable to Licensed Products for which the Company has not exercised the Development Option or Income Share Option, as described above.

The Collaboration Agreement continues on a country-by-country and Licensed Product-by-Licensed Product basis until the expiration of all payment obligations under the agreement. With respect to any Licensed Product for which the Company has exercised an Income Share Option, the Collaboration Agreement continues until there are no longer sales of such Licensed Product in the United States or Europe. Either party has the right to terminate the agreement if the other party has materially breached in the performance of its obligations under the agreement and such breach has not been cured within the applicable cure period. Ultragenyx may also terminate the Collaboration Agreement in its sole discretion upon 90 days' prior written notice to the Company.

Stock Purchase Agreement

In connection with the execution of the Collaboration Agreement, Ultragenyx and the Company also entered into a stock purchase agreement (the "Stock Purchase Agreement") on the Effective Date, pursuant to which the Company issued and sold 521,719 shares of its common stock (the "Shares") to Ultragenyx at a price of $76.6695 per share for an aggregate purchase price of approximately $40,000. The Stock Purchase Agreement contains customary representations, warranties and covenants of each of the parties thereto. Following the sale of the Shares, Ultragenyx beneficially owned approximately 14.45% of the Company's outstanding common stock. As of December 31, 2023, Ultragenyx beneficially owned approximately 2.6% of the Company's outstanding common stock.

Investor Agreement

In connection with the consummation of the transactions contemplated by the Stock Purchase Agreement, the Company and Ultragenyx entered into an Investor Agreement (the "Investor Agreement") on the Effective Date. Pursuant to the terms of

the Investor Agreement, Ultragenyx agreed that, so long as it holds at least 10% of the Company's outstanding common stock, the Shares will be subject to a voting agreement, such that until the earliest to occur of certain specified events, and subject to specified conditions, Ultragenyx will, and will cause its permitted transferees to, vote in accordance with the recommendation of the Company's Board of Directors with respect to specified matters.

Accounting Treatment

The Company concluded that the Collaboration Agreement and the Stock Purchase Agreement should be combined and treated as a single arrangement for accounting purposes as the agreements were entered into contemporaneously and in contemplation of one another.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Ultragenyx, is a customer. The Company identified the following promises in the Collaboration Agreement that were evaluated under the scope of ASC 606: (1) an exclusive worldwide license to the Licensed Products; (2) an obligation to perform research and development services; and (3) an obligation to participate in a joint steering committee. The Company assessed the promised goods and services to determine if they are distinct. Based on this assessment, the Company determined that Ultragenyx cannot benefit from the promised goods and services separately from the others as they are highly interrelated and therefore not distinct. Due to the early stage of the Licensed Products, the research and development services could not be performed by another party. The Company's skill-set, knowledge and expertise are required to conduct the research and development services and the research and development services are expected to involve significant further development of the Licensed Products. Accordingly, the promised goods and services represent one combined performance obligation and the entire transaction price will be allocated to that single combined performance obligation.

The Company determined the transaction price under ASC 606 at the inception of the Collaboration Agreement to be $22,513, which represents the excess proceeds from the equity investment under the Stock Purchase Agreement, when measured at fair value after taking into consideration a discount for lack of marketability, plus the estimated reimbursement of research and development costs, which represents variable consideration. The Company included the estimated reimbursement of research and development costs in the transaction price at the inception of the arrangement because the Company is required to perform research and development services and the contract requires Ultragenyx to reimburse the Company for costs incurred. Also, since the related revenue would be recognized only as the costs are incurred, the Company determined it is not probable that a significant reversal of cumulative revenue would occur. The Company evaluated how much variable consideration related to development and regulatory milestones, and the Company's potential exercise of its Development Option or Income Share Option per Licensed Product, to include in the transaction price using the most likely amount approach and concluded that no amount should be included in the transaction price due to the high degree of uncertainty and risk associated with these potential payments. The Company also determined that royalties and sales milestones relate solely to the license of intellectual property and are therefore excluded from the transaction price under the sales- or usage-based royalty exception of ASC 606. Revenue related to these royalties and sales milestones will only be recognized when the associated sales occur, and relevant thresholds are met.

The Company determined that revenue under the Collaboration Agreement should be recognized over time as Ultragenyx simultaneously receives the benefit from the Company as the Company performs under the single performance obligation over time. The Company will recognize revenue for the single performance obligation using a cost-to-cost input method as the Company has concluded it best depicts the research and development and joint steering committee participation services performed. Under this method, the transaction price is recognized over the contract's entire performance period, using costs incurred relative to total estimated costs to determine the extent of progress towards completion.

Ultragenyx was a related party because Ultragenyx was one of the Company's significant stockholders as of December 31, 2021 and continued to be a stockholder as of December 31, 2022. However, Ultragenyx is not a significant stockholder as of December 31, 2023. The Company did not recognize any related party collaboration revenue associated with its collaboration with Ultragenyx during the year ended December 31, 2023. The Company recognized $8,094 and $13,620 related party collaboration revenue during the year ended December 31, 2022 and 2021, respectively. Further, the Company has made no payments to Ultragenyx during the years ended December 31, 2023 and 2022. There are no amounts due from Ultragenyx as of December 31, 2023 and December 31, 2022. The amount received is deferred as a contract liability on the Company's consolidated balance sheet as the performance obligation has been fully satisfied as of December 31, 2022.

During the year ended December 31, 2023, the Company had no changes in related party collaboration receivables or contract liabilities. The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities during the year ended December 31, 2022:

	Balance as of December 31, 2021	Additions	Deductions	Balance as of December 31, 2022
Related party collaboration receivable	$ 110	$ 14	$ (124)	$ —
Contract liabilities:				
Deferred revenue	8,080	—	(8,080)	—

The changes in the related party collaboration receivables balance during the year ended December 31, 2022 are the result of amounts owed to the Company for research and development services provided, offset by the collections received from Ultragenyx.

There was no deferred revenue related to Collaboration Agreement during the years ended December 31, 2023 and 2022. Additionally, there was no related party collaboration receivables during the years ended December 31, 2023 and 2022.

Costs incurred relating to the Collaboration Agreement consist of internal and external research and development costs, which primarily include salaries and benefits, lab supplies, preclinical research studies, clinical studies, consulting services, and commercial development. These costs are included in research and development expenses in the Company's consolidated statement of operations during the years ended December 31, 2022 and 2021. There was no costs incurred during the year ended December 31, 2023.

4. Acquisition

On September 29, 2022, the Company entered into an Agreement and Plan of Merger with AavantiBio. The Acquisition closed on December 2, 2022 and was announced on December 5, 2022. This acquisition allows the Company to add to its pipeline of assets. The Company acquired AavantiBio for a total purchase price of $9,169, including (i) $1 in cash and (ii) 1,354,258 shares of its common stock, par value $0.001 per share with a fair value of $9,168 to AavantiBio equityholders. The price per share of the Company's common stock used in the calculation of the purchase price is based on the closing price of Solid's common stock on the Nasdaq Global Select Market on December 2, 2022, which was $6.77.

The Acquisition was accounted for as a business combination in which the Company, as the accounting acquirer, recorded the assets acquired and liabilities assumed from AavantiBio at their fair values as of the acquisition date. The Company recognized a gain on the purchase of AavantiBio of $18,236 as the net assets acquired of $27,405 were greater than the purchase price of $9,169. Prior to recognizing the gain, the Company reassessed the measurement and recognition of identifiable assets acquired, and liabilities assumed and concluded that the valuation procedures and resulting measures were appropriate, in all material respects. The Company believes that its ability to negotiate a purchase price lower than the fair market value of the acquired net assets was due to a combination of factors, including the then prevailing market conditions and the uncertain future macroeconomic environment. The Company believes the seller, as a smaller, less well capitalized company, was motivated to complete the transaction under the terms described above as growing economic uncertainty and a rising interest rate environment negatively impacted their ability to raise additional capital.

The Company incurred acquisition related costs of $4,870, which are included in general and administrative expenses in the Company's consolidated statements of comprehensive loss for the fiscal year ended December 31, 2022.

The fair value is determined utilizing the fair value hierarchy as described in *Note 2, Summary of Significant Accounting Policies,* and *Note 5, Fair Value of Financial Assets and Liabilities*.

5. 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 and indicate the level of the fair value hierarchy utilized to determine such fair values:

	Fair Value Measurements as of December 31, 2023:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ —	$ 62,398	$ —	$ 62,398
Available-for-sale securities	—	49,625	—	49,625
	$ —	$ 112,023	$ —	$ 112,023

	Fair Value Measurements as of December 31, 2022:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ —	$ 69,374	$ —	$ 69,374
Available-for-sale securities	—	58,338	—	58,338
	$ —	$ 127,712	$ —	$ 127,712

As of December 31, 2023 and December 31, 2022 the fair values of the Company's cash equivalents and available-for-sale securities were determined using Level 2 inputs. During the year ended December 31, 2023 and December 31, 2022, there were no transfers between Level 1, Level 2 and Level 3, respectively.

The fair value of the Company's cash, restricted cash, accounts payable, accrued expenses and other current liabilities approximate their carrying value due to their short-term maturities.

6. Available-for-Sale Securities

As of December 31, 2023, the fair value of available-for-sale debt securities by type of security was as follows:

	December 31, 2023			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
Treasury bills	$ 49,610	$ 15	$ —	$ 49,625
	$ 49,610	$ 15	$	$ 49,625

As of December 31, 2022, the fair value of available-for-sale debt securities by type of security was as follows:

	December 31, 2022			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
Treasury bills	$ 34,780	$ —	$ (30)	$ 34,750
Corporate bond securities	23,626	—	(38)	23,588
	$ 58,406	$	$ (68)	$ 58,338

The estimated fair value and amortized cost of the Company's available-for-sale securities by contractual maturity are summarized as follows:

	December 31, 2023	
	Amortized Cost	Fair Value
Due in one year or less	$ 49,610	$ 49,625
Total available-for-sale securities	$ 49,610	$ 49,625

	December 31, 2022	
	Amortized Cost	Fair Value
Due in one year or less	$ 58,406	$ 58,338
Total available-for-sale securities	$ 58,406	$ 58,338

The average maturity of the Company's available-for-sale securities as of December 31, 2023 and December 31, 2022 was approximately 0.4 years and 0.5 years, respectively.

7. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consisted of the following:

	December 31,	
	2023	2022
Prepaid research and development expenses	$ 3,980	$ 2,913
Prepaid expenses and other assets	2,114	3,003
	$ 6,094	$ 5,916

8. Property and Equipment

Property and equipment consisted of the following:

	December 31,	
	2023	2022
Furniture and fixtures	$ 936	$ 868
Laboratory equipment	16,137	16,416
Leasehold improvements	481	384
Computer equipment	842	677
Computer software	553	553
Construction in process	410	1,715
	19,359	20,613
Less accumulated depreciation	12,735	10,956
	$ 6,624	$ 9,657

Depreciation expense was $2,581, $2,408 and $2,964 for the years ended December 31, 2023, 2022 and 2021, respectively. The Company recognized an impairment loss of $374, $0, and $92 for the years ended December 31, 2023, 2022 and 2021, respectively.

9. Accrued Expenses

Accrued expenses and other current liabilities consisted of the following:

	December 31,	
	2023	2022
Accrued research and development	$ 2,614	$ 3,033
Accrued compensation	5,948	8,370
Accrued other	1,575	5,288
	$ 10,137	$ 16,691

10. Equity-Based Compensation

Equity Incentive Plans

In connection with the closing of the Company's initial public offering, the Company's Board of Directors and stockholders approved the 2018 Omnibus Incentive Plan (the "2018 Plan"), which provides for the reservation of 333,400 shares of common stock for equity awards. On June 16, 2020, the Company's stockholders approved the 2020 Equity Incentive Plan (as amended or restated, the "2020 Plan") which consisted of, at the time of approval, (i) 200,000 shares of common stock and (ii) additional shares of common stock (up to 325,268) as is equal to (i) the number of shares reserved under the 2018 Plan that remain available for grant under the 2018 Plan as of immediately prior to the date the 2020 Plan was approved by the Company's stockholders and (ii) the number of shares subject to awards granted under the 2018 Plan which awards expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right.

As of the effective date of the 2020 Plan, no further awards will be made under the 2018 Plan. Any options or awards outstanding under the 2018 Plan remain outstanding and effective and are governed by their existing terms.

On June 16, 2021, the Company's stockholders approved an amendment to the 2020 Plan to reserve an additional 466,666 shares of common stock for issuance under the plan.

On December 1, 2022, the Company's stockholders approved an amendment and restatement of the 2020 Plan to (i) increase the number of shares of common stock reserved for issuance under the plan by 866,666 shares to 1,533,333 shares, subject to adjustment in the event of stock splits and other similar events, (ii) provide for an annual increase, to be added on the first day of each fiscal year during the term of the plan, beginning with the fiscal year ending December 31, 2023, of 5% of the number of shares of common stock outstanding on the first day of such fiscal year or a lesser number of shares determined by the Company's Board of Directors, (iii) provide that up to 1,858,601 shares of common stock may be granted as "incentive stock options" under the plan, (iv) extend the term of the plan to December 1, 2032 and (v) revise certain provisions of the plan relating to the Company's Board of Directors' ability to delegate authority to make awards under the plan.

At December 31, 2023, 738,778 shares remained available for future issuance under the 2020 Plan. Under the 2020 Plan, stock options may not be granted at less than fair value on the date of grant.

2021 Employee Stock Purchase Plan

The 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 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 473,525 shares. At December 31, 2023, 397,546 shares remained available for future issuance under the ESPP.

Stock Options

The following table summarizes the Company's stock option activity for the year ended December 31, 2023:

	Number of Options	Weighted Average Exercise Price	Remaining Contractual Life (in years)
Outstanding at December 31, 2022	1,433,968	$ 36.22	8.88
Granted	1,141,449	5.38	
Exercised	—	—	
Expired	(54,941)	81.84	
Forfeitures	(260,804)	15.07	
Outstanding at December 31, 2023	2,259,672	$ 21.93	8.20
Vested and expected to vest as of December 31, 2023	2,259,672	$ 21.93	8.20
Exercisable at December 31, 2023	616,133	$ 57.99	7.69

At December 31, 2023, the Company had an aggregate of $8,949 of unrecognized equity-based compensation cost related to stock options outstanding which is expected to be recognized over a weighted average period of 2.8 years. The intrinsic value of stock options outstanding as of December 31, 2023 and 2022 was $863 and $0, respectively. The intrinsic value of stock options exercisable as of December 31, 2023 and 2022 was $27 and $0, respectively. No stock options were exercised during the year ended December 31, 2023.

The fair value of each option award is estimated on the date of grant using the Black-Scholes option-pricing model using the assumptions noted in the following table for the years ended December 31:

	2023	2022	2021
Expected volatility	121.8% - 129.60%	118.5% - 130.3%	115.5% - 123.9%
Expected dividends	0.0%	0.0%	0.0%
Expected term (in years)	5.31 - 6.25	5.31 - 6.25	5.10 - 6.25
Risk-free rate	3.5% - 4.9%	1.4% - 3.9%	0.4% - 1.4%

The weighted average fair value of options to purchase shares of common stock granted during the year ended December 31, 2023 and 2022 was $4.76 and $7.59, respectively.

Restricted Stock Units

In 2023, 2022 and 2021, the Company's Board of Directors issued restricted stock units to employees. Restricted stock unit grants typically vest over one to four years.

The following table summarizes the Company's restricted stock unit activity for the year ended December 31, 2023:

	Units	Weighted-Average Grant Date Fair Value
Unvested at December 31, 2022	512,557	$ 8.39
Granted	666,516	5.46
Vested	(183,951)	7.64
Forfeitures	(117,941)	7.96
Outstanding at December 31, 2023	877,181	$ 6.37
Unvested as of December 31, 2023	877,181	$ 6.37

At December 31, 2023, the Company had an aggregate of $4,293 of unrecognized equity-based compensation cost related to restricted stock units outstanding. The unrecognized expense for the restricted stock units is expected to be recognized over a weighted average period of 2.59 years.

Restricted Common Stock

In connection with the Company's statutory corporate conversion on January 25, 2018, all restricted Series B and D common units were converted into restricted shares of common stock. As of December 31, 2021, there were no remaining restricted shares of common stock outstanding.

No restricted common shares vested during the years ended December 31, 2023 and 2022. The aggregate intrinsic value of restricted common shares that vested during the year ended December 31, 2021 was $199.

At December 31, 2023, the Company had an aggregate of $0 of unrecognized equity-based compensation related restricted shares of common stock.

The Company recorded equity-based compensation expense related to all of its share-based awards to employees and non-employees in the following captions within its consolidated statements of operations for the years ended December 31, 2023, 2022, and 2021:

	For the Year Ended December 31,		
	2023	2022	2021
Research and development expenses	$ 3,094	$ 2,756	$ 6,289
General and administrative expenses	4,531	4,781	7,084
	$ 7,625	$ 7,537	$ 13,373

11. Leases

The Company has operating leases for laboratory and office space in Massachusetts, North Carolina and Florida.

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 $305 with annual escalation of approximately 3%. The lease includes a $10,223 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 $1,833, subject to decrease on a set schedule, as a security deposit pursuant to the lease.

During the year ended December 31, 2022, the Company recorded a failed sales-leaseback transaction related to certain lab equipment. The related financing liabilities are recorded on the Company's consolidated balance sheets within financing liabilities. In connection with this transaction, the Company also recorded a cash inflow within the financing activities under proceeds from financing liabilities of $2,143.

As of December 31, 2023, minimum future lease payments for these operating and finance leases were as follows:

	Finance Leases	Operating Leases
2024	$ 810	$ 4,396
2025	651	4,110
2026	—	4,123
2027	—	4,239
Thereafter	—	21,482
Total	1,461	38,350
Less: Imputed Interest	(242)	13,788
Total Lease Liabilities	$ 1,703	$ 24,562

The Company recorded rent expense of $5,255, $3,881 and $2,568 for the years ended December 31, 2023, 2022, and 2021, respectively.

Short-term lease and variable lease costs were not material for the year ended December 31, 2023 and 2022.

The supplemental disclosure of cash flow information related to the Company's leases and the weighted average remaining lease term and weighted average discount rate of the Company's leases are as follows:

	For the Year Ended December 31, 2023	For the Year Ended December 31, 2022	For the Year Ended December 31, 2021
Other information			
Cash paid for amounts included in the measurement of lease liabilities	$ 5,310	$ 2,840	$ 2,408
Operating lease liabilities arising from obtaining right-of-use-assets	$ 410	$ 29,126	$ 310
Finance lease liabilities arising from obtaining right-of-use assets	$ —	$ —	$ —
Weighted-average remaining lease term (in years)			
Operating lease	8.5	9.3	1.0
Finance lease	1.8	2.5	2.3
Weighted-average discount rate			
Operating lease	10.7%	10.6%	11.9%
Finance lease	22.8%	21.3%	10.7%

12. Commitments and Contingencies

Letter of Credit

The Company had an outstanding letter of credit in the amount of $1,833 at December 31, 2023 and 2022, 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, 2023 and 2022.

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, 2023.

13. 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 $1,000 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 $375 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 $3,400 upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. 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 $41, $96, and $60 for the years ended December 31, 2023, 2022, and 2021, 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 Duchene 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 $1,000 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 $750 in 2021 and $1,300 in 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 Company paid $750 in February 2021 and $1,300 in February 2022. The license agreement was also amended such that the Company is required to pay an aggregate of approximately $1,900 upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. 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 $132, $133, and $195 for the years ended December 31, 2023, 2022, and 2021, respectively, under the agreement.

The University of Michigan License Agreement

In 2016, the Company entered into a license agreement with the Regents of the University of Michigan, (the "University of Michigan"), a constitutional corporation of Michigan, under which the Company obtained an exclusive, royalty-bearing, sublicensable, worldwide license to make, sell and distribute products under certain patents owned by the University of Michigan related to microdystrophin and utrophin spectrin-like nucleic acid sequences for any use that, but for this agreement, would comprise an infringement of a valid claim included in the licensed patent rights.

In consideration for the rights granted by the agreement, the Company paid a one-time license fee and a separate fee to cover past patent prosecution costs, which the Company recorded as a research and development expense in 2016. The Company was required to reimburse the University of Michigan for costs incurred in applying for, prosecuting and maintaining patents, and pay up to an aggregate of approximately $1,000 upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2023, 2022, and 2021. The Company was also required to pay a royalty of a low single digit percentage on future sales by the Company or its sublicensees of products developed using the licensed rights, with a minimum annual royalty after certain milestones are achieved. In addition, the Company was required to pay an annual maintenance fee in any year in which the minimum annual royalty is not reached.

Under the agreement, the University of Michigan reserved for itself and its affiliates the right to use the licensed rights for non-commercial research, public service, internal and educational purposes and the right to grant the same limited non-commercial rights to other non-profit research institutions.

The Company recorded research and development expense in the amount of $0, $0 and $37 for the years ended December 31, 2023, 2022, and 2021, 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 $2,900 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 $50. 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 agreement entered in 2023, the Company agreed to pay to UFRF cumulative sales milestones of up to $8,500 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 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 (10) 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 €1,500, 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 €15,000, cumulative sales milestone payments of up to €15,000, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits.

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.

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, 2023, potential future milestone payments under these agreements totaled an aggregate of $12,000. None of these milestones were assessed to be probable as of December 31, 2023.

14. Net Loss per Share

Basic and diluted net loss per share were calculated as follows:

The numerator for basic and diluted net loss per share is as follows:

	For the Year Ended December 31,		
	2023	2022	2021
Net loss	$ (96,015)	$ (85,981)	$ (72,188)

The denominator is as follows:

	For the Year Ended December 31,		
	2023	2022	2021
Weighted average common stock outstanding, basic and diluted	19,884,007	8,512,089	6,974,136
Weighted average pre-funded warrants to purchase common stock	—	—	143,888
Total	19,884,007	8,512,089	7,118,024

Net loss per share, basic and diluted is as follows:

	For the Year Ended December 31,		
	2023	2022	2021
Net loss per share, basic and diluted	$ (4.83)	$ (10.10)	$ (10.14)

The following potential common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	For the Year Ended December 31,		
	2023	2022	2021
Options to purchase shares of common stock	2,259,672	1,433,968	400,842
Unvested restricted stock units	877,181	512,557	33,979
	3,136,853	1,946,525	434,821

15. Income Taxes

The Company recorded no tax benefit for the years ended December 31, 2023 and 2022 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, 2023 and 2022 is as follows:

	December 31, 2023	December 31, 2022
Income tax computed at federal statutory tax rate	21.0%	21.0%
State taxes, net of federal benefit	6.6%	7.4%
Permanent differences	(0.5)%	(1.4)%
Bargain purchase gain	0.0%	4.5%
Tax credits	7.0%	11.5%
Change in deferred tax rate	0.2%	0.1%
Stock compensation cancelations	(1.0)%	(1.9)%
Impact of ownership change	(108.5)%	0.0%
Other items	0.4%	(0.6)%
Valuation allowance	74.8%	(40.6)%
	0.0%	0.0%

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, 2023 and 2022 are as follows:

	December 31, 2023	December 31, 2022
Deferred tax assets:		
Tax loss carryforwards	$ 16,622	$ 70,965
Tax credit carryforwards	7,537	46,786
Deferred expenses	6,755	7,174
Accrued expenses	1,506	1,901
Stock compensation	8,496	7,839
Intangible assets	57,336	36,553
Depreciation	672	116
Other	80	166
Total deferred tax assets	99,004	171,500
Valuation allowance	(91,705)	(163,566)
Deferred tax liabilities:		
Right-of-use asset	(7,299)	(7,934)
Depreciation	—	—
Total deferred tax liabilities	(7,299)	(7,934)
Net deferred taxes	$ —	$ —

As of December 31, 2023, the Company has federal net operating loss carryforwards of $59,392 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, 2023, the Company has state net operating loss carryforwards of approximately $66,402 which may be available to offset future taxable income, of which $64,032 begins to expire in 2032 and $2,371 has unlimited carryforward. The Company also had federal and state tax credits of $6,599 and $1,187, respectively, which may be used to offset future tax liability and each of which begin to expire in 2042.

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 Internal Revenue Code Section 382. 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 to assess whether a change of control has 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 carryfowards before the ownership change date of December 2, 2022 are not available for utilization and have been written off. The carryfowards as of December 31, 2023 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, 2023	December 31, 2022
Valuation allowance at beginning of year	$ 163,566	$ 128,570
(Decreases) increases recorded to income tax provision	(71,861)	34,996
Valuation allowance at end of year	$ 91,705	$ 163,566

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, 2019 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, 2023 and 2022, 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, 2023 and 2022, no interest and penalties have been recorded.

16. 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 $626, $527, and $241 of contributions during the years ended December 31, 2023, 2022, and 2021, respectively.

17. Restructuring

April 2022 Plan

In April 2022, the Company implemented changes to its corporate strategy to prioritize the advancement of its then-key programs, SGT-001 and SGT-003. In connection with the changes to corporate operations, the Company reduced headcount by approximately 35 percent. During the year ended December 31, 2022, the Company recorded and paid aggregate restructuring charges of $1,520 related to severance and other employee related costs in connection with the changes to its corporate strategy. The Company does not expect to incur any additional significant costs associated with this restructuring.

November 2022 Plan

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 $5,658 related to the reduction in force, consisting of severance and other employee termination benefits. The Company paid $3,669 of this amount during the year ended December 31, 2023. The Company expects that approximately the remaining $252 will be paid by the first quarter of 2024.

The following table shows the total amount incurred and the liability related to the associated restructuring plans for the years ended December 31, 2023, 2022 and 2021:

One-Time Employee Termination Benefits	April 2022	November 2022
Accrued restructuring charges as of December 31, 2021	$ —	$ —
Accrual recorded as a result of restructuring charges	1,520	5,658
Amounts paid during the period	(1,520)	(1,737)
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

18. Subsequent Events

On January 11, 2024, the Company issued and sold 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, in the January 2024 Private Placement. The Company received approximately $104,034 of net proceeds from the January 2024 Private Placement after deducting offering costs.

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Directors and Executive Officers (as of April 26, 2024)

Directors

Alexander Cumbo, President and Chief Executive Officer, Solid Biosciences Inc.

Ian Smith, Executive Chairman, Solid Biosciences Inc.

Martin Freed, M.D., F.A.C.P., Independent consultant to private pharmaceutical, biotechnology, and healthcare companies

Ilan Ganot, Co-Founder and Former Chief Executive Officer, Solid Biosciences Inc., Chief Executive Officer and director, Alesta Therapeutics

Clare Kahn, Ph.D., R&D Strategy Officer, X-VAX Technology Inc.

Georgia Keresty, Ph.D., M.PH., Chief Operating Officer to stealth mode biotechnology start-up company

Adam Koppel, M.D., Ph.D., Partner, Bain Capital Life Sciences

Sukumar Nagendran, M.D., President, Head of Research and Development and a director, Taysha Gene Therapies, Inc.

Rajeev Shah, Managing Partner, RA Capital Management, L.P.

Adam Stone, Chief Investment Officer, Perceptive Advisors

Lynne Sullivan, Chief Financial Officer, UNITY Biotechnology, Inc.

Executive Officers

Gabriel Brooks, M.D., Chief Medical Officer

Jessie Hanrahan, Ph.D., Chief Regulatory Officer

Paul Herzich, Chief Technology Officer

David Tyronne Howton, Chief Operating Officer, General Counsel and Secretary

Jennifer Marlowe, Ph.D., Chief Scientific Officer

Kevin Tan, Chief Financial Officer and Treasurer

Legal Counsel
Wilmer Cutler Pickering Hale and Dorr LLP; Boston, MA

Transfer Agent and Registrar
Computershare Trust Company, N.A.; Canton, MA

Independent Auditors
PricewaterhouseCoopers LLP; Boston, MA

2024 Virtual Annual Meeting
The Annual Meeting of Stockholders will be held June 11, 2024, 8:00 am ET