UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2024

OR

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

For the transition period from _____ to _____

Commission File Number: 001-38360

Solid Biosciences Inc.

(Exact Name of Registrant as Specified in its Charter)

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

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

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

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

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 is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 1, 2024 the registrant had 39,954,607 shares of common stock, $0.001 par value per share, outstanding.

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q. 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 Quarterly Report on Form 10-Q.

The forward-looking statements in this Quarterly Report on Form 10-Q 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 capacity constraints and the scalability and commercial viability of our manufacturing methods and processes;
- our plans to develop and commercialize our neuromuscular (e.g., SGT-003, 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 Quarterly Report on Form 10-Q. In addition, even if our results of operations, financial condition, business and prospects are consistent with the forward-looking statements contained in this Quarterly Report on Form 10-Q, those results may not be indicative of results to be expected in subsequent periods.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q 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 Quarterly Report

on Form 10-Q are made as of the date of this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q, the terms “Solid,” “the Company,” “we,” “us” and “our” refer to Solid Biosciences Inc., and its consolidated subsidiaries, unless the context indicates otherwise.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements (unaudited)

SOLID BIOSCIENCES INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Unaudited)
(in thousands, except share and per share data)

	September 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 64,394	$ 74,015
Available-for-sale securities	106,723	49,625
Prepaid expenses and other current assets	8,377	6,094
Total current assets	179,494	129,734
Non-current assets:		
Operating lease, right-of-use assets	24,859	26,539
Property and equipment, net	5,067	6,624
Other non-current assets	475	209
Restricted cash	1,931	1,833
Total non-current assets	32,332	35,205
Total assets	$ 211,826	$ 164,939
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 3,458	$ 2,032
Accrued expenses and other current liabilities	13,227	10,161
Operating lease liabilities	1,718	1,855
Finance lease liabilities	1,051	469
Derivative liabilities	3,400	—
Total current liabilities	22,854	14,517
Non-current liabilities:		
Operating lease liabilities, excluding current portion	21,643	22,707
Finance lease liabilities, excluding current portion	307	1,234
Total non-current liabilities	21,950	23,941
Total liabilities	44,804	38,458
Commitments and contingencies (Note 10)		
Preferred stock, $0.001 par value — 10,000,000 shares authorized; no shares issued and outstanding at September 30, 2024 and December 31, 2023	—	—
Common stock, $0.001 par value — 120,000,000 and 60,000,000 shares authorized at September 30, 2024 and December 31, 2023, respectively; 38,930,203 and 20,386,606 shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively	39	20
Additional paid-in capital	907,720	785,199
Accumulated other comprehensive income	116	15
Accumulated deficit	(740,853)	(658,753)
Total stockholders' equity	167,022	126,481
Total liabilities and stockholders' equity	$ 211,826	$ 164,939

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

SOLID BIOSCIENCES INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Unaudited)
(in thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 27,327	$ 16,702	$ 65,661	$ 61,110
General and administrative	7,855	6,412	24,171	20,940
Restructuring charges	—	—	—	(63)
Total operating expenses	35,182	23,114	89,832	81,987
Loss from operations	(35,182)	(23,114)	(89,832)	(81,987)
Other income, net:				
Interest income	2,328	1,962	7,544	5,822
Interest expense	(82)	(106)	(265)	(339)
Other income, net	211	278	453	825
Total other income, net	2,457	2,134	7,732	6,308
Net loss	$ (32,725)	$ (20,980)	$ (82,100)	$ (75,679)
Net loss per share, basic and diluted	$ (0.79)	$ (1.05)	$ (2.04)	$ (3.83)
Weighted average shares of common stock outstanding, basic and diluted	41,443,317	20,059,641	40,182,303	19,767,174

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

SOLID BIOSCIENCES INC.
CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(Unaudited)
(in thousands)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Net loss	$ (32,725)	$ (20,980)	$ (82,100)	$ (75,679)
Other comprehensive income (loss):				
Unrealized gain (loss) on available-for-sale securities	131	(12)	101	70
Comprehensive loss	$ (32,594)	$ (20,992)	$ (81,999)	$ (75,609)

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

SOLID BIOSCIENCES INC.
CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(Unaudited)
(in thousands, except share data)

	Nine Months Ended September 30, 2024					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at January 1, 2024	20,386,606	$ 20	$ 785,199	$ 15	$ (658,753)	$ 126,481
Issuance of common stock in private placement, net of issuance costs of $4,407	16,973,103	17	89,437	—	—	89,454
Issuance of pre-funded warrants in private placement, net of issuance costs of $704	—	—	14,293	—	—	14,293
Issuance of common stock in public offering, net of sales commissions of $78	350,664	1	3,055	—	—	3,056
Vesting of restricted stock units	98,677	—	—	—	—	—
Exercises of common stock options	24,639	—	151	—	—	151
Unrealized loss on available-for-sale securities	—	—	—	(19)	—	(19)
Equity-based compensation	—	—	1,611	—	—	1,611
Net loss	—	—	—	—	(24,303)	(24,303)
Balance at March 31, 2024	37,833,689	38	893,746	(4)	(683,056)	210,724
Issuance of common stock in public offering, net of sales commissions of $138	526,953	1	5,356	—	—	5,357
Vesting of restricted stock units	131,859	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	56,855	—	145	—	—	145
Exercises of common stock options	1,703	—	10	—	—	10
Unrealized loss on available-for-sale securities	—	—	—	(11)	—	(11)
Equity-based compensation	—	—	2,092	—	—	2,092
Net loss	—	—	—	—	(25,072)	(25,072)
Balance at June 30, 2024	38,551,059	39	901,349	(15)	(708,128)	193,245
Issuance of common stock in public offering, net of sales commissions of $79	330,670	—	3,099	—	—	3,099
Vesting of restricted stock units	6,874	—	—	—	—	—
Exercises of common stock options	41,600	—	277	—	—	277
Unrealized gain on available-for-sale securities	—	—	—	131	—	131
Equity-based compensation	—	—	2,995	—	—	2,995
Net loss	—	—	—	—	(32,725)	(32,725)
Balance at September 30, 2024	38,930,203	$ 39	$ 907,720	$ 116	$ (740,853)	$ 167,022

	Nine Months Ended September 30, 2023					
	Common Stock		Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at January 1, 2023	19,556,732	$ 20	$ 774,452	$ (68)	$ (562,738)	$ 211,666
Vesting of restricted stock units	16,400	—	—	—	—	—
Unrealized gain on available-for-sale securities	—	—	—	73	—	73
Equity-based compensation	—	—	2,118	—	—	2,118
Net loss	—	—	—	—	(30,070)	(30,070)
Balance at March 31, 2023	19,573,132	20	776,570	5	(592,808)	183,787
Issuance of common stock in public offering, net of sales commissions of $65	420,000	—	2,539	—	—	2,539
Vesting of restricted stock units	36,321	—	—	—	—	—
Issuance of common stock under employee stock purchase plan	14,936	—	78	—	—	78
Unrealized gain on available-for-sale securities	—	—	—	9	—	9
Equity-based compensation	—	—	1,944	—	—	1,944
Net loss	—	—	—	—	(24,629)	(24,629)
Balance at June 30, 2023	20,044,389	20	781,131	14	(617,437)	163,728
Vesting of restricted stock units	17,362	—	—	—	—	—
Unrealized loss on available-for-sale securities	—	—	—	(12)	—	(12)
Equity-based compensation	—	—	1,625	—	—	1,625
Net loss	—	—	—	—	(20,980)	(20,980)
Balance at September 30, 2023	20,061,751	$ 20	$ 782,756	$ 2	$ (638,417)	$ 144,361

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

SOLID BIOSCIENCES INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Unaudited)
(in thousands)

	Nine Months Ended September 30,	
	2024	2023
Cash flows from operating activities:		
Net loss	$ (82,100)	$ (75,679)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of discount on available-for-sale securities	(2,789)	(1,257)
Equity-based compensation expense	6,698	5,687
Depreciation, impairment, and amortization expense	1,967	2,368
Non-cash lease expense	1,826	1,881
Non-cash acquired in-process research and development	3,400	—
Other	(90)	—
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(2,549)	1,124
Accounts payable	1,390	(898)
Operating lease liabilities	(1,262)	(1,261)
Accrued expenses and other liabilities	3,142	(5,322)
Net cash used in operating activities	(70,367)	(73,357)
Cash flows from investing activities:		
Purchases of property and equipment	(450)	(1,425)
Proceeds from maturities of available-for-sale securities	134,800	68,632
Purchases of available-for-sale securities	(189,008)	(102,814)
Other	5	—
Net cash used in investing activities	(54,653)	(35,607)
Cash flows from financing activities:		
Proceeds from issuance of common stock and pre-funded warrants in private placement	108,858	—
Payments of common stock and pre-funded warrants issuance costs in private placement	(5,111)	—
Proceeds from issuance of common stock in public offering, net of sales commissions	11,512	2,539
Proceeds from exercises of common stock options	438	—
Employee stock purchase plan purchases	145	78
Payments of principal portion of finance lease obligations	(345)	—
Net cash provided by financing activities	115,497	2,617
Net decrease in cash, cash equivalents, and restricted cash	(9,523)	(106,347)
Cash, cash equivalents, and restricted cash at beginning of period	75,848	157,217
Cash, cash equivalents, and restricted cash at end of period	$ 66,325	$ 50,870
Supplemental disclosure of cash flow information:		
Cash paid for interest	$ (265)	$ —
Supplemental disclosure of non-cash investing and operating activities:		
Right-of-use assets acquired through operating leases	$ 407	$ 121
Decrease in right-of-use assets due to lease termination	$ (261)	$ (252)
Decrease in property and equipment due to asset exchange	$ —	$ (950)
Property and equipment purchases included in accounts payable and accrued expenses	$ 36	$ —

The following table provides a reconciliation of cash, cash equivalents, and restricted cash reported within the condensed consolidated balance sheets to the amounts reported in the condensed consolidated statements of cash flows:

	September 30,	
	2024	2023
Cash and cash equivalents	$ 64,394	$ 49,037
Restricted cash, non-current	1,931	1,833
Total cash and cash equivalents, and restricted cash, as reported in the condensed consolidated statements of cash flows	$ 66,325	$ 50,870

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

1. Nature of the Business and Basis of Presentation

Nature of business

Solid Biosciences Inc. was organized in March 2013 under the name SOLID Ventures Management, LLC and operated as a Delaware limited liability company until immediately prior to the effectiveness of its registration statement on Form S-1 on January 25, 2018, at which time it completed a statutory corporate conversion into a Delaware corporation and changed its name to Solid Biosciences Inc. (the "Company"). On December 2, 2022, the Company completed its acquisition of AavantiBio, Inc. ("AavantiBio"), a privately held gene therapy company focused on transforming the lives of patients with Friedreich's ataxia ("FA") and rare cardiomyopathies (the "Acquisition"). Upon the consummation of the Acquisition, the Company acquired AavantiBio's gene therapy programs, AVB-202-TT for FA and AVB-401 for BAG3 mediated dilated cardiomyopathy, as well as additional assets for the treatment of other cardiac diseases, platform technologies and know-how related thereto. AavantiBio is a wholly owned subsidiary of the Company.

The Company is a life sciences company focused on advancing a portfolio of current and future gene therapy candidates (collectively, "Candidates"), including SGT-003 for the treatment of Duchenne muscular dystrophy ("Duchenne"), SGT-501 for the treatment of catecholaminergic polymorphic ventricular tachycardia, 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, the Company's mission is to improve the daily lives of patients living with these devastating diseases.

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on licenses, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing to fund operations. Candidates currently under development will require significant additional research and development efforts, including extensive preclinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting capabilities.

The Company's Candidates are in development. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from, among others, other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, partners, and consultants.

Liquidity

The accompanying condensed 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 September 30, 2024, the Company has funded its operations primarily with the proceeds from the sale of redeemable preferred units and member units as well as the sale of common stock and pre-funded warrants to purchase shares of its common stock in private placements and the sale of common stock in its initial public offering, follow-on public offering in March 2021 and under its at-the-market sales agreement.

On January 11, 2024, the Company issued and sold in a private placement 16,973,103 shares of the Company's common stock at a price per share of $5.53 and, to one investor in lieu of shares of common stock, pre-funded warrants to purchase 2,712,478 shares of common stock at a price of $5.529 per pre-funded warrant (the "January 2024 Private Placement"). The Company received $103.7 million of net proceeds from the January 2024 Private Placement after deducting offering costs. No warrants were exercised during the nine months ended September 30, 2024.

During the three and nine months ended September 30, 2024, the Company issued and sold 330,670 and 1,208,287 shares of its common stock, respectively, pursuant to the Company's "at-the-market-offering" sales agreement (the "ATM Sales Agreement"), between the Company and Jefferies LLC. During the three and nine months ended September 30, 2024, the Company received net proceeds of $3.1 million and $11.5 million, respectively, from sales pursuant to the ATM Sales Agreement.

The Company has evaluated whether there are conditions and events that, considered in the aggregate, 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

September 30, 2024, the Company had an accumulated deficit of $740.9 million. During the three and nine months ended September 30, 2024, the Company incurred a net loss of $32.7 million and $82.1 million, respectively, and used $70.4 million of cash in operations for the nine months ended September 30, 2024. The Company expects to continue to generate operating losses for the foreseeable future. Based upon its current operating plan, the Company expects that its cash, cash equivalents and available-for-sale securities of $171.1 million excluding restricted cash of $1.9 million, as of September 30, 2024, will be sufficient to fund its operating expenses and capital expenditure requirements for at least twelve months from the date of issuance of these condensed consolidated 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 partnerships and 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.

Basis of presentation

These condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP") along with the rules and regulations of the Securities and Exchange Commission for interim financial information and include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation. The year-end condensed consolidated balance sheet data presented for comparative purposes was derived from the Company's audited financial statements but does not include all disclosures required by GAAP to constitute a complete set of financial statements. These condensed consolidated financial statements have been prepared on the same basis as the Company's annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for a fair statement of the Company's financial position at September 30, 2024 and its results of operations, changes in stockholders' equity, and cash flows for the interim periods ended September 30, 2024 and 2023.

These unaudited condensed consolidated interim financial statements should be read in conjunction with the Company's audited consolidated financial statements and notes thereto included in the Company's Annual Report on Form 10-K for the year ended December 31, 2023. The results of operations for the three and nine months ended September 30, 2024 are not necessarily indicative of the operating results to be expected for the year ending December 31, 2024, for any other interim period, or for any other future year.

Reclassifications

Certain amounts reported within cash flows from operating activities in the condensed consolidated statement of cash flows for the prior period have been reclassified to conform to current period presentation. These reclassifications are not material and had no effect on the previously reported net cash used in operations.

Significant judgments and estimates

The preparation of the Company's condensed 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 condensed consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, estimates related to the recognition of research and development expenses, equity-based compensation, and derivative liabilities. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ materially from the Company's estimates.

Summary of significant accounting policies

Except as otherwise noted below, there have been no changes to the significant accounting policies disclosed in the Company's most recent Annual Report on Form 10-K.

Equity-based compensation

The Company measures all stock options and other stock-based awards granted to employees, directors, and non-employees based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company applies the straight-line method of expense recognition to all awards with only service-based vesting conditions.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. 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 values of restricted stock units and performance-based restricted stock units are measured at the grant date based on the closing price of the Company's common stock on the date of grant. For restricted stock units, the fair value of the award is recognized on a straight-line basis over the requisite service periods. For performance-based restricted stock unit awards, which are subject to the achievement of performance milestones, the fair value is recognized as expense over the requisite service periods when the achievement of such performance milestones determined to be probable. If a performance milestone is not determined to be probable or is not met, no equity-based compensation expense is recognized, and any previously recognized expense is reversed.

Forfeitures are recognized as a reduction of equity-based compensation expense as they occur.

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

The Company does not currently hold any treasury shares. Upon the exercise of stock options and the vesting of restricted stock units and performance stock units, the Company issues new shares of common stock and delivers them to the participant.

Asset acquisition

Acquisitions of assets or a group of assets that do not meet the definition of a business are accounted as asset acquisitions using the cost accumulation method, whereby the cost of the acquisition, including certain transaction costs, is allocated to the assets acquired on the basis of relative fair values. No goodwill is recognized in an asset acquisition. Intangible assets that are acquired in an asset acquisition for use in research and development activities which have an alternative future use are capitalized as in-process research and development ("IPR&D"). Acquired IPR&D which has no alternative future use is recognized as research and development expense at acquisition. Contingent milestone payments associated with asset acquisitions are recognized when probable and estimable. These amounts are expensed to research and development if there is no alternative future use associated with the asset or capitalized as an intangible asset if an alternative future use of the asset exists.

Derivative liabilities

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

Recently issued accounting pronouncements

In November 2023, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2023-07, *Segment Reporting (Topic 280)*: *Improvements to Reportable Segment Disclosures*, which requires public entities, including those with a single reportable segment to: (i) provide disclosures of significant segment expenses and other segment items if they are regularly provided to the chief operating decision maker (the CODM) and included in each reported measure of segment profit or loss; (ii) provide all annual disclosures about a reportable segment's profit or loss and assets currently required by Accounting Standards Codification 280, Segment Reporting, in interim periods; and (iii) disclose the CODM's title and position, as well as an explanation of how the CODM uses the reported measures and other disclosures. ASU 2023-07 does not change how a public entity identifies its operating segments, aggregates those operating segments or applies the quantitative thresholds to determine its reportable segments. ASU 2023-07 is required to be applied retrospectively and is effective for fiscal years beginning after December 15, 2023, and interim periods within fiscal years beginning after December 15, 2024, with early adoption permitted. We expect to include additional disclosures as a result of the implementation ASU 2023-07, however, these changes are not expected to have a material effect on our consolidated financial statements.

In December 2023, the FASB issued ASU 2023-09, *Income Taxes (Topic 740): Improvements to Income Tax Disclosures*. This ASU updates income tax disclosure requirements primarily by requiring specific categories and greater disaggregation within the rate reconciliation and disaggregation of income taxes paid by jurisdiction. This ASU is effective for annual periods beginning after

December 15, 2024 and is applicable to the Company's fiscal year beginning January 1, 2025, with early application permitted. The Company is currently evaluating the impact of adopting this ASU on its consolidated financial statements and disclosures.

In November 2024, the FASB issued ASU 2024-03, *Disaggregation of Income Statement Expenses (Topic 220)* requiring that public business entities disclose additional information about specific expense categories in the notes to financial statements at interim and annual reporting periods. The amendments in this ASU are effective for annual reporting periods beginning after December 15, 2026, and interim reporting periods beginning after December 15, 2027. The requirements in this ASC may be applied either prospectively to financial statements issued for reporting periods after the effective date or retrospectively to any or all prior periods presented in the financial statements. The Company is currently evaluating the impact of adopting this ASU on its consolidated financial statements and disclosures.

2. FA212 Asset Acquisition

On September 19, 2024, the Company entered into an asset purchase agreement with FA212 LLC ("FA212") for the purchase of certain intellectual property, including patents and assigned licenses related to a pre-clinical drug candidate, assigned manufacturing contracts as well as research and development materials such as manufactured materials and samples.

The Company paid FA212 an upfront payment of $1.0 million. Additionally, the Company agreed to pay FA212 development milestone payments of up to $34.0 million, cumulative sales milestone payments of up to $21.0 million, and tiered royalties on net sales in the low-single-digits. The Company also assumed contingent development milestone payments of up to $4.2 million, regulatory milestone payments of up to $13.0 million, cumulative sales milestone payments of up to $27.5 million, and tiered royalties on worldwide net sales in the mid-single digits with the University of Pennsylvania, who is the IP owner.

Certain development milestone payments to FA212 are payable in either cash, equity, or a combination of both at the companies discretion. Such contingent payments were determined to be derivative liabilities and were initially recorded at their fair value of $3.4 million, see Note 4 - Fair Value Measurements, which is included in the aggregate acquisition cost below.

The Company determined that the FA212 Agreement represented an asset acquisition of IPR&D assets with no alternative future use and recognized the aggregate acquisition cost of $5.1 million as research and development expense in the condensed consolidated statement of operations. The acquisition did not qualify as a business combination as the acquisition did not include both an input and substantive processes, including an assembled workforce, that together contribute to the ability to create outputs.

3. License and Research Agreements

On June 29, 2023, the Company entered into a license agreement (the "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 Agreement, Maugeri granted the Company an exclusive worldwide sublicensable license for certain Maugeri patent rights, including existing patent rights, and those in any improvements or know-how made in performance of the 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 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 Agreement was regulatory review in Italy, which was completed in the third quarter of 2023 and, upon the completion of the condition precedent, the Agreement became effective.

The Company paid Maugeri an upfront license fee of €1.5 million which was recorded as research and development expense during the second quarter of 2023. Additionally, the Company agreed to cumulative developmental, regulatory, and commercial milestone payments of up to €15.0 million, cumulative sales milestone payments of up to €15.0 million, upon achievement of specified milestone events, and tiered royalties on worldwide net sales in the low-to-mid-single-digits.

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

4. Fair Value Measurements

Fair value is an exit price, representing the amount that would be received from the sale of an asset or paid to transfer a liability in an orderly transaction between market participants. As such, fair value is a market-based measurement that should be determined based on assumptions that market participants would use in pricing an asset or liability. Valuation techniques used to measure fair value are performed in a manner to maximize the use of observable inputs and minimize the use of unobservable inputs. As a basis for considering such assumptions, the accounting literature establishes a three-tier value hierarchy which prioritizes the inputs used in measuring fair value as follows:

- Level 1—Observable inputs, such as 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 following tables present information about the Company's financial assets and liabilities that are measured at fair value on a recurring basis:

	September 30, 2024			
	Level 1	Level 2	Level 3	Total
Financial assets				
Cash equivalents:				
Money market funds	$ —	$ 19,622	$ —	$ 19,622
Treasury bills	—	4,991	—	4,991
Government bonds	—	1,298	—	1,298
Total cash equivalents	—	25,911	—	25,911
Available-for-sale securities (Note 5):				
Treasury bills	—	63,635	—	63,635
Government bonds	—	43,088	—	43,088
Total available-for-sale securities	—	106,723	—	106,723
Total financial assets	$ —	$ 132,634	$ —	$ 132,634
Financial liabilities				
Derivative liabilities	$ —	$ —	$ 3,400	$ 3,400
Total financial liabilities	$ —	$ —	$ 3,400	$ 3,400

	December 31, 2023			
	Level 1	Level 2	Level 3	Total
Financial assets				
Cash equivalents:				
Money market funds	$ —	$ 62,141	$ —	$ 62,141
Certificates of deposit	—	257	—	257
Total cash equivalents	—	62,398	—	62,398
Available-for-sale securities (Note 5):				
Treasury bills	—	49,625	—	49,625
Total available-for-sale securities	—	49,625	—	49,625
Total financial assets	$ —	$ 112,023	$ —	$ 112,023

As of September 30, 2024 and December 31, 2023, the fair values of the Company's cash equivalents and available-for-sale securities were determined using Level 2 inputs.

The Company estimated the fair value of the derivative liabilities by using a Monte Carlo simulation forecasting the timing and likelihood of certain development milestone events being achieved and discounting the probability adjusted payments using an appropriate discount rate based on market interest rates. The main assumptions when determining the fair value of the derivative liabilities are the timing of and probability of achieving certain milestones, the estimated volatility of the Company's common stock, and the discount rate. The estimated fair value presented is not necessarily indicative of an amount that could be realized in a current market exchange. The use of alternative inputs and estimation methodologies could have a material effect on these estimates of fair value.

Significant unobservable inputs for the derivative liabilities are as follows:

Derivative Liabilities	Fair Values at September 30, 2024	Valuation Technique	Unobservable Input	Range	Average
Development Milestones	$3,400	Monte Carlo Simulation	Probability of achieving certain development milestones	2.0% - 80.0%	28.6%
			Volatility	114.0%	114.0%
			Discount Rate	3.5% - 4.8%	3.8%
			Timing of achieving certain development milestones	0.3 to 5.5 years	2.4 years

As of the acquisition date, see Note 2 - FA212 Asset Acquisition, the derivative liabilities were recorded at a fair value of $3.4 million and there has been no gain or loss recorded for a change in fair value for the three and nine months ended September 30, 2024.
As of September 30, 2024, the fair values of the Company's derivative liabilities were determined using Level 3 inputs. During the nine months ended September 30, 2024 and the year ended December 31, 2023, there were no transfers between Level 1, Level 2, and Level 3.

As of September 30, 2024 and December 31, 2023, the Company's accounts payable, accrued expenses, and other current liabilities approximated their estimated fair values due to the short term nature of these financial instruments.

5. Available-for-Sale Securities

A summary of the Company's available-for-sale securities is presented below:

	September 30, 2024			
Description	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Treasury bills maturing in one year or less	$ 63,580	$ 55	$ —	$ 63,635
Government bonds maturing in one year or less	43,028	60	—	$ 43,088
Total available-for-sale securities	$ 106,608	$ 115	$ —	$ 106,723

	December 31, 2023			
Description	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Treasury bills maturing in one year or less	$ 49,610	$ 15	$ —	$ 49,625
Total available-for sale securities	$ 49,610	$ 15	$ —	$ 49,625

The weighted average contractual maturity of the Company's available-for-sale securities was approximately 0.5 years and 0.4 years as of September 30, 2024 and December 31, 2023, respectively.

6. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

	September 30, 2024	December 31, 2023
Prepaid research and development expenses	$ 6,768	$ 3,980
Prepaid other and other current assets	1,609	2,114
Total	$ 8,377	$ 6,094

7. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following:

	September 30, 2024	December 31, 2023
Accrued research and development expense	$ 6,643	$ 2,614
Accrued compensation	5,117	5,948
Accrued other and other current liabilities	1,467	1,599
Total	$ 13,227	$ 10,161

8. Equity-Based Compensation

Equity-based compensation expense

The Company has classified equity-based compensation in its condensed consolidated statements of operations as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Research and development	$ 934	$ 677	$ 2,314	$ 2,285
General and administrative	2,061	948	4,384	3,402
Total	$ 2,995	$ 1,625	$ 6,698	$ 5,687

Equity incentive plans

As of September 30, 2024, the Company’s approved equity incentive plans include: the 2018 Omnibus Incentive Plan (the “2018 Plan”); Amended and Restated 2020 Equity Incentive Plan (the “2020 Plan”); the 2021 Employee Stock Purchase Plan; and the 2024 Inducement Stock Incentive Plan (the "2024 Inducement Plan"). These plans are administered by the Board of Directors (the "Board") and permit the granting of stock options, stock appreciation rights, restricted stock, restricted stock units ("RSUs"), performance awards, and other stock-based or cash-based awards. Upon the adoption of the 2020 Plan, the Company no longer grants new equity awards under its 2018 Plan.

Amended and Restated 2020 Equity Incentive Plan

On June 11, 2024, the Company's stockholders approved an amendment to the 2020 Plan to increase the number of shares of common stock reserved for issuance under the plan by 2,000,000 shares. As of September 30, 2024, there were 2,007,144 stock options outstanding, 1,007,794 RSUs outstanding, 2,165,325 performance stock units outstanding, and 171,622 shares remained available for future issuance under the 2020 Plan.

2024 Inducement Stock Incentive Plan

In March 2024, the Board approved the 2024 Inducement Plan, which provides for the reservation of 1,000,000 shares of common stock for equity granted as an inducement material to the individual’s entering into employment with the Company and in accordance with the requirements of Nasdaq Stock Market Rule 5635(c)(4). As of September 30, 2024, there were 100,000 stock options outstanding, 227,141 RSUs outstanding, and 672,859 shares remained available for future issuance under the 2024 Inducement Plan.

Stock options

The table below summarizes the activity with respect to stock options for the nine months ended September 30, 2024:

	Number of Options		Weighted Average Exercise Price	Remaining Contractual Life (in years)
Outstanding at January 1, 2024	2,259,672	$	21.93	
Granted	1,219,522	$	7.81	
Exercised	(67,942)	$	6.44	
Expired	(41,659)	$	24.77	
Forfeitures	(428,550)	$	8.95	
Outstanding at September 30, 2024	2,941,043	$	18.29	8.26
Vested and expected to vest as of September 30, 2024	2,941,043	$	18.29	8.26
Exercisable at September 30, 2024	1,029,832	$	38.16	6.88

The assumptions used in the Black-Scholes option-pricing model for all stock options granted during each period presented are as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Common stock price	$8.64	$3.62-$4.13	$5.76-$8.64	$3.62-$7.33
Expected volatility	119.7%	121.8%-121.9%	119.7%-128.7%	121.8%-129.6%
Expected dividends	0.0%	0.0%	0.0%	0.0%
Expected term (in years)	6.08	6.25	5.31-6.25	5.31-6.25
Risk-free rate	3.7%	4.2%-4.3%	3.7%-4.4%	3.5%-4.3%

The weighted average grant date fair values of stock options granted during the three months ended September 30, 2024 and 2023 were $7.56 and $3.28, and the weighted average grant date fair values of stock options granted during the nine months ended September 30, 2024 and 2023 were $6.96 and $5.04, respectively.

The Company recognized $1.6 million and $0.9 million of equity-based compensation expense in connection with stock options during the three months ended September 30, 2024 and 2023, respectively. During the nine months ended September 30, 2024 and 2023, the Company recognized equity-based compensation expense in connection with stock options of $3.6 million and $4.0 million, respectively. As of September 30, 2024, there was $10.5 million of total unrecognized equity-based compensation cost related to unvested stock options. This cost is expected to be recognized over a weighted average period of 2.56 years.

Restricted stock units

The following table summarizes activity with respect to RSUs during the nine months ended September 30, 2024:

	Units		Weighted-Average Grant Date Fair Value
Nonvested RSUs at January 1, 2024	877,181	$	6.37
Granted	1,124,028	$	8.03
Vested	(237,315)	$	6.69
Forfeitures	(244,607)	$	6.88
Nonvested RSUs at September 30, 2024	1,519,287	$	7.47

The Company recognized $0.8 million and $0.7 million of equity-based compensation expense in connection with RSUs during the three months ended September 30, 2024 and 2023, respectively. During the nine months ended September 30, 2024 and 2023, the Company recognized equity-based compensation expense in connection with RSUs of $2.2 million and $1.6 million, respectively. As of September 30, 2024, there was $9.4 million of total unrecognized equity-based compensation cost related to nonvested RSUs. This cost is expected to be recognized over a weighted average period of 3.08 years.

Performance stock units

In June 2024, the Board approved a grant of performance-based restricted stock unit awards (“PSUs" or “Performance Awards”) to the Company’s executive team. Each PSU represents the contingent right to receive one share of the Company’s common stock. These Performance Awards provide for the vesting of 25% of the target number of underlying RSUs granted upon the achievement of each of four independent performance milestones predetermined by the Board (“Performance Milestones”), subject to the grantee’s continued service with the Company (the “Approval Conditions”).

The Performance Milestones are tied to the achievement of certain business objectives and are non-market and non-financial in nature. The Board will determine that all Approval Conditions have been satisfied and the number of units that will ultimately vest on the 2026 Evaluation Date, which will occur in the first quarter of 2026, and the 2027 Evaluation Date, which will occur in the first quarter of 2027. A maximum of 25% of the target number of RSUs may vest at the 2026 Evaluation Date and the percentage of the target number of RSUs allocable to any Performance Milestone that has not been achieved on or prior to the 2027 Evaluation Date shall be cancelled.

The Company granted 2,165,325 PSUs during the nine months ended September 30, 2024 with a weighted average grant date fair value of $7.51 per unit. There were no PSUs granted during the three months ended September 30, 2024. During the three and nine months ended September 30, 2024, the Company recognized $0.6 million and $0.7 million in expense for these grants, respectively.

As of September 30, 2024, there was $3.3 million of unrecognized equity-based compensation cost related to nonvested PSUs based on the achievement of all Performance Milestones. The Company expects to recognize this cost over a weighted average period of 2.13 years.

9. Leases

On December 22, 2022, the Company entered into a sub-lease agreement (the "Sub-Lease") with Arkea Bio Corp ("Arkea"). The Sub-Lease permits use by Arkea of a portion of the space leased by the Company at 500 Rutherford Avenue in Charlestown, Massachusetts. The Company subleased approximately 12,461 square feet of the 49,869 square foot building interior space. The Sub-Lease term originally ended on February 28, 2025. The Sub-Lease was subsequently amended by the Sub-Lease Amendment, by and between Arkea and the Company, dated May 10, 2024, to increase the subleased area to approximately 13,714 square feet and extend the term to February 29, 2028.

During the three and nine months ended September 30, 2024, the Company recorded sublease income of $0.2 million and $0.8 million, respectively, within other income, net. During the three and nine months ended September 30, 2023, the Company recorded sublease income of $0.3 million and $0.8 million, respectively.

10. Commitments and Contingencies

Letter of credit

The Company had an outstanding letter of credit in the amount of $1.9 million and $1.8 million at September 30, 2024 and December 31, 2023, respectively, which is required as a condition of the Company’s office and laboratory lease.

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 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 of the Company. 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 condensed consolidated financial statements as of September 30, 2024 and December 31, 2023.

Legal proceedings

The Company may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which the Company is focused. The Company is not aware of any material legal proceedings or claims as of September 30, 2024.

11. Net Loss per Share

The following table sets forth the computation of the Company's basic and diluted net loss per share:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Numerator:				
Net loss	$ (32,725)	$ (20,980)	$ (82,100)	$ (75,679)
Denominator:				
Shares used to compute net loss per share, basic and diluted				
Weighted average shares of common stock outstanding	38,730,839	20,059,641	37,568,821	19,767,174
Weighted average shares of pre-funded warrants to purchase common stock	2,712,478	—	2,613,482	—
Weighted average shares of common stock outstanding used to compute basic and diluted net loss per share	41,443,317	20,059,641	40,182,303	19,767,174
Net loss per share, basic and diluted	$ (0.79)	$ (1.05)	$ (2.04)	$ (3.83)

Included within weighted average shares of common stock outstanding for the three and nine months ended September 30, 2024 are 2,712,478 shares of common stock issuable upon the exercise of the pre-funded warrants as the pre-funded warrants are exercisable at any time for nominal consideration, and as such, the shares are considered outstanding for the purpose of calculating basic and diluted net loss per share. There were no pre-funded warrants issued and outstanding during the three and nine months ended September 30, 2023.

The outstanding securities presented below were excluded from the calculation of net loss per share because the inclusion of such securities would have been anti-dilutive due to the Company’s net loss per share during the periods presented.

	Three and Nine Months Ended September 30,	
	2024	2023
Options to purchase common stock	2,941,043	2,189,102
Nonvested restricted stock units	1,519,192	946,182
Nonvested performance stock units	2,165,325	—
Shares subject to employee stock purchase plan	244,302	—
Warrants	9,230	—
Total	6,879,092	3,135,284

Item 2. 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 unaudited condensed consolidated financial statements and related notes appearing elsewhere in this quarterly report on Form 10-Q and our audited financial statements and related notes for the year ended December 31, 2023 included in our annual report filed on Form 10-K on March 13, 2024.

Some of the statements contained in this discussion and analysis or set forth elsewhere in this quarterly report on Form 10-Q, including information with respect to our plans and strategy for our business, constitute forward looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended. We have based these forward-looking statements on our current expectations and projections about future events. The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this quarterly report on Form 10-Q particularly including those risks identified in Part II, Item 1A "Risk Factors" and our other filings with the Securities and Exchange Commission, or the SEC.

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this quarterly report on Form 10-Q. Statements made herein are made as of the date of the filing of this Form 10-Q with the SEC and should not be relied upon as of any subsequent date. Even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this quarterly report on Form 10-Q, they may not be predictive of results or developments in future periods. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made.

Overview

Solid Biosciences Inc. (we, us, our, the Company, or Solid) is 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, or BAG3, additional assets for the treatment of cardiac diseases, platform technologies and know-how related thereto.

We are continuing to advance our pipeline of Candidates. The U.S. Food and Drug Administration, or the FDA, has granted orphan drug designation to SGT-003 for the treatment of Duchenne, and the FDA and the European Medicines Agency, or EMA, have granted orphan drug designation to SGT-501 for the treatment of CPVT. We expect to submit an Investigational New Drug, or IND,

application for SGT-501 for the treatment of CPVT in the first half of 2025. The FDA has also granted Rare Pediatric Disease and fast track designation for SGT-003.

Patient dosing in the Phase 1/2 INSPIRE DUCHENNE multicenter clinical trial of SGT-003 began in the second quarter of 2024, and at the time of filing, SGT-003 has been well tolerated and no Serious Adverse Events (SAEs) were observed in the patients dosed. In September 2024, we amended the INSPIRE DUCHENNE clinical trial protocol to increase the anticipated participant enrollment size, expand the participant cohort age groups, and extend the timepoints of certain secondary objective measurements. In the first quarter of 2025, we anticipate reporting initial safety, expression and biomarker data from the first patients dosed, and provide a trial update, in first quarter of 2025 following the completion and collective assessment of 90-day muscle biopsies. We plan to expand clinical development of SGT-003 in the United States and in Canada and Europe. A Clinical Trial Application for INSPIRE DUCHENNE was authorized in Canada in the second quarter of 2024. In anticipation of an expanded clinical trial, we have initiated work for additional GMP batches of SGT-003.

AAV-SLB101, Solid's proprietary capsid used in SGT-003, was well tolerated in the first patients dosed in the INSPIRE DUCHENNE trial, and was well tolerated in NHP and mouse studies. We have also transitioned our BAG3 and TNNT2 cardiac programs to AAV-SLB101.

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 genes, called transgenes. The transgenes are then utilized by the body to produce desired proteins to compensate for mutated genes, 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 $32.7 million and $82.1 million for the three and nine months ended September 30, 2024 and $21.0 million and $75.7 million for the three and nine months ended September 30, 2023, respectively. As of September 30, 2024, we had an accumulated deficit of $740.9 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 September 30, 2024, we had cash, cash equivalents, and available-for-sale securities of $171.1 million, excluding restricted cash of $1.9 million. We believe that our cash, cash equivalents, and available-for-sale securities as of September 30, 2024 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

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

Operating expenses

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

Research and development expenses

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

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

Research and development activities are central to our business model. We are still in the early stages of development of our Candidates. Candidates in later stages of clinical development generally have higher development costs than those in preclinical development or in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will continue to increase for the foreseeable future if and as we conduct clinical trials for SGT-003, initiate clinical trials for our other Candidates, and continue to identify and develop additional Candidates.

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Allocated research and development expenses:				
SGT-001	$ 74	$ 174	$ 568	$ 3,245
SGT-003	5,288	4,735	9,629	19,642
SGT-501	3,299	174	10,529	1,824
Other development programs	7,053	1,227	12,229	4,409
Total allocated research and development expenses	15,714	6,310	32,955	29,120
Unallocated research and development expenses:				
Personnel related expenses	6,270	6,175	18,187	19,100
External expenses	5,343	4,217	14,519	12,890
Total unallocated research and development expenses	11,613	10,392	32,706	31,990
Total research and development expenses	$ 27,327	$ 16,702	$ 65,661	$ 61,110

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

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

General and administrative expenses

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

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

Other income, net

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

Critical Accounting Policies and Use of Estimates

Our discussion and analysis of our financial condition and results of operations is based on our condensed consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of our condensed consolidated financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets and liabilities, costs and expenses and the disclosure of contingent assets and liabilities in our condensed consolidated financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about

the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ materially from these estimates.

During the nine months ended September 30, 2024, there were no material changes to our critical accounting policies, except as noted in Note 1 – Nature of the Business and Basis of Presentation. Our critical accounting policies are described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical accounting policies and use of estimates" in our Annual Report on Form 10-K for the year ended December 31, 2023 and the notes to the unaudited condensed consolidated financial statements included in Part I, Item 1, "Financial Statements (unaudited)," of this quarterly report on Form 10-Q. We believe that of our critical accounting policies, the following accounting policies involve the most judgment and complexity:

- accrued research and development expenses;
- equity-based compensation; and
- derivative liabilities.

Accordingly, we believe the policies set forth above are critical to fully understanding and evaluating our financial condition and results of operations. If actual results or events differ materially from the estimates, judgments and assumptions used by us in applying these policies, our reported financial condition and results of operations could be materially affected.

Results of Operations

Comparison of the three months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the periods indicated (in thousands, except percentages):

	Three Months Ended September 30,			
	2024	2023	Change	% Change
Operating expenses:				
Research and development	$ 27,327	$ 16,702	$ 10,625	63.6%
General and administrative	7,855	6,412	1,443	22.5%
Total operating expenses	35,182	23,114	12,068	52.2%
Loss from operations	(35,182)	(23,114)	(12,068)	52.2%
Other income, net:				
Interest income	2,328	1,962	366	18.7%
Interest expense	(82)	(106)	24	(22.6)%
Other income, net	211	278	(67)	(24.1)%
Total other income, net	2,457	2,134	323	15.1%
Net loss	$ (32,725)	$ (20,980)	$ (11,745)	56.0%

Research and development expenses

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

	Three Months Ended September 30,			
	2024	2023	Change	% Change
Allocated research and development expenses:				
SGT-001	$ 74	$ 174	$ (100)	(57.5)%
SGT-003	5,288	4,735	553	11.7%
SGT-501	3,299	174	3,125	1796.0%
Other development programs	7,053	1,227	5,826	474.8%
Total allocated research and development expenses	15,714	6,310	9,404	149.0%
Unallocated research and development expenses:				
Personnel related expenses	6,270	6,175	95	1.5%
External expenses	5,343	4,217	1,126	26.7%
Total unallocated research and development expenses	11,613	10,392	1,221	11.7%
Total research and development expenses	$ 27,327	$ 16,702	$ 10,625	63.6%

Research and development expenses for the three months ended September 30, 2024 were $27.3 million, compared to $16.7 million for the three months ended September 30, 2023. The increase of $10.6 million in research and development expenses was due to a

$5.8 million increase in other development program expenses primarily due to the FA212 asset acquisition and other research costs, a $3.1 million increase in costs for SGT-501 primarily related to manufacturing and research costs, a $1.1 million increase in external expenses, a $0.6 million increase in costs for SGT-003 primarily related to clinical and manufacturing costs, and a $0.1 million increase in personnel related expenses, offset by a $0.1 million decrease in costs for SGT-001 primarily related to lower clinical and research costs due to our decision to deprioritize SGT-001.

General and administrative expenses

General and administrative expenses were $7.9 million for the three months ended September 30, 2024, compared to $6.4 million for the three months ended September 30, 2023. The increase of $1.4 million was primarily related to a $1.5 million increase in personnel related costs, and a $0.2 million increase in consulting fees, offset by a $0.3 million decrease in temporary services.

Other income, net

Other income, net was $2.5 million for the three months ended September 30, 2024, compared to $2.1 million for the three months ended September 30, 2023. The activity was primarily related to the increase in interest income on available-for-sale securities included within our portfolio due to higher investment balances from proceeds of our amended and restated at-the market offering sales agreement, dated March 13, 2024, by and between us and Jefferies LLC, or Jefferies, or the ATM Sales Agreement, and January 2024 Private Placement.

Comparison of the nine months ended September 30, 2024 and 2023

The following table summarizes our results of operations for the periods indicated (in thousands, except percentages):

	Nine Months Ended September 30,			
	2024	2023	Change	% Change
Operating expenses:				
Research and development	$ 65,661	$ 61,110	$ 4,551	7.4 %
General and administrative	24,171	20,940	3,231	15.4 %
Restructuring charges	—	(63)	63	(100.0)%
Total operating expenses	89,832	81,987	7,845	9.6 %
Loss from operations	(89,832)	(81,987)	(7,845)	9.6 %
Other income, net:				
Interest income	7,544	5,822	1,722	29.6 %
Interest expense	(265)	(339)	74	(21.8)%
Other income, net	453	825	(372)	(45.1)%
Total other income, net	7,732	6,308	1,424	22.6 %
Net loss	$ (82,100)	$ (75,679)	$ (6,421)	8.5 %

Research and development expenses

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

	Nine Months Ended September 30,			
	2024	2023	Change	% Change
Allocated research and development expenses:				
SGT-001	$ 568	$ 3,245	$ (2,677)	(82.5)%
SGT-003	9,629	19,642	(10,013)	(51.0)%
SGT-501	10,529	1,824	8,705	477.2 %
Other development programs	12,229	4,409	7,820	177.4 %
Total allocated research and development expenses	32,955	29,120	3,835	13.2 %
Unallocated research and development expenses:				
Personnel related expenses	18,187	19,100	(913)	(4.8)%
External expenses	14,519	12,890	1,629	12.6 %
Total unallocated research and development expenses	32,706	31,990	716	2.2 %
Total research and development expenses	$ 65,661	$ 61,110	$ 4,551	7.4 %

Research and development expenses for the nine months ended September 30, 2024 were $65.7 million, compared to $61.1 million for the nine months ended September 30, 2023. The increase of $4.6 million in research and development expenses was primarily due to an $8.7 million increase in costs for SGT-501 primarily related to manufacturing and research costs, a $7.8 million increase in other development program expenses primarily due to the FA212 asset acquisition and other research costs, and a $1.6 million increase in external expenses, offset by a $10.0 million decrease for SGT-003 primarily related to manufacturing and research costs, a $2.7 million decrease in costs for SGT-001 due to our decision to deprioritize SGT-001, and a $0.9 million decrease in personnel related expenses.

General and administrative expenses

General and administrative expenses were $24.2 million for the nine months ended September 30, 2024, compared to $20.9 million for the nine months ended September 30, 2023. The increase of $3.2 million was primarily related to a $2.2 million increase in personnel related costs, a $1.3 million increase in legal fees, and a $0.3 million increase in software costs, offset by a $0.6 million decrease in temporary services.

Other income, net

Other income, net was $7.7 million for the nine months ended September 30, 2024 compared to other expense of $6.3 million for the nine months ended September 30, 2023. The activity was primarily related to the increase in interest income on available-for-sale securities included within our portfolio due to higher investment balances from proceeds from our ATM Sales Agreement and the January 2024 Private Placement.

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 pre-funded 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 ATM Sales Agreement. Through September 30, 2024, we raised an aggregate of $144.6 million of gross proceeds from our sales of preferred units prior to the completion of our initial public offering, and an aggregate of $662.0 million of net proceeds from the sale of our common stock through public offerings, including our IPO and a 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 and restated in March 2024, under which we may offer and sell, from time to time, shares of our common stock through Jefferies as sales agent. Any such sales being made by any method that is deemed an “at-the-market offering” as defined in Rule 415 promulgated under the Securities Act. We will pay Jefferies a commission of up to 3% of the gross proceeds of any sales of common stock pursuant to the ATM Sales Agreement. During the year ended December 31, 2023, we sold 602,030 shares pursuant to the ATM Sales Agreement resulting in net proceeds of $3.0 million. During the three and nine months ended September 30, 2024, we sold 330,670 and 1,208,287 shares pursuant to the ATM Sales Agreement resulting in net proceeds of $3.1 million and $11.5 million, respectively.

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 $103.7 million of net proceeds from the January 2024 Private Placement after deducting offering costs.

As of September 30, 2024, we had cash, cash equivalents and available-for-sale securities of $171.1 million, excluding restricted cash of $1.9 million, and had no debt outstanding.

Cash flows

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

	Nine Months Ended September 30,	
	2024	2023
Cash used in operating activities	$ (70,367)	$ (73,357)
Cash used in investing activities	(54,653)	(35,607)
Cash provided by financing activities	115,497	2,617
Net decrease in cash, cash equivalents, and restricted cash	$ (9,523)	$ (106,347)

Operating activities

During the nine months ended September 30, 2024, operating activities used $70.4 million of cash, primarily resulting from our net loss of $82.1 million partially offset by changes in our operating assets and liabilities of $0.7 million and non-cash charges of $11.0 million. Net cash used by changes in our operating assets and liabilities during the nine months ended September 30, 2024 consisted of an increase of $2.5 million in prepaid and other assets, and a decrease in the operating lease liability of $1.3 million, offset by an increase in accrued expenses and other liabilities of $3.1 million, and an increase in accounts payable of $1.4 million. Non-cash activities were driven by equity-based compensation of $6.7 million, depreciation and amortization expense of $2.0 million, non-cash lease expense of $1.8 million, and a change in the fair value of derivative liabilities of $3.4 million, partially offset by amortization on available-for-sale securities of $2.8 million.

During the nine months ended September 30, 2023, operating activities used $73.4 million of cash, primarily resulting from our net loss of $75.7 million and changes in our operating assets and liabilities of $6.4 million, partially offset by non-cash charges of $8.7 million. Net cash used by changes in our operating assets and liabilities during the nine months ended September 30, 2023 consisted of a decrease in accrued expenses and other current and non-current liabilities of $5.3 million, a decrease in the operating lease liability of $1.3 million, and a decrease in accounts payable of $0.9 million, partially offset by a decrease in prepaid and other assets of $1.1 million. Non-cash activities were driven by equity-based compensation of $5.7 million, depreciation and impairment expense of $2.4 million, and non-cash lease expense of $1.9 million, partially offset by amortization on available-for-sale securities of $1.3 million.

Investing activities

During the nine months ended September 30, 2024, investing activities used $54.7 million of cash, resulting from the purchases of available-for sale securities of $189.0 million and the purchases of property plant and equipment of $0.5 million, partially offset by the maturity of available-for sale securities of $134.8 million.

During the nine months ended September 30, 2023, investing activities used $35.6 million of cash, resulting from the purchases of available-for sale securities of $102.8 million and the purchases of property plant and equipment of $1.4 million, partially offset by the maturity of available-for sale securities of $68.6 million.

Financing activities

During the nine months ended September 30, 2024, financing activities provided $115.5 million of cash, resulting from the net proceeds from the issuance of common stock and pre-funded warrants to purchase shares of common stock of $115.3 million, proceeds from the exercise of common stock options for $0.4 million, and proceeds from the issuance of shares of $0.1 million under the Company's 2021 Employee Stock Purchase Plan, or ESPP, offset by payments of the principal portion of finance lease obligations of $0.3 million.

During the nine months ended September 30, 2023, financing activities provided $2.6 million of cash, resulting from the proceeds from the issuance of common stock of $2.5 million and purchases of shares under the ESPP.

Funding requirements

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

- continue to enroll patients in our expanded 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 United States Food and Drug Administration, or FDA, and other regulatory authorities;
- submit regulatory filings relating to the development of our Candidates and seek marketing approvals for our Candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- scale up our manufacturing processes and arrange manufacturing for larger quantities of our Candidates for preclinical and clinical development and potential commercialization;

- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control, and scientific personnel;
- build out new facilities or expand existing facilities to support our activities;
- acquire or in-license other drugs, drug candidates, technologies, and intellectual property; and
- add operational, financial and management information systems and personnel.

As of September 30, 2024, we had cash, cash equivalents and available-for-sale securities of $171.1 million, excluding restricted cash of $1.9 million. Based on our current operating plan, we believe that our cash, cash equivalents, and available-for-sale securities as of September 30, 2024 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 Candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our Candidates. Our future capital requirements will depend on many factors, including:

- the progress, costs and results of the INSPIRE DUCHENNE trial 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 facility compliant with the current Good Manufacturing Practice regulations enforced by the FDA, or cGMPs, located at one of our CMOs. We intend to establish the capability and capacity to supply Candidates at commercial scale from multiple sources.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval for any Candidates or generate revenue from the sale of any products for which we may obtain marketing approval. In addition, our Candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if ever. Accordingly, we will need to obtain substantial additional funds to achieve our business objectives.

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

declaring dividends, which could adversely impact our ability to conduct our business, and may require the issuance of warrants, which could potentially dilute existing stockholders' ownership interests.

If we raise additional funds through licensing agreements and strategic collaborations with third parties, we may have to relinquish valuable rights to our technology, future revenue streams, research programs, or Candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds, we may be required to delay, limit, reduce and/or terminate development of our Candidates or any future commercialization efforts or grant rights to develop and market Candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

During the nine months ended September 30, 2024, other than our acquisition agreement with FA212 to make potential milestone payments and pay royalties based on achievement of specified milestones, there were no material changes to our contractual obligations and commitments from those described in "Management's Discussion and Analysis of Financial Condition and Results of Operations" in our Annual Report on Form 10-K for the fiscal year ended December 31, 2023.

Recently Issued Accounting Pronouncements

A summary of significant recent accounting pronouncements that we expect to adopt is included in Note 1 – Nature of the Business and Basis of Presentation to our condensed consolidated financial statements (See Part I, Item 1 – Financial Statements, of this Quarterly Report on Form 10-Q).

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. As of September 30, 2024, our cash equivalents consisted of money market funds, treasury bills, and government bonds that have contractual maturities of less than 90 days from the date of acquisition. As of September 30, 2024, our investments consisted of treasury bills and government bonds that have contractual maturities of less than one year. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of the investments in 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.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our President and Chief Executive Officer and our Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2024. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, means 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 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 the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2024, our President and Chief Executive Officer and our Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

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 have occurred during the three months ended September 30, 2024 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

None.

Item 1A. Risk Factors.

You should carefully consider the following risk factors, in addition to the other information contained in this Quarterly Report on Form 10-Q, 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 Quarterly Report on Form 10-Q occurs, our business, operating results and financial condition could be seriously harmed and the trading price of our common stock could decline. This Quarterly Report on Form 10-Q 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 Quarterly Report on Form 10-Q. 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.

RISK FACTOR SUMMARY

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

- We 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 clinical development, regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- 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.
- We have never completed a clinical trial and may be unable to do so for any Candidate, including SGT-003 and other Candidates.
- One or more adverse events or other undesirable side effects caused by our product Candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay, denial or withdrawal of regulatory approval by the FDA or other comparable foreign regulatory authorities.
- 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 narrower indication than we seek.
- We face significant competition and our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may adversely affect our ability to develop, successfully market or commercialize our Candidates. Changes within the competitive landscape could lead us to alter our clinical trial strategy, baseline eligibility criteria or make other modifications to clinical trial designs.
- We may not be successful in finding strategic collaborators for continuing development of our Candidates or platform technologies, or for successfully commercializing or competing in the market for certain indications.
- We have limited gene therapy manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-003, SGT-501, or other current and future Candidates. In addition, changes to manufacturing sites or processes, or formulations for our Candidates may result in additional cost or delay.
- We expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.
- Our gene transfer approach utilizes capsids derived from a virus, which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of gene transfer Candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for our Candidates.
- We heavily rely on certain in-licensed patents and other intellectual property rights in connection with our development of our Candidates and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize our Candidates.
- If we are unable to obtain and maintain patent protection for our Candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our Candidates may be adversely affected.
- Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.
- The price of our common stock has been, and in the future is likely to be, volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

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.

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 loss was $82.1 million for the nine months ended September 30, 2024. Our net losses were $96.0 million and $86.0 million for the years ended December 31, 2023 and 2022, respectively. As of September 30, 2024, we had an accumulated deficit of $740.9 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, clinical development of SGT-003, as well as building out our new management team. We expect that it could be several years before we have a commercialized product, and we may never have a commercialized product. We expect to continue to incur significant expenses and see continued operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if, and as, we:

- continue to enroll patients in our expanded 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;

- scale up our manufacturing processes and arrange manufacturing for larger quantities of our Candidates for preclinical and clinical development and potential commercialization;
- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control and scientific personnel;
- build out new facilities or expand existing facilities to support our activities;
- acquire or in-license other drugs, drug Candidates, technologies and intellectual property; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more Candidates with significant market potential. This will require us to be successful in a range of challenging activities, and our expenses will increase substantially as we continue to enroll patients in and conduct the INSPIRE DUCHENNE trial and continue to develop our pipeline and complete ongoing and planned preclinical studies and clinical trials of our Candidates, obtain marketing approval for our Candidates, develop adjacent technologies such as assays, develop and validate commercial-scale manufacturing processes, manufacture, market and sell any future Candidates for which we may obtain marketing approval and satisfy any post-marketing requirements. Moreover, the manufacturing process requires materials which may fluctuate in cost or be limited or unavailable to us, as well as relationships with contract development and manufacturing organizations to facilitate the manufacturing process. We may never succeed in any of these activities and, even if we do, we may never generate revenue that is significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause stockholders to lose all or part of their investment.

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

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, conduct clinical trials of, and seek marketing approval for our Candidates. In addition, if we obtain marketing approval for our Candidates, we expect to incur significant expenses related to product sales, marketing, manufacturing and distribution. We also expect to continue to incur additional costs associated with operating as a public company. While we believe that our cash, cash equivalents and available-for-sale securities as of September 30, 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 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 Candidates, if approved, may not achieve commercial success. Our product revenue, if any, will be derived from or based on sales of our Candidates that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all, and may be impacted by the economic climate and market conditions. Our ability to raise additional funds may be adversely impacted by general economic conditions, both inside and outside the U.S., including disruptions to, and instability and volatility in, the credit and financial markets in the U.S. and worldwide, heightened inflation, interest rate and currency rate fluctuations, and economic slowdown or recession as well as concerns related to public health emergencies or pandemics, 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 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 Investigational New Drug, or 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 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 Candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

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

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

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

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

Risks related to the development of our Candidates

Our gene transfer Candidates 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 Candidate efficacy and/or safety as required by regulatory authorities. We may fail to access relevant, adequate, or necessary animal models, including genetic models of disease and non-human primates in particular, for use in such studies as requested by regulatory authorities. We may also experience substantial delays as a result of our reliance on CROs to conduct all animal model experimentation necessary to assess the efficacy and safety of our Candidates. Any of these factors may result in delays to Candidate progression, inability to obtain regulatory approval, and/or substantial increases in Candidate development costs.

In addition, the product specifications and the clinical trial requirements of the FDA, the European Commission, the 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 clinical development, regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.

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

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

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

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

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

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

One of our prior clinical trials had been placed on clinical hold by the FDA in the past, and we cannot guarantee that similar events will not happen in 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 Candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of SGT-003 or other Candidates.

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

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

We will need to successfully initiate our planned 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 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 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. In the second quarter of 2024, patient dosing commenced in our INSPIRE DUCHENNE trial. We have not yet dosed human subjects with any of our other current 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 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 Candidate for its intended indications. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in obtaining animals in sufficient quantities to run our preclinical studies;
- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement with the appropriate external parties on dose escalation;
- delays in enrolling patients in clinical trials;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board or independent ethics committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials, including because such trials have restrictive eligibility criteria or may be placebo-controlled trials and patients are not guaranteed to receive treatment with our Candidates, or as a result of alternative therapies or competing trials;
- difficulty in finding suitable animal models to demonstrate a disease specific phenotype;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with FDA good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union and other countries;
- delays in the testing, validation, manufacturing and delivery of our Candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in subjects completing participation in a trial or returning for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event, or after an inspection of our clinical trial operations, trial sites or manufacturing facilities or otherwise;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- delays as a result of public health emergencies or pandemics, 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, or DAP, for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. In June 2024, as mandated by FDORA, the FDA issued draft guidance outlining the general requirements for DAPs. Unlike most guidance documents issued by the FDA, the DAP guidance when finalized will have the force of law because FDORA specifically dictates that the form and manner for submission of DAPs are specified in FDA guidance. 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 Candidates to earlier versions. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, which we have done in the past and which could result in delays. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our Candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our Candidates.

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

We do not have the personnel capacity to conduct or manage the clinical trials that will be necessary for the development of our Candidates. For our INSPIRE DUCHENNE trial we are relying, and for any 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 our other Candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-003 and our other Candidates are critical to our success. Because of our primary focus on rare diseases, we may have difficulty enrolling a sufficient number of eligible patients. The timing of any clinical trials depends on our ability to recruit patients to participate as well as complete required follow-up periods. If patients are

unwilling or unable to participate in our gene therapy clinical trials, including because of negative publicity from adverse events related to our Candidates, other approved therapies, or due to competitive clinical trials or approvals for similar patient populations, clinical trials in products employing our capsid or our platform or for other reasons, the timeline for recruiting patients, conducting clinical trials and obtaining regulatory approval of SGT-003 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 Candidate under study;
- perceived risks and benefits of gene therapy-based approaches to the treatment of diseases;
- release or disclosure of data from our completed or ongoing clinical trials;
- availability of competing therapies and clinical trials;
- severity of the disease;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians;
- ability to monitor subjects adequately during and after treatment; and
- in the case of pivotal trials, the risk that patients may opt not to enroll because they are not assured treatment with our Candidate.

If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies governing clinical trials, our development plans may be impacted. For example, in December 2022, with the passage of FDORA, Congress required sponsors to develop and submit a diversity action plan for each Phase 3 clinical trial or any other "pivotal study" of a new drug or biological product. These plans are meant to encourage the enrollment of more diverse patient populations in late-stage clinical trials of FDA-regulated products. 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 narrower indication than we seek.

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

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

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

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

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

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;

- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions or the imposition of civil or criminal penalties; or
- litigation involving patients using our products.

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

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

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

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

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

The regulations relating to the promotion of products for unapproved uses are complex and subject to substantial interpretation by the FDA, EMA, Medicines and Healthcare products Regulatory Agency, or MHRA, and other government agencies. In September 2021, the FDA published final regulations which describe the types of evidence that the agency will consider in determining the intended use of a drug product. Physicians may nevertheless prescribe our products off-label to their patients in a manner that is inconsistent with the approved label. We intend to implement compliance and training programs designed to ensure that our sales and marketing practices comply with applicable regulations. Notwithstanding these programs, the FDA or other government agencies may allege or

find that our practices constitute prohibited promotion of our products for unapproved uses. We also cannot be sure that our employees will comply with company policies and applicable regulations regarding the promotion of products for unapproved uses.

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

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

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

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

Even if we receive FDA approval of one or more of our Candidates in the United States, approval of a Candidate in the United States by the FDA does not ensure approval of such Candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Future sales of our Candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approval. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. If we submit a marketing authorization application, or MAA, to the EMA for approval of

SGT-003 or other Candidates in the European Union, obtaining such approval from the European Commission following the opinion of the EMA is a lengthy and expensive process. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our Candidates in certain countries.

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

Additionally, we could face heightened risks with respect to obtaining marketing authorization in the United Kingdom as a result of the withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. The United Kingdom is no longer part of the European Single Market and EU Customs Union. As of January 1, 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 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 Candidates that receive marketing approval outside the United States, including tariffs, trade barriers and regulatory requirements; economic weakness, including inflation, or political instability in particular foreign economies and markets; compliance with tax, employment, immigration and labor laws for employees living or traveling abroad; foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country; and workforce uncertainty in countries where labor unrest is more common than in the United States.

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

Regulatory requirements governing gene therapy products have changed frequently and will likely continue to change in the future. Moreover, there is substantial, and sometimes uncoordinated, overlap in those responsible for regulation of gene therapy products. For example, in the United States, the FDA has established the Office of Therapeutic Products within the Center for Biologics Evaluation and Research, or the CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Gene therapy clinical trials may also be subject to review and oversight by an institutional biosafety committee, a local institutional committee that reviews and oversees basic and clinical research conducted at the institution participating in the clinical trial. Although the FDA decides whether individual gene therapy protocols may proceed, the review process and determinations of other reviewing bodies can impede or delay the initiation of a clinical trial, even if the FDA has reviewed the trial and approved its initiation.

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

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

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

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

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

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

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

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

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

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

The FDA and Congress may further reevaluate the Orphan Drug Act and its regulations and policies. This may be particularly true in light of a decision from the Court of Appeals for the 11th Circuit in September 2021 finding that, for the purpose of determining the scope of exclusivity, the term “same disease or condition” means the designated “rare disease or condition” and could not be interpreted by the FDA to mean the “indication or use.” Thus, the Court of Appeals concluded that orphan drug exclusivity applies to the entire designated disease or condition rather than the “indication or use.” Although there have been legislative proposals to overrule this decision, they have not been enacted into law. In January 2023, the FDA announced that, in matters beyond the scope of

that court order, FDA will continue to apply its existing regulations tying orphan-drug exclusivity to the uses or indications for which the orphan drug was approved. We do not know if, when, or how the FDA or Congress may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA or Congress may make to its orphan drug regulations and policies, our business could be adversely impacted.

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

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

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

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

We may seek approval of one or more of our Candidates using the FDA's accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate an effect on a surrogate or intermediate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA or other applicable regulatory agency makes the determination regarding whether a surrogate or intermediate endpoint is reasonably likely to predict long-term clinical benefit. Given that expression of microdystrophin has not yet been established to predict long-term clinical benefit, it is not currently accepted, and it is possible the FDA and/or other applicable regulatory agencies could decide never to accept it, as a surrogate endpoint for the accelerated approval pathway for the treatment of Duchenne.

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

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

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

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

We may seek a regenerative medicine advanced therapy designation for some of our Candidates. A regenerative medicine advanced therapy is defined as cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues may meet the definition of a regenerative medicine therapy. The regenerative medicine advanced therapy program is intended to facilitate efficient development and expedite review of regenerative medicine advanced therapies, which are intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition. A BLA for a regenerative medicine advanced therapy may be eligible for priority review or accelerated approval through (1) surrogate or intermediate endpoints reasonably likely to predict long-term clinical benefit or (2) reliance upon data obtained from a meaningful number of sites. Benefits of such designation also include early interactions with the FDA to discuss any potential surrogate or intermediate endpoint to be used to support accelerated approval. A regenerative medicine therapy that is granted accelerated approval and is subject to post-approval requirements may fulfill such requirements through the submission of clinical evidence, clinical 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 Candidates meets the criteria for designation as a regenerative medicine advanced therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a regenerative medicine advanced therapy designation for a Candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our Candidates qualify as regenerative medicine advanced therapies, the FDA may later decide that the biological products no longer meet the conditions for qualification.

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

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

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

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

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

In order to receive a priority review voucher upon BLA approval, the product must receive designation from the FDA as a product for a rare pediatric disease prior to approval of the marketing application. A "rare pediatric disease" is a disease that is serious or

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

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

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

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

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

If a therapy is intended for the treatment of a serious condition and 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 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 Candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

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

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

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

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

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

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

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

We are aware of a number of companies and research institutions developing gene transfer programs progressing in Duchenne. For example, in June 2023, Sarepta Therapeutics, Inc., or Sarepta, announced that it had received accelerated approval for its gene therapy candidate ELEVIDYS for the treatment of ambulatory pediatric patients aged 4 through 5 years with Duchenne. In 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. On June 20, 2024, Sarepta announced that the FDA approved an expansion to the labeled indication for ELEVIDYS to include a full approval for the treatment of ambulatory Duchenne patients who are at least 4 years of age. The FDA also granted accelerated approval for non-ambulatory patients aged 4 and older. We are also aware of several companies and research institutions conducting clinical trials of product candidates focused on systemic gene transfers for Duchenne, including Genethon with a product candidate currently being evaluated in a Phase 1/2/3 clinical trial, REGENXBIO Inc. with a product candidate in Phase 1/2 clinical development. Pfizer Inc., or Pfizer had a product candidate in Phase 3 clinical development. On June 12, 2024, Pfizer announced that its candidate did not meet the primary endpoint or demonstrate significance in key secondary endpoints of the trial and on July 30, 2024, announced it had discontinued its DMD program. We are also aware of several companies and research institutions conducting clinical trials in small molecule product candidates focused on CPVT, including Armgo Pharmaceuticals, Inc. with an orally administered Rycal in a Phase 2 clinical trial and Cardurion Pharmaceuticals, Inc. with an orally administered CAMKII-delta inhibitor candidate in a Phase 2 clinical trial.

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

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

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

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

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 Candidates. If we do not accurately evaluate the commercial potential for a particular Candidate, we may relinquish valuable rights to that Candidate through strategic collaborations, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such Candidate. Alternatively, we may allocate internal resources to a Candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. If any of these events occur, we may be forced to abandon our development efforts with respect to a particular Candidate or fail to develop a potentially successful Candidate.

Risks related to the manufacturing and commercialization of our Candidates

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

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 Candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may not pursue commercialization of any Candidates that achieve regulatory approval or may elect not to continue or renew commercialization programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that may divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a Candidate, repeat or conduct new clinical trials or require a new formulation of a Candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding Candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such Candidates on a discretionary basis;

- collaborators, including Ultragenyx, could develop products that compete directly or indirectly with our Candidates and products pursuant to the collaboration;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our Candidates and products if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- Candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our Candidates;
- a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a Candidate or product;
- a collaborator with marketing and distribution rights to one or more of our Candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over intellectual property or proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of Candidates, might lead to additional responsibilities for us with respect to Candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly obtain, maintain, enforce, defend or protect our intellectual property or proprietary rights or may use our proprietary information in such a way as to potentially lead to disputes or legal proceedings that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property or proprietary rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable Candidates.

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

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

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

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

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

We have limited experience manufacturing SGT-003, SGT-501 and our other current or Candidates. The manufacturing process we have used historically and the manufacturing process we plan to use in the future to produce product for our Candidates are complex and our processes have not been validated for commercial use. As Candidates progress through preclinical studies and clinical trials to marketing approval and commercialization, it is common that various aspects of the development program, such as manufacturing methods and formulation, are altered along the way in an effort to enhance safety, quality, efficacy, yield, manufacturing batch size, minimize costs and achieve consistent results. For example, we have moved to a transient transfection-based manufacturing process for SGT-003 and while we have observed positive results in preclinical and clinical 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 studies to date. Similarly, in the future we may further alter our existing process or introduce an alternative process or formulation of one or more of our Candidates during the course of our planned preclinical studies or clinical trials. Such changes carry the risk that they will not achieve these intended objectives. Any of these changes could cause our Candidates to perform differently and affect the results of planned clinical trials or other future clinical trials conducted with the altered materials. This could delay initiation or completion of clinical trials, require the conduct of bridging studies or clinical trials or the repetition of one or more studies or clinical trials, increase development costs, delay approval of our Candidates and jeopardize our ability to commercialize our Candidates, if approved, and generate revenue.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Even with the requisite approvals from the FDA in the United States, the European Commission in the European Union and other regulatory authorities internationally, the commercial success of our Candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and, in particular for each of our current and future Candidate, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community due to ethical, social, medical and legal concerns. If our products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, our current and future Candidates, if approved for commercial sale, will depend on multiple factors, including:

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

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

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

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

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

In particular, our success will depend upon physicians who specialize in the treatment of our pipeline indications, prescribing treatments that involve the use of viral capsids in lieu of, or in addition to, other treatments with which they are more familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion may delay or impair the development and commercialization or demand for any Candidate we may develop. A public backlash developed against gene therapy following the death of a patient in 1999 during a gene therapy clinical trial of research subjects with ornithine transcarbamylase, or OTC, deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene. The death of the clinical trial subject was due to complications of adenovirus capsid administration. Dr. James M. Wilson, former chair of our Scientific Advisory Board, was a co-investigator of the 1999 trial while he was Director of the Institute for Human Gene Therapy of the University of Pennsylvania. Serious adverse events in our clinical trials, including the events that led to the previously-lifted clinical holds on IGNITE DMD or other clinical trials involving gene transfer products or our competitors' products, even if not ultimately attributable to the relevant Candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our Candidates, stricter labeling requirements for our Candidates, if approved, and a decrease in demand for our Candidates.

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

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

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

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

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

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

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

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

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

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

Additionally, in countries where the pricing of gene therapy products is subject to governmental control, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. Political, economic and regulatory developments may further complicate pricing negotiations, and pricing negotiations may continue after reimbursement has

been obtained. Reference pricing used by various European Union member states and parallel distribution, or arbitrage between low-priced and high-priced member states, can further reduce prices. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our Candidate to other available therapies. Reimbursement of our products may be unavailable or limited in scope or amount, which would adversely affect our revenue, if any.

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

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

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

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

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

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

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management's attention from our existing Candidates and initiatives in pursuing such acquisition or strategic collaboration;
- retention of key employees, the loss of key personnel and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or collaboration or even to offset transaction costs.

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

Risks related to our business operations

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

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

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

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 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 Candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

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

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. This could include violations of the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards, regulations, guidance or codes of conduct. If any such

actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

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

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

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

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

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

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, Congress repealed the "individual mandate." The repeal of this provision of the Health Care Reform Law, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the Health Care Reform Law is an essential and inseverable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the TCJA, the remaining provisions of the Health Care Reform Law are invalid as well. The U.S. Supreme Court heard this case on November 10, 2020 and on June 17, 2021, dismissed this action after finding that the plaintiffs do not have standing to challenge the constitutionality of the statute. It is unclear how such litigation and other efforts to repeal and replace the Health Care Reform Law will impact the

Health Care Reform Law and our business. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

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 HHS 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 costs and 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 drug products. At the federal level, Congress and the current administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. For example, the former 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, the HHS and the FDA published a final rule allowing states and other entities to develop a Section 804 Importation Program to import certain prescription drugs from Canada into the United States. That regulation was challenged in a lawsuit by the Pharmaceutical Research and Manufacturers of America, or PhRMA, but the case was dismissed by a federal district court in February 2023 after the court found that PhRMA did not have standing to sue HHS. Several states have passed laws allowing for the importation of drugs from Canada. Certain of these states have submitted Section 804 Importation Program proposals and are awaiting FDA approval. Vermont has submitted a concept letter to HHS. 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 Inflation Reduction Act, or IRA.

The IRA has implications for Medicare Part D, which is a program available to individuals who are entitled to Medicare Part A or enrolled in Medicare Part B to give them the option of paying a monthly premium for outpatient prescription drug coverage. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), with prices that can be negotiated subject to a cap; imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (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. The first cycle of negotiations for the Medicare Drug Price Negotiation Program commenced in the summer of 2023. On August 15, 2024, HHS published the results of the first Medicare drug price negotiations for ten selected drugs that treat a range of conditions, including diabetes, chronic kidney disease, and rheumatoid arthritis. The prices of these ten drugs will become effective January 1, 2026. On October 2, 2024, in final guidance, CMS indicated that it will announce the selection of up to 15 additional drugs covered by Part D for the second cycle of negotiations by February 1, 2025. This second cycle of negotiations with participating drug companies will occur during 2025, and any negotiated prices for this second set of drugs will be effective starting January 1, 2027.

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. There have been various decisions by the courts considering these cases since they were filed. HHS has generally won the substantive disputes in these cases, and various federal district court judges have expressed skepticism regarding the merits of the legal arguments being pursued by the pharmaceutical industry. Certain of these cases are now on appeal. We expect that litigation involving these and other provisions of the IRA will continue with unpredictable and uncertain results. Accordingly, while it is currently unclear how the IRA will be effectuated, we cannot predict with certainty what impact any federal or state health reforms will have on us, but such changes could impose new or more stringent regulatory requirements on our activities or result in reduced reimbursement for our products, any of which could adversely affect our business, results of operations and financial condition.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, if approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our Candidates or additional pricing pressures.

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

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

Finally, in the European Union, similar political, economic and regulatory developments may affect our ability to profitably commercialize our Candidates, if approved. In addition to continuing pressure on prices and cost containment measures, legislative developments at the European Union or member state level may result in significant additional requirements or obstacles that may increase our operating costs. The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of healthcare and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our Candidates, restrict or regulate post-approval activities and affect our ability to commercialize our Candidates, if approved.

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

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

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

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

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

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

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

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

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

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

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

In 2018, California passed into law the California Consumer Privacy Act, or CCPA, which took effect on January 1, 2020 and imposed many requirements on businesses that process the personal information of California residents. Many of the CCPA's requirements are similar to those found in the General Data Protection Regulation, or GDPR, including requiring businesses to provide notice to data subjects regarding the information collected about them and how such information is used and shared, and providing data subjects the right to request access to such personal information and, in certain cases, request the erasure of such personal information. The CCPA also affords California residents the right to opt-out of "sales" of their personal information. The CCPA

contains significant penalties for companies that violate its requirements. In November 2020, California voters passed a ballot initiative for the California Privacy Rights Act, or CPRA, which went into effect on January 1, 2023 and significantly expanded the CCPA to incorporate additional GDPR-like provisions including requiring that the use, retention, and sharing of personal information of California residents be reasonably necessary and proportionate to the purposes of collection or processing, granting additional protections for sensitive personal information, and requiring greater disclosures related to notice to residents regarding retention of information. The CPRA also created a new enforcement agency – the California Privacy Protection Agency – whose sole responsibility is to enforce the CPRA and other California privacy laws, which will further increase compliance risk. The provisions in the CPRA may apply to some of our business activities. In addition to California, at least eighteen 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.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Risks related to our intellectual property

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

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

Under our existing license agreements, we do not have, and under future license agreements we may not have, the right to control the preparation, filing and prosecution of patent applications, or the maintenance, enforcement and defense of the patents and patent applications that we license from third parties. For example, under our inbound license agreements with the University of Missouri and the University of Washington, each of the applicable licensors controls the prosecution of patent applications and the maintenance of patents and patent applications. Therefore, we cannot be certain that the licensed patents and applications will be prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to maintain, enforce or defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our Candidates that are the subject of such licensed rights could be adversely affected. For more information, see Part I, Item 1, "Business—Strategic partnerships and collaborations/licenses" of our Annual Report on Form 10-K for the year ended December 31, 2023 and Note 3 to the unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

Moreover, licenses to additional third-party intellectual property, technology and materials may be required for our development programs but may not be available in the future or may not be available on commercially reasonable terms. For example, third parties may claim that the constructs containing the gene or protein of interest and the AAV capsids we are developing for use in product candidates are covered by patents held by them. We believe that we would have valid defenses to any such claims; however, if any such claims were ultimately successful, we might require a license to continue to use and sell Candidates and such AAV capsids. Such licenses may not be available on commercially reasonable terms, or at all. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign 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 Candidates or will effectively prevent others from commercializing competitive products. Further, each of the provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of each provisional patent application. If we do not timely file a non-provisional patent application in respect of a provisional patent application, we may lose our priority date with respect to such provisional patent application and any patent protection on the inventions disclosed in such provisional patent application. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether such future patent applications will result in the issuance of patents that effectively protect any of our Candidates or will effectively prevent others from commercializing competitive products.

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

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

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

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

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

It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Although we enter into non-disclosure and confidentiality agreements with parties who have access to confidential or patentable aspects of our research and development output, such as our employees, corporate collaborators, outside scientific

collaborators, CROs, contract manufacturers, consultants, advisors and other third parties, any of these parties may breach the agreements and disclose such output before a patent application is filed, thereby jeopardizing our ability to seek patent protection.

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

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

Moreover, the coverage claimed in a patent application can be significantly reduced before the patent is issued, and its scope can be reinterpreted after issuance. Even if patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Any patents that we license or may own in the future may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our Candidates will be protectable or remain protected by valid and enforceable patents. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative products in a non-infringing manner.

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

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

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

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

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

Even if they are unchallenged, the patents and pending patent applications included in our patent rights may not provide us with any meaningful protection or prevent competitors from designing around our patent claims to circumvent our patent rights by developing similar or alternative therapeutics in a non-infringing manner. For example, a third party may develop a competitive therapeutic that provides benefits similar to one or more of our Candidates but that uses a capsid or an expression construct that falls outside the scope of our patent protection. If the patent protection provided by the patents and patent applications we license or pursue with respect to our Candidates is not sufficiently broad to impede such competition, our ability to successfully commercialize our Candidates could be negatively affected.

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

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

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

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

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

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

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

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

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 Candidates. Disputes may arise regarding intellectual property subject to our licensing agreements, including:

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

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

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

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

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

It is also possible that we have failed to identify relevant third-party patents or applications for which we may need a license to develop and commercialize our Candidates. For example, applications filed before November 29, 2000, and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our Candidates because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our Candidates. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale or use of a current or future Candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our Candidates.

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

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

claims that we have done so, could prevent us from manufacturing and commercializing our Candidates or force us to cease some or all of our business operations.

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

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

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

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

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

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

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

manufacture substantially in the United States or that under the circumstances domestic manufacture is not commercially feasible. This preference for U.S. manufacturers may limit our ability to contract with non-U.S. product manufacturers for products covered by such intellectual property. To the extent any of our current or future intellectual property is generated through the use of U.S. government funding, the provisions of the Bayh-Dole Act may similarly apply.

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

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

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property rights, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents, if pursued and obtained, or the marketing of competing products in violation of our intellectual property and proprietary rights generally. Proceedings to enforce our intellectual property and proprietary rights in foreign jurisdictions could (i) result in substantial costs and divert our efforts and attention from other aspects of our business, (ii) put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and (iii) provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

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

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

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

In addition, our trade secrets may otherwise become known or be independently discovered by competitors. Competitors could purchase our Candidates, if approved, and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe, misappropriate or otherwise violate our intellectual property rights, design around our protected know-how and trade secrets, or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to

prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If our trade secrets are not adequately protected so as to protect our market against competitors' products and technologies, our competitive position could be adversely affected.

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

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

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. Moreover, even when we obtain agreements assigning intellectual property to us, the assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property. Moreover, individuals executing agreements with us may have preexisting or competing obligations to a third party, such as an academic institution, and thus an agreement with us may be ineffective in perfecting ownership of inventions developed by that individual.

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

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

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

genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent-eligible subject matter, but that complementary DNA may be patent-eligible.

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

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

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

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

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

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

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

related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources.

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

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

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

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

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, was enacted as part of the Health Care Reform Law to establish an abbreviated pathway for the approval of biosimilar and interchangeable biological products. The regulatory pathway establishes legal authority for the FDA to review and approve biosimilar biologics, including the possible designation of a biosimilar as "interchangeable" based on its similarity to an approved biologic. Under the BPCIA, a reference biological product is granted 12 years of 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 Candidates we develop as a biological product under a BLA should qualify for the 12-year period of exclusivity. However, there is a risk that this exclusivity could be shortened due to congressional action or otherwise, or that the FDA will not consider the subject Candidates to be reference products for competing products, potentially creating the opportunity for biosimilar competition sooner than anticipated. Moreover, the extent to which a biosimilar, once approved, will be substituted for any

one of the reference products in a way that is similar to traditional generic substitution for non-biological products will depend on a number of marketplace and regulatory factors that are still developing. Nonetheless, the approval of a biosimilar to our Candidates would have a material adverse impact on our business due to increased competition and pricing pressure.

Risks related to ownership of our common stock

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

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

This concentration of voting power may:

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

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

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

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

In 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 Candidates, or our clinical development programs and our commercialization efforts;
- the results of our efforts to discover, develop, acquire or in-license additional Candidates;
- actual or anticipated changes in our development timelines;
- our ability to raise additional capital;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our Candidates;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of health care payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions;
- the liquidity for our stock and daily share volumes transacted;
- our ability to maintain our listing on the Nasdaq Global Select Market; and
- the other factors described in this “Risk Factors” section.

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

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

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

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

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

We are a smaller reporting company, and we will remain a smaller reporting company so long as the market value of our common stock held by non-affiliates is less than $250 million measured on the last business day of our second fiscal quarter, or our annual revenues are more than $100 million during the most recently completed fiscal year and the market value of our common stock held by non-affiliates is more than $700 million measured on the last business day of our second fiscal quarter. Smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being permitted to provide only two years of audited financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations"; 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 of the Sarbanes-Oxley Act, or Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain a smaller reporting company with less than $100 million in 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 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Securities

We did not sell any securities that were not registered under the Securities Act during the three months ended September 30, 2024.

Item 5. Other Information.

From time to time, our directors and officers (as defined in Rule 16a-1(f) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) engage in open-market transactions with respect to Company securities, including to satisfy tax withholding obligations when equity awards vest or are exercised, and for diversification or other personal reasons.

Transactions in Company securities by directors and officers are required to be made in accordance with our insider trading policy, which requires that the transactions be in accordance with applicable U.S. federal securities laws that prohibit trading while in possession of material nonpublic information. Rule 10b5-1 under the Exchange Act provides an affirmative defense that enables directors and officers to prearrange transactions in the Company's securities in a manner that avoids concerns about initiating transactions while in possession of material nonpublic information.

The following table describes, for the third quarter of 2024, each trading arrangement for the sale or purchase of Company securities adopted or terminated by our directors and officers that is either (1) a contract, instruction or written plan intended to satisfy the

affirmative defense conditions of Rule 10b5-1(c) (a "Rule 10b5-1 trading arrangement") or (2) a "non-Rule 10b5-1 trading arrangement" (as defined in Item 408(c) of Regulation S-K):

Name (Title)	Action Taken (Date of Action)	Type of Trading Arrangement	Nature of Trading Arrangement	Duration of Trading Arrangement	Aggregate Number of Securities
Gabriel Brooks, M.D. *(Chief Medical Officer)*	Adoption (August 15, 2024)	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions relating to all equity awards that have or may be granted	Sale	Until final settlement of any covered Restricted Stock Unit ("RSU")	Indeterminable(1)
Alexander Cumbo *(President, Chief Executive Officer and Director)*	Adoption (August 18, 2024)	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions relating to all equity awards that have or may be granted	Sale	Until final settlement of any covered RSU	Indeterminable(1)
Jessie Hanrahan Ph. D. *(Chief Regulatory Officer)*	Adoption (August 16, 2024)	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions relating to all equity awards that have or may be granted	Sale	Until final settlement of any covered RSU	Indeterminable(1)
Paul Herzich *(Chief Technology Officer)*	Adoption (August 16, 2024)	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions relating to all equity awards that have or may be granted	Sale	Until final settlement of any covered RSU	Indeterminable(1)
Kevin Tan *(Chief Financial Officer and Treasurer)*	Adoption (August 15, 2024)	Durable Rule 10b5-1 trading arrangement for sell-to-cover transactions relating to all equity awards that have or may be granted	Sale	Until final settlement of any covered RSU	Indeterminable(1)

(1) The number of shares subject to covered RSUs that will be sold to satisfy applicable tax withholding obligations upon vesting is unknown as the number will vary based on the extent to which vesting conditions are satisfied, the market price of the Company's common stock at the time of settlement and the potential future grant of additional RSUs subject to this arrangement. This trading arrangement, which applies to RSUs whether vesting is based on the passage of time and/or the achievement of performance goals, provides for the automatic sale of shares that would otherwise be issuable on each settlement date of a covered RSU in an amount sufficient to satisfy the applicable withholding obligation, with the proceeds of the sale delivered to the Company in satisfaction of the applicable withholding obligation.

Item 6. Exhibits.

Exhibit Number	Description
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1**	Certification of Principal Executive Officer 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 Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema Document
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)

* Filed herewith.
** Furnished herewith.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Solid Biosciences Inc.

Date: November 6, 2024

By: /s/ Alexander Cumbo

Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 6, 2024

By: /s/ Kevin Tan

Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)

Exhibit 31.1

Certification of Principal Executive Officer pursuant to Exchange Act Rules 13a-14(a) and 15d-14(a), as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002

I, Alexander Cumbo, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Solid Biosciences Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

    a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

    b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

    c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

    d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

    a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

    b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ Alexander Cumbo

Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)
Dated: November 6, 2024

Exhibit 31.2

Certification of Principal Financial Officer pursuant to Exchange Act Rules 13a-14(a) and 15d-14(a), as adopted pursuant to Section 302 of Sarbanes-Oxley Act of 2002

I, Kevin Tan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Solid Biosciences Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

/s/ Kevin Tan

Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)
Dated: November 6, 2024

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Solid Biosciences Inc. (the "Company") for the quarter ended September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Alexander Cumbo, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 6, 2024

/s/ Alexander Cumbo

Alexander Cumbo
President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report on Form 10-Q of Solid Biosciences Inc. (the "Company") for the quarter ended September 30, 2024 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Kevin Tan, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to his knowledge:

(1) the Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 6, 2024

/s/ Kevin Tan

Kevin Tan
Chief Financial Officer
(Principal Financial and Accounting Officer)