

**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, 2021

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

(State or other jurisdiction of
incorporation or organization)

141 Portland Street, Fifth Floor

Cambridge, MA

(Address of principal executive offices)

90-0943402

(I.R.S. Employer
Identification No.)

02139

(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, 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

Non-accelerated filer

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, 2021, the registrant had 110,295,600 shares of common stock, \$0.001 par value per share, outstanding.

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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, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	\$ 109,676	\$ 154,744
Available-for-sale securities	120,084	—
Prepaid expenses and other current assets	8,366	4,157
Restricted cash, current	90	—
Accounts receivable - related party	594	—
Total current assets	238,810	158,901
Property and equipment, net	6,886	8,153
Operating lease, right-of-use assets	1,218	3,579
Other non-current assets	—	209
Restricted cash, excluding current portion	2,072	327
Total assets	<u>\$ 248,986</u>	<u>\$ 171,169</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 4,148	\$ 3,274
Accrued expenses	8,003	8,640
Operating lease liabilities	1,227	1,999
Finance lease liabilities	226	208
Other current liabilities	73	—
Deferred revenue - related party	8,865	10,359
Total current liabilities	22,542	24,480
Operating lease liabilities, excluding current portion	468	2,419
Finance lease liabilities, excluding current portion	353	525
Deferred revenue - related party, excluding current portion	2,260	10,359
Other non-current liabilities	—	1,300
Total liabilities	<u>25,623</u>	<u>39,083</u>
Commitments and contingencies		
Preferred stock, \$0.001 par value; 10,000,000 shares authorized at September 30, 2021 and December 31, 2020; no shares issued and outstanding at September 30, 2021 and December 31, 2020	—	—
Common stock, \$0.001 par value; 300,000,000 shares authorized at September 30, 2021 and December 31, 2020; 110,295,600 shares issued and outstanding at September 30, 2021 and 84,893,994 shares issued and outstanding at December 31, 2020; 2,158,329 pre-funded warrants outstanding at September 30, 2021 and December 31, 2020	112	87
Additional paid-in capital	681,420	536,568
Accumulated other comprehensive loss	(22)	—
Accumulated deficit	(458,147)	(404,569)
Total stockholders' equity	223,363	132,086
Total liabilities and stockholders' equity	<u>\$ 248,986</u>	<u>\$ 171,169</u>

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)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2021</u>	<u>2020</u>	<u>2021</u>	<u>2020</u>
Collaboration revenue - related party	\$ 3,537	\$ —	\$ 10,466	\$ —
Operating expenses:				
Research and development	14,425	16,045	44,144	49,158
General and administrative	7,143	5,181	19,924	15,957
Restructuring charges	—	—	—	1,944
Total operating expenses	<u>21,568</u>	<u>21,226</u>	<u>64,068</u>	<u>67,059</u>
Loss from operations	<u>(18,031)</u>	<u>(21,226)</u>	<u>(53,602)</u>	<u>(67,059)</u>
Other income (expense), net	48	(20)	24	132
Net loss	<u>\$ (17,983)</u>	<u>\$ (21,246)</u>	<u>\$ (53,578)</u>	<u>\$ (66,927)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.16)</u>	<u>\$ (0.44)</u>	<u>\$ (0.51)</u>	<u>\$ (1.39)</u>
Weighted average shares of common stock outstanding, basic and diluted	<u>112,312,970</u>	<u>48,295,468</u>	<u>104,706,800</u>	<u>48,172,686</u>

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)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2021</u>	<u>2020</u>	<u>2021</u>	<u>2020</u>
Net loss	\$ (17,983)	\$ (21,246)	\$ (53,578)	\$ (66,927)
Other comprehensive loss:				
Unrealized loss on available-for-sale securities	(11)	0	(22)	(1)
Comprehensive loss	<u>\$ (17,994)</u>	<u>\$ (21,246)</u>	<u>\$ (53,600)</u>	<u>\$ (66,928)</u>

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)

	For the Three Months Ended September 30, 2021					
	Common Stock	Amount	Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at June 30, 2021	112,454,321	\$ 112	\$ 678,001	\$ (11)	\$ (440,164)	\$ 237,938
Equity-based compensation	—	—	3,416	—	—	3,416
Exercise of stock options	1,000	—	3	—	—	3
Forfeiture of restricted stock awards	(1,392)	—	—	—	—	—
Unrealized loss on available-for-sale securities	—	—	—	(11)	—	(11)
Net loss	—	—	—	—	(17,983)	(17,983)
Balance at September 30, 2021	112,453,929	\$ 112	\$ 681,420	\$ (22)	\$ (458,147)	\$ 223,363

	For the Nine Months Ended September 30, 2021					
	Common Stock	Amount	Additional Paid in Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2020	87,052,323	\$ 87	\$ 536,568	\$ —	\$ (404,569)	\$ 132,086
Equity-based compensation	—	—	9,958	—	—	9,958
Sale of common stock, net of issuance costs of \$8,872	25,000,000	25	134,853	—	—	134,878
Exercise of stock options	11,625	—	41	—	—	41
Vesting of restricted stock units	419,193	—	—	—	—	—
Forfeiture of restricted stock awards	(29,212)	—	—	—	—	—
Unrealized loss on available-for-sale securities	—	—	—	(22)	—	(22)
Net loss	—	—	—	—	(53,578)	(53,578)
Balance at September 30, 2021	112,453,929	\$ 112	\$ 681,420	\$ (22)	\$ (458,147)	\$ 223,363

	For the Three Months Ended September 30, 2020					
	Common Stock	Amount	Additional Paid In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at June 30, 2020	48,364,092	\$ 48	\$ 402,625	\$ —	\$ (361,960)	\$ 40,713
Equity-based compensation	—	—	2,764	—	—	2,764
Vesting of restricted stock units	162,725	—	—	—	—	—
Forfeiture of restricted stock awards	(52,599)	—	—	—	—	—
Net loss	—	—	—	—	(21,246)	(21,246)
Balance at September 30, 2020	48,474,218	\$ 48	\$ 405,389	\$ —	\$ (383,206)	\$ 22,231

	For the Nine Months Ended September 30, 2020					
	Common Stock	Amount	Additional Paid In Capital	Accumulated Other Comprehensive Income (Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2019	48,283,270	\$ 48	\$ 396,278	\$ 1	\$ (316,279)	\$ 80,048
Equity-based compensation	—	—	9,111	—	—	9,111
Vesting of restricted stock units	284,700	—	—	—	—	—
Forfeiture of restricted stock awards	(93,752)	—	—	—	—	—
Unrealized loss on available-for-sale securities	—	—	—	(1)	—	(1)
Net loss	—	—	—	—	(66,927)	(66,927)
Balance at September 30, 2020	48,474,218	\$ 48	\$ 405,389	\$ —	\$ (383,206)	\$ 22,231

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,	
	2021	2020
Cash flows from operating activities:		
Net loss	\$ (53,578)	\$ (66,927)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of (discount)/premium on available-for-sale securities	535	(20)
Equity-based compensation expense	9,958	9,111
Depreciation expense	2,204	3,126
Gain on disposal of property and equipment	9	—
Changes in operating assets and liabilities:		
Prepaid expenses and other current and non-current assets	(1,639)	1,450
Accounts receivable - related party	(594)	—
Accounts payable	639	(2,602)
Accrued expenses and other current and non-current liabilities	(4,831)	(2,044)
Deferred revenue- related party, current and non-current	(9,593)	—
Net cash used in operating activities	<u>(56,890)</u>	<u>(57,906)</u>
Cash flows from investing activities:		
Purchases of property and equipment	(621)	(839)
Proceeds from sale and maturities of available-for-sale securities	3,930	7,900
Purchases of available-for-sale securities	(124,571)	(401)
Net cash (used in) provided by investing activities	<u>(121,262)</u>	<u>6,660</u>
Cash flows from financing activities:		
Proceeds from issuance of common stock, net of issuance costs	134,878	—
Proceeds from exercise of stock options	41	—
Net cash provided by financing activities	<u>134,919</u>	<u>—</u>
Net decrease in cash, cash equivalents and restricted cash	<u>(43,233)</u>	<u>(51,246)</u>
Cash, cash equivalents, and restricted cash at beginning of period	155,071	76,370
Cash, cash equivalents, and restricted cash at end of period	<u>\$ 111,838</u>	<u>\$ 25,124</u>
Supplemental disclosure of non-cash investing and financing activities:		
Change in unrealized loss	\$ (22)	\$ —
Decrease in right of use asset due to lease termination	\$ (1,233)	\$ —
Property and equipment included in accounts payable and accruals	<u>\$ 345</u>	<u>\$ —</u>

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

SOLID BIOSCIENCES INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(unaudited, amounts in thousands, except share and per share data)

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”).

The Company’s mission is to cure Duchenne muscular dystrophy (“Duchenne”), a genetic muscle-wasting disease predominantly affecting boys. It is caused by mutations in the dystrophin gene, which result in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Without functioning dystrophin and certain associated proteins, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. The Company’s lead product candidate, SGT-001, is a gene transfer candidate under investigation for its ability to drive functional dystrophin protein expression in patients’ muscles and improve the course of the disease. SGT-001 has been granted Rare Pediatric Disease Designation and Fast Track Designation in the United States and Orphan Drug Designations in both the United States and European Union. The Company filed an Investigational New Drug application (“IND”) in September 2017 and initiated a Phase I/II clinical trial for SGT-001 in the United States during the fourth quarter of 2017, which is called IGNITE DMD. In November 2019, IGNITE DMD was placed on clinical hold by the U.S. Food and Drug Administration (“FDA”). In October 2020, following changes to the clinical protocol designed to enhance patient safety, improvements to its manufacturing process, and the provision of additional efficacy and safety data to the FDA, the Company announced that the FDA lifted the clinical hold placed on the Company’s IGNITE DMD Phase I/II clinical trial. In February 2021, the Company resumed treatment of patients in the IGNITE DMD clinical trial. In May 2021, the Company announced the advancement of a next-generation Duchenne microdystrophin gene transfer program, SGT-003, a preclinical candidate that combines a novel and rationally-designed capsid candidate from this library with Solid’s proprietary nNOS containing microdystrophin construct.

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

The Company’s product 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, 2021, the Company has funded its operations primarily with the proceeds from the sale of redeemable preferred units and member units, the sale of common stock and prefunded warrants to purchase shares of its common stock in private placements, the sale of common stock in its initial public offering and follow-on public offering in March 2021 and sales under its at-the-market sales agreement.

In accordance with Accounting Standards Codification (“ASC”) 205-40, Going Concern, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company’s ability to continue as a going concern within one year after the date the financial statements are issued. As of September 30, 2021, the Company had an accumulated deficit of \$458,147. During the three and nine months ended September 30, 2021, the Company incurred a net loss of \$17,983 and \$53,578, respectively, and the Company used \$56,890 of cash in operations for the nine months ended September 30, 2021. The Company expects to continue to generate operating losses in the foreseeable future. Based upon its current operating plan, the Company expects that its cash, cash equivalents and available-for-sale securities of \$229,760 as of September 30, 2021, will be sufficient to fund its operating expenses and capital expenditure requirements for at least twelve months from the date of issuance of these financial statements. However, the Company has based this estimate on assumptions that may prove to be wrong, and its operating plan may change as a result of many factors currently unknown to it. As a result, the Company could deplete its capital resources sooner than it currently expects. The Company expects to finance its future cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances or licensing arrangements. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its research and development programs, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects.

The accompanying condensed consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America (“GAAP”). The accompanying condensed consolidated financial statements include the accounts of Solid Biosciences Inc. and its wholly owned or controlled subsidiaries. All intercompany accounts and transactions have been eliminated. In the opinion of management, the Company’s accompanying unaudited condensed consolidated financial statements include all adjustments, consisting of normal recurring accruals, necessary for a fair statement of the Company’s financial statements for interim periods in accordance with GAAP. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company’s consolidated financial statements and the accompanying notes included in the Company’s Annual Report on Form 10-K for the year ended December 31, 2020. 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. The results of operations for the three and nine months ended September 30, 2021 are not necessarily indicative of the operating results for the full year or for any other subsequent interim period.

2. Summary of Significant Accounting Policies

The Company’s accounting policies are described in the “Notes to Consolidated Financial Statements” in its Annual Report on Form 10-K for the year ended December 31, 2020 and updated, as necessary, in this report.

Use of 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 revenue recognition, the recognition of research and development expenses and equity-based compensation. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from the Company’s estimates.

The full extent to which the COVID-19 pandemic will directly or indirectly impact the Company’s business, results of operations and financial condition, including clinical trials and employee-related amounts, will depend on future developments that are highly uncertain, including new information that may emerge concerning COVID-19 and the actions taken to contain it or treat its impact. The Company has made estimates of the impact of COVID-19 within its financial statements and there may be changes to those estimates in future periods. Actual results could differ from the Company’s estimates.

Cash Equivalents

The Company considers all short-term, highly liquid investments with original maturities of 90 days or less at acquisition date to be cash equivalents.

Restricted Cash

The Company held restricted cash of \$2,162 and \$327 in separate restricted bank accounts as security deposits for leases of the Company's facilities as of September 30, 2021 and December 31, 2020, respectively. As of September 30, 2021 and December 31, 2020, the Company classified \$90 and \$0 as a current asset and \$2,072 and \$327 as a non-current asset respectively. A reconciliation of the amounts of cash and cash equivalents and restricted cash from the cash flow statement to the balance sheet is as follows:

	September 30, 2021	December 31, 2020	September 30, 2020	December 31, 2019
Cash and cash equivalents as presented on balance sheet	\$ 109,676	\$ 154,744	\$ 24,797	\$ 76,043
Restricted cash, current, as presented on balance sheet	90	—	—	—
Restricted cash, non-current, as presented on balance sheet	2,072	327	327	327
Cash and cash equivalents and restricted cash as presented on cash flow statement	<u>\$ 111,838</u>	<u>\$ 155,071</u>	<u>\$ 25,124</u>	<u>\$ 76,370</u>

Leases

At inception of a contract, the Company determines if a contract meets the definition of a lease. A lease is a contract, or part of a contract, that conveys the right to control the use of identified property, plant, or equipment (an identified asset) for a period of time in exchange for consideration. The Company determines if the contract conveys the right to control the use of an identified asset for a period of time. The Company assesses throughout the period of use whether the Company has both of the following: (1) the right to obtain substantially all of the economic benefits from use of the identified asset and (2) the right to direct the use of the identified asset. This determination is reassessed if the terms of the contract are changed. Leases are classified as operating or finance leases based on the terms of the lease agreement and certain characteristics of the identified asset. Right-of-use assets and lease liabilities are recognized at the lease commencement date based on the present value of the minimum future lease payments. The Company's policy is to not record leases with an original term of twelve months or less on the consolidated balance sheets. The Company recognizes lease expense for these short-term leases on a straight-line basis over the lease term. Certain lease agreements include rental payments that are adjusted periodically for inflation or other variables. In addition to rent, the leases may require the Company to pay additional amounts for taxes, insurance, maintenance and other expenses, which are generally referred to as non-lease components. Such adjustments to rental payments and variable non-lease components are treated as variable lease payments and recognized in the period in which the obligation for these payments was incurred. Variable lease components and variable non-lease components are not measured as part of the right of use asset and liability. Only when lease components and their associated non-lease components are fixed are they accounted for as a single lease component and recognized as part of a right of use asset and liability. Total contract consideration is allocated to the combined fixed lease and non-lease components.

In June 2021, the Company entered into a lease with Hood Park LLC ("Landlord"), pursuant to which the Company will lease approximately 49,869 square feet of office, laboratory, research and development and manufacturing space located in Charlestown, Massachusetts ("Premises"). The Company intends to relocate its corporate headquarters to the Premises in early 2022. The term of the lease commences on the later of (i) the date the Landlord delivers the Premises to the Company or (ii) the earlier of (a) the date the Company's work on the Premises is substantially completed, (b) the date the Company commences business operations in the Premises, or (c) the one hundred twentieth (120th) day following the Landlord's satisfaction of item (i) above. The lease commencement date is anticipated to be in the first half of 2022. The date on which the Company will become responsible for paying rent under the lease will be 61 days following the lease commencement date. The initial term of the lease will be for a ten-year period commencing on the lease commencement date, unless earlier terminated. The lease provides the Company with an option to extend the lease for an additional five-year term. The Company and the Landlord are each obligated to undertake certain improvements prior to the commencement of the lease. The Landlord delivered the Premises to the Company in September 2021. The Company plans to make certain improvements to the Premises prior to commencement of the lease, which will be partially paid for with a tenant improvement allowance. The Company was required to post a customary letter of credit in the amount of \$1,833, subject to decrease on a set schedule, as a security deposit pursuant to the lease. As of September 30, 2021, the lease had not commenced. For the three and nine months ended September 30, 2021, the Company incurred approximately \$680 of tenant improvement allowance receivable.

In June 2021, the Company determined that it no longer needed a floor of office space that it was renting and terminated its lease with another landlord. As a result of the termination, the Company wrote off \$1,314 and \$1,233 of the remaining lease liability and right-of-use asset, respectively, associated with the lease.

Segment Data

The Company manages its operations as a single segment for the purposes of assessing performance and making operating decisions. The Company's singular focus is on developing treatments through gene therapy and other means for patients with Duchenne. All of the Company's tangible assets are held in the United States.

Related Parties

In October 2020, the Company entered into a collaboration and license agreement (the "Collaboration Agreement") with Ultragenyx Pharmaceutical Inc. ("Ultragenyx"). In connection with the Collaboration Agreement, Ultragenyx also purchased 7,825,797 shares of the Company's common stock, which resulted in Ultragenyx becoming a related party of the Company.

In November 2020, the Company entered into a consulting agreement with Danforth Advisors, LLC, or Danforth, an affiliate of Stephen DiPalma, the Company's interim chief financial officer. Pursuant to the consulting agreement, Danforth provides the Company with the chief financial officer services of Mr. DiPalma, and other services, including financial planning, offering support and accounting services, in exchange for fees payable to Danforth based on hourly rates. The Company has paid Danforth approximately \$194 and \$381 for the three and nine months ended September 30, 2021, respectively, and \$0 for each of the three and nine months ended September 30, 2020. In accordance with the consulting agreement, in November 2020, the Company issued to Danforth a warrant to purchase 30,000 shares of the Company's common stock at an exercise price per share of \$3.29. As of September 30, 2021, the shares had vested in full. The consulting agreement may be terminated by either party without cause upon 60 days' prior written notice to the other party and with cause upon 30 days' prior written notice to the other party.

Recently Adopted Accounting Pronouncements

In December 2019, the Financial Accounting Standards Board (the "FASB") issued Accounting Standards Update ("ASU") No. 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes*, which simplifies the accounting for income taxes by removing certain exceptions to the general principles in ASC 740 and clarifies and amends existing guidance to improve consistent application. The standard became effective for the Company beginning January 1, 2021. The amendments that are related to changes in ownership of foreign equity method investments or foreign subsidiaries are to be applied on a modified retrospective basis through a cumulative-effect adjustment to retained earnings as of the beginning of the fiscal year of adoption. The amendments that are related to franchise taxes that are partially based on income are to be applied on either a retrospective basis for all periods presented or a modified retrospective basis through a cumulative-effect adjustment to retained earnings as of the beginning of the fiscal year of adoption. All other amendments under this ASU are to be applied on a prospective basis. The Company adopted the guidance effective January 1, 2021. The adoption of this new standard did not have a material impact on the Company's financial statements.

Recently Issued Accounting Pronouncements

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*, which was subsequently modified by several ASU's issued in 2018 and 2019. The standard introduces a new current expected credit loss ("CECL") model for measuring expected credit losses for certain types of financial instruments measured at amortized cost and replaces the incurred loss model. The CECL model requires an entity to recognize an allowance for credit losses for the difference between the amortized cost basis of a financial instrument and the amount the entity expects to collect over the instrument's contractual life after consideration of historical experience, current conditions, and reasonable and supportable forecasts. The standard eliminates the concept of other-than-temporary impairment and requires an entity to determine whether any impairment is the result of a credit loss or other factors. ASU 2016-13 is effective for the Company on January 1, 2023. The Company is currently evaluating the potential impact that this standard may have on its financial statements and related disclosures.

3. Collaborations

Ultragenyx Collaboration

Collaboration Agreement

On October 22, 2020 (the "Effective Date"), the Company entered into a collaboration and license agreement with Ultragenyx Pharmaceutical Inc. to focus on the development and commercialization of new gene therapies for Duchenne Muscular Dystrophy. The Company granted Ultragenyx an exclusive worldwide license for any pharmaceutical product that expresses the Company's proprietary microdystrophin construct from AAV8 and variants thereof in clade E for the treatment of Duchenne Muscular Dystrophy and other diseases resulting from the lack of functional dystrophin (the "Licensed Products"). The Company retains exclusive rights to all other uses of its microdystrophin proteins, including under its existing SGT-001 program.

The Company will conduct certain research and development activities with respect to the development of the Licensed Products. Ultragenyx will reimburse the Company for personnel and out-of-pocket costs that the Company incurs in conducting such development activities.

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

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

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

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

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

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

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

Stock Purchase Agreement

In connection with the execution of the Collaboration Agreement, Ultragenyx and the Company also entered into a stock purchase agreement (the "Stock Purchase Agreement") on October 22, 2020 (the "Closing Date"), pursuant to which the Company issued and sold 7,825,797 shares of its common stock (the "Shares") to Ultragenyx at a price of \$5.1113 per share for an aggregate purchase price of approximately \$40,000. The Stock Purchase Agreement contains customary representations, warranties and covenants of each of the parties thereto. Following the sale of the Shares, Ultragenyx beneficially owned approximately 14.45% of the Company's outstanding common stock. As of September 30, 2021, Ultragenyx beneficially owned less than 10% of the Company's outstanding common stock.

Investor Agreement

In connection with the consummation of the transactions contemplated by the Stock Purchase Agreement, the Company and Ultragenyx entered into an Investor Agreement (the "Investor Agreement") on the Effective Date. Pursuant to the terms of the Investor Agreement, Ultragenyx agreed that the Shares will be subject to a lock-up restriction, such that Ultragenyx will not, and will also cause its affiliates not to, without the prior approval of the Company and with certain exceptions, sell, transfer or otherwise dispose of the Shares until the earliest to occur of (i) 18 months after the Effective Date, (ii) the termination of the Collaboration Agreement or (iii) other specified events.

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

Accounting Treatment

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

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

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

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

During the three and nine months ended September 30, 2021, the Company recognized \$3,537 and \$10,466, respectively, of related party collaboration revenue, associated with its collaboration with Ultragenyx related to research and development services performed during the period and the corresponding cost reimbursement receivable.

The following table presents changes in the balances of the Company's related party collaboration receivables and contract liabilities during the three and nine months ended September 30, 2021:

	<u>Balance as of June 30, 2021</u>	<u>Additions</u>	<u>Deductions</u>	<u>Balance as of September 30, 2021</u>
Related party collaboration receivable	\$ 286	\$ 308	\$ —	\$ 594
Contract liabilities:				
Deferred revenue	14,354	—	(3,229)	11,125

	<u>Balance as of December 31, 2020</u>	<u>Additions</u>	<u>Deductions</u>	<u>Balance as of September 30, 2021</u>
Related party collaboration receivable	\$ —	\$ 874	\$ (280)	\$ 594
Contract liabilities:				
Deferred revenue	20,718	—	(9,593)	11,125

The change in receivables balance for the three and nine months ended September 30, 2021 is driven by amounts owed to the Company for research and development services provided, partially offset by \$280 in collections received from Ultragenyx during the nine months ended September 30, 2021.

As of September 30, 2021, there was \$11,125 of deferred revenue related to the Collaboration Agreement, which is classified as either current or non-current in the accompanying condensed consolidated balance sheet based on the period the services are expected to be delivered. Additionally, as of September 30, 2021, there was \$594 of related party collaboration receivables related to reimbursable costs expected to be received from Ultragenyx for research and development services performed.

Costs incurred relating to the Collaboration Agreement consist of internal and external research and development costs, which primarily include salaries and benefits, lab supplies, preclinical research studies, clinical studies, consulting services, and commercial development. These costs are included in research and development expenses in the Company's condensed consolidated statement of operations during the three and nine months ended September 30, 2021.

4. Fair Value of Financial Assets and Liabilities

The following tables present information about the Company's assets and liabilities that are measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

Fair Value Measurements as of September 30, 2021				
Using:				
	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>	<u>Total</u>
Assets:				
Cash equivalents	\$ —	\$ 83,719	\$ —	\$ 83,719
Available-for-sale securities	\$ —	\$ 120,084	\$ —	\$ 120,084
	<u>\$ —</u>	<u>\$ 203,803</u>	<u>\$ —</u>	<u>\$ 203,803</u>

Fair Value Measurements as of December 31, 2020				
Using:				
	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>	<u>Total</u>
Assets:				
Cash equivalents	\$ —	\$ 84,462	\$ —	\$ 84,462
	<u>\$ —</u>	<u>\$ 84,462</u>	<u>\$ —</u>	<u>\$ 84,462</u>

As of September 30, 2021 and December 31, 2020, the fair values of the Company's available-for-sale debt securities, which consisted of treasury bills, commercial paper, and corporate bond securities, was determined using Level 2 inputs. During the nine months ended September 30, 2021 and the year ended December 31, 2020, there were no transfers between Level 1, Level 2 and Level 3.

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

5. Available-for-Sale Securities

As of September 30, 2021, the fair value of available-for-sale securities by type of security was as follows:

	September 30, 2021			Fair Value
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	
Investments:				
Treasury bill	\$ 6,999	\$ —	\$ —	\$ 6,999
Corporate bond securities	\$ 101,110	\$ —	\$ (22)	\$ 101,088
Commercial paper	\$ 11,997	\$ —	\$ —	\$ 11,997
	<u>\$ 120,106</u>	<u>\$ —</u>	<u>\$ (22)</u>	<u>\$ 120,084</u>

As of December 31, 2020, there were no available-for-sale securities.

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

	September 30, 2021	
	Amortized Cost	Fair Value
Due in one year or less	\$ 120,106	\$ 120,084
Total available-for-sale securities	<u>\$ 120,106</u>	<u>\$ 120,084</u>

The weighted average maturity of the Company's available-for-sale securities as of September 30, 2021 was approximately 0.6 years.

6. Property and Equipment

Property and equipment consists of the following:

	September 30, 2021	December 31, 2020
Furniture and fixtures	\$ 213	\$ 203
Laboratory equipment	10,315	9,631
Leasehold improvements	4,713	4,713
Computer equipment	436	436
Computer software	553	553
Construction in process	1,557	1,330
	<u>17,787</u>	<u>16,866</u>
Less accumulated depreciation	10,901	8,713
	<u>\$ 6,886</u>	<u>\$ 8,153</u>

Depreciation expense was \$752 and \$2,204 for the three and nine months ended September 30, 2021.

7. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

	September 30, 2021	December 31, 2020
Prepaid research and development expenses	\$ 4,614	\$ 2,674
Tenant improvement allowance receivable	680	-
Prepaid expenses and other assets	3,072	1,483
	<u>\$ 8,366</u>	<u>\$ 4,157</u>

8. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following:

	September 30, 2021	December 31, 2020
Accrued research and development	\$ 2,136	\$ 2,091
Accrued compensation	2,247	3,834
Accrued other	3,620	2,715
	<u>\$ 8,003</u>	<u>\$ 8,640</u>

9. Stockholders' Equity

In July 2019, the Company issued and sold in a private placement (i) 10,607,525 shares of its common stock at a price per share of \$4.65 and (ii) 2,295,699 pre-funded warrants to purchase shares of its common stock at a price per warrant of \$4.64. Each pre-funded warrant is exercisable for one share of common stock at an exercise price of \$0.01 and the pre-funded warrants have no expiration date. The Company received gross proceeds from the private placement of \$59,977, before deducting offering costs of \$2,102. In October 2020, 137,460 of these pre-funded warrants were exercised. No warrants were exercised during the three and nine months ended September 30, 2021 and 2020.

In October 2020, in connection with the execution of the Collaboration Agreement, Ultragenyx and the Company also entered into the Stock Purchase Agreement, pursuant to which the Company issued and sold 7,825,797 shares of its common stock to Ultragenyx at a price of \$5.1113 per share for an aggregate purchase price of approximately \$40,000.

In October 2020, the Company sold 6,309,632 shares of common stock pursuant to a sales agreement (the "ATM Sales Agreement"), by and between the Company and Jefferies LLC ("Jefferies"), resulting in net proceeds to the Company of \$23,209.

In December 2020, the Company issued and sold in a private placement 24,324,320 shares of its common stock at a price per share of \$3.70. The Company received net proceeds from the private placement of \$86,210 after deducting placement agent fees and offering expenses of \$3,790.

In March 2021, the Company issued and sold in a public offering 25,000,000 shares of its common stock at a price to the public of \$5.575 per share. The Company received net proceeds of \$134,878 after deducting underwriting discounts and commissions and offering expenses.

10. Equity-Based Compensation

In connection with the closing of the Company's initial public offering, the board of directors and stockholders approved the 2018 Omnibus Incentive Plan, which provides for the reservation of 5,001,000 shares of common stock for equity awards. On June 16, 2020, the Company's stockholders approved the 2020 Equity Incentive Plan ("2020 Plan") which consists of (i) 3,000,000 shares of common stock and (ii) additional shares of common stock (up to 4,879,025) as is equal to (i) the number of shares reserved under the Company's 2018 Omnibus Incentive Plan ("2018 Plan") that remain available for grant under the 2018 Plan as of immediately prior to the date the 2020 Plan was approved by the Company's stockholders and (ii) the number of shares subject to awards granted under the 2018 Plan which awards expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right. In June 2021, the Company's stockholders approved an amendment to the 2020 Plan to reserve an additional 7,000,000 shares of common stock for issuance under the plan. At September 30, 2021, 8,836,782 shares remained available for future issuance under the 2020 Plan. Under the 2020 Plan, stock options may not be granted at less than fair value on the date of grant. In June 2021, the Company's stockholders also approved the 2021 Employee Stock Purchase Plan ("ESPP"), which provides for 1,102,885 shares to be available for purchase by eligible employees according to its

terms. The first offering period under the ESPP commenced on September 1, 2021. At September 30, 2021, 1,102,885 shares remained available for future issuance under the ESPP.

During the three and nine months ended September 30, 2021, the Company granted options for the purchase of 458,350 and 3,460,245, respectively, shares of common stock. During the three and nine months ended September 30, 2021, the Company granted 98,650 restricted stock units.

The Company recorded equity-based compensation expense related to all of its share-based awards to employees and non-employees in the following captions within its condensed consolidated statements of operations:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Research and development	\$ 1,601	\$ 1,524	\$ 5,083	\$ 4,379
General and administrative	1,815	1,240	4,875	3,881
Restructuring	—	—	—	851
Total	<u>\$ 3,416</u>	<u>\$ 2,764</u>	<u>\$ 9,958</u>	<u>\$ 9,111</u>

11. Income Taxes

During the three and nine months ended September 30, 2021 and 2020, the Company recorded no income tax benefits for the net operating losses incurred or for the research and development tax credits and orphan drug credits generated in each year due to its uncertainty of realizing a benefit from those items. The Company has provided a valuation allowance for the full amount of its net deferred tax assets because, at September 30, 2021 and December 31, 2020, it was more likely than not that any future benefit from deductible temporary differences and net operating loss and tax credit carryforwards would not be realized.

As of September 30, 2021 and December 31, 2020, the Company had not recorded any amounts for unrecognized tax benefits. The Company files income tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's C-Corporation tax years beginning with the year ended December 31, 2018 are open under statute. Any tax credit or net operating loss carryforward can be adjusted in future periods after the respective year of generation's statute of limitation has closed.

12. Commitments and Contingencies

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, 2021.

13. Net Loss per Share

Basic and diluted net loss per share attributable to common stockholders were calculated as follows:

The numerator for basic and diluted net loss per share attributable to common stockholders is as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Net loss attributable to common stockholders	<u>\$ (17,983)</u>	<u>\$ (21,246)</u>	<u>\$ (53,578)</u>	<u>\$ (66,927)</u>

The denominator is as follows:

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2021</u>	<u>2020</u>	<u>2021</u>	<u>2020</u>
Weighted average shares of common stock outstanding, basic and diluted	110,154,641	45,999,769	102,548,471	45,876,987
Weighted average shares of pre-funded warrants to purchase common stock	2,158,329	2,295,699	2,158,329	2,295,699
Total	112,312,970	48,295,468	104,706,800	48,172,686

Net loss per share attributable to common stockholders, basic and diluted is as follows:

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2021</u>	<u>2020</u>	<u>2021</u>	<u>2020</u>
Net loss per share attributable to common stockholders	\$ (0.16)	\$ (0.44)	\$ (0.51)	\$ (1.39)

The following potential common stock equivalents, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share attributable to common stockholders for the periods indicated because including them would have had an anti-dilutive effect for the nine months ended September 30:

	<u>2021</u>	<u>2020</u>
Options to purchase shares of common stock	5,851,431	3,324,885
Unvested shares of common stock	1,849	102,753
Unvested restricted stock units	391,269	1,254,545
	<u>6,244,549</u>	<u>4,682,183</u>

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, 2020 included in our annual report filed on Form 10-K on March 15, 2021.

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

Our mission is to cure Duchenne muscular dystrophy, or Duchenne, a genetic muscle-wasting disease predominantly affecting boys, with symptoms that usually manifest between three and five years of age. Duchenne is a progressive, irreversible and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated prevalence of 10,000 to 15,000 cases in the United States alone. Duchenne is caused by mutations in the dystrophin gene, which result in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Without functioning dystrophin and certain associated proteins, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. There is no cure for Duchenne and, for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments.

Our efforts are focused on our lead product candidate, SGT-001, a gene transfer candidate under investigation for its ability to drive functional dystrophin protein expression in patients’ muscles and improve the course of the disease. Based on our preclinical program that included multiple animal species of different phenotypes and genetic variations, as well as preliminary clinical trial biomarker results, we believe that SGT-001, has the potential to slow or even halt the progression of Duchenne, regardless of the type of genetic mutation or stage of the disease. SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, and Fast Track Designation, in the United States and Orphan Drug Designations in both the United States and European Union. The safety and efficacy of SGT-001 are currently being evaluated in a Phase I/II clinical trial called IGNITE DMD. In March 2021, we announced that the seventh patient, who was the fourth patient in the 2E14 vg/kg cohort, was treated with SGT-001, under an amended clinical protocol designed to enhance patient safety, incorporating the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and increasing the prednisone dose in the first month post dosing. This patient was safely dosed, with transient and manageable adverse events, none of which were serious.

In April 2021, an eighth patient, also in the 2E14 vg/kg cohort, 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. This type of event is described in our Investigators Brochure and is not considered unexpected.

Following dosing of these two patients with our second-generation manufacturing process and clinical strategy, we conducted an extensive review of all clinical data, resulting in a strengthened risk mitigation plan, which has also been submitted to the FDA. Activities are underway to advance IGNITE DMD with the next patient dosing anticipated in the fourth quarter of 2021.

No new drug-related safety findings have been identified in patients one through eight in post-dosing periods of 90 days to more than 3.5 years. We continue to follow dosed patients and collect data to support the potential benefit from dosing with SGT-001.

In May 2021, we reported long-term biopsy data collected from patients four through six, who were dosed at the 2E14 vg/kg dose level. Analyses of the biopsies, taken 2 years, 1.5 years and 1 year post-dosing, respectively, demonstrated durable and widespread expression of the microdystrophin protein. The long-term results are consistent with the day 90, interim data reported in March 2021 and continue to demonstrate the functionality of the SGT-001 microdystrophin, as highlighted by the recruitment of key dystrophin associated proteins: beta-sarcoglycan and neuronal Nitric Oxide Synthase or nNOS. The long-term muscle biopsy results were analyzed by two methods, western blot and immunofluorescence. As reflected in the table below, the Western Blot data indicate that expression was maintained in patient four and increased compared to the day 90 biopsies in patients five and six. Immunofluorescence data show microdystrophin function via continued localization to the muscle membrane, with the percent positive fibers remaining comparable to the day 90 biopsies in all three patients.

Patient No.	Last Timepoint Post-Dosing	Western Blot		Immunofluorescence (% Positive Fibers)	
		Day 90	Last Timepoint	Day 90	Last Timepoint
4	24 months	BLQ *	BLQ *	10-20%	10-30%
5	18 months	17.50%	69.80%	50-70%	85%
6	12 months	8.00%	20.30%	50-70%	50-60%

*Below the limit of quantification

Further morphological analysis of the muscle biopsies conducted by us indicate that sustained microdystrophin protein expression and function resulted in membrane stabilization, evidenced by minimal progression of muscle deterioration since the day 90 timepoint. These long-term pathophysiological improvements support the recently reported positive trends in the clinical biomarker and functional data.

In September 2021, we reported 1.5-year functional data and patient-reported outcome measures for patients four through six in IGNITE DMD, who were dosed at the 2E14 vg/kg dose level. Also in September 2021, we reported additional pulmonary function data from the first six patients in IGNITE DMD, three who were dosed at the 5E13 vg/kg dose level and three who were dosed at the 2E14 vg/kg dose level.

In May 2021, we announced the advancement of a next-generation Duchenne microdystrophin gene transfer program, SGT-003. We have developed a library of novel capsids that show increased muscle tropism, decreased liver biodistribution and drive improved efficiency compared with AAV9 in various *in vitro* and *in vivo* models. SGT-003 is a preclinical candidate that combines a novel and rationally-designed capsid candidate from this library with Solid's proprietary nNOS containing microdystrophin construct. We are currently targeting an IND submission for SGT-003 in early-2023.

In November 2021, in collaboration with REGENXBIO, we formally launched the Pathway Development Consortium (PDC), a multistakeholder initiative which aims to identify, develop, expand and maintain pathways to effective therapies for patients diagnosed early in life with rare diseases. The PDC seeks to achieve these goals by bringing together a broad and diverse group of stakeholders from the rare disease and AAV gene therapy communities, including patients, industry, regulators, academia and payers, among others, for meaningful scientific and policy discussions.

Since our inception, we have devoted substantial resources to identifying and developing SGT-001, SGT-003 and our other product candidates, developing our manufacturing processes, organizing and staffing our company and providing general and administrative support for these operations. We have incurred significant losses every year since our inception. We do not have any products approved for sale. To date, we have not generated any revenue from product sales. Our ability to eventually generate any product revenue sufficient to achieve profitability will depend on the successful development, approval and eventual commercialization of SGT-001, SGT-003 and our other product candidates. If successfully developed and approved, we intend to commercialize SGT-001 and SGT-003 in the United States and European Union and may enter into licensing agreements or strategic collaborations in other markets. If we generate product sales or enter into licensing agreements or strategic collaborations, we expect that any revenue we generate will fluctuate from quarter to quarter and year to year as a result of the timing and amount of any product sales, license fees, milestone payments and other payments. If we fail to complete the development of SGT-001, SGT-003 and our other product candidates in a timely manner or obtain regulatory approval of them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

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 \$53.6 million for the nine months ended September 30, 2021 and \$66.9 million for the nine months ended September 30, 2020. As of September 30, 2021, we had an accumulated deficit of \$458.1 million. We expect to incur significant expenses and operating losses for the foreseeable future.

In January 2020, we announced a reduction in workforce by approximately 30% as part of a strategic plan designed to create a leaner company focused on advancing SGT-001.

As we seek to develop and commercialize SGT-001, SGT-003 or any other product 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 SGT-001, SGT-003 or our other product 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.

In October 2020, we entered into a collaboration and license agreement, or the Collaboration Agreement, with Ultragenyx Pharmaceutical Inc., or Ultragenyx, pursuant to which we granted Ultragenyx an exclusive worldwide license under certain intellectual property rights controlled by us to make, have made, use, distribute, offer for sale, sell, import and export, including to research, develop, modify, enhance, improve, register, distribute, commercialize, or otherwise dispose of AAV8 or other clade E AAV variant pharmaceutical products that express our MD5 nNOS binding domain form of microdystrophin protein for the diagnosis, treatment, cure, mitigation or prevention of Duchenne and other disease indications resulting from a lack of functional dystrophin, including Becker muscular dystrophy. On a product-by-product basis, we have the option to fund 30% of the development costs in the United States and European Union and share 30% of the net income and net losses on net sales of such Licensed Product in the United States and European Union. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement with Ultragenyx, pursuant to which we issued and sold 7,825,797 shares of our common stock to Ultragenyx for an aggregate purchase price of approximately \$40 million.

During the year ended December 31, 2020, we sold 6,309,632 shares pursuant to the ATM Sales Agreement, resulting in net proceeds of \$23.2 million.

In December 2020, we completed a private placement of shares of common stock resulting in net proceeds to us of \$86.2 million, after deducting offering costs.

In March 2021, we issued and sold in a public offering 25,000,000 shares of our common stock at a price per share to the public of \$5.75, including the full exercise by the underwriters of an option to purchase additional shares of common stock, or the March 2021 Offering. We received net proceeds of approximately \$134.9 million after deducting underwriter discounts and commissions and offering expenses.

As of September 30, 2021, we had cash, cash equivalents, and available-for-sale securities of \$229.8 million. We believe that our cash, cash equivalents, and available-for-sale securities as of September 30, 2021 will enable us to fund our operating expenses and capital expenditure requirements into the second quarter of 2023. 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.

The ongoing COVID-19 pandemic has caused federal, state, and local governments to implement measures to slow the spread of the outbreak through quarantines, strict travel restriction and bans, heightened border scrutiny and other measures. We are following, and will continue to follow, recommendations from the U.S. Centers for Disease Control and Prevention as well as federal, state, and local governments regarding working-from-home practices for non-essential employees. As a result, we have modified our business practices, including implementing a work from home policy for all employees who are able to perform their duties remotely. We expect to continue to take actions as may be required or recommended by government authorities or as we determine are in the best interests of our employees, and other business partners in light of COVID-19. The full extent of the impact of COVID-19 on our business, results of operations and financial condition will depend on future developments that are highly uncertain, including the length and severity of this pandemic, the actions taken to contain it or treat its impact and the impact on our clinical development, employees, vendors and suppliers, all of which are uncertain and cannot be predicted. We will continue to monitor the situation closely.

Financial operations overview

Revenue

Collaboration revenue

Collaboration revenue was \$3.5 million and \$10.5 million for the three and nine months ended September 30, 2021, respectively. We recognized this revenue related to research services and cost reimbursement from the Collaboration Agreement.

Product revenue

We have not generated any product revenue to date and do not expect to generate any product revenue from the sale of our products for the foreseeable future, if ever. If our development efforts for SGT-001, SGT-003 or our other product candidates are successful and result in marketing approval, we may generate product revenue in the future from product sales.

Operating expenses

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

Research and development expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of SGT-001, SGT-003 and our other product candidates and include:

- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct research and preclinical activities on our behalf, as well as contract manufacturing organizations, or CMOs, that manufacture SGT-001, SGT-003 and our other product candidates for use in our preclinical studies and clinical trials;
- salaries, benefits and other related costs, including equity-based compensation expense, for personnel engaged in research and development functions;
- costs of outside consultants, engaged to assist in our research and development activities, including their fees, equity-based compensation and related travel expenses;
- costs of laboratory supplies and acquiring, developing and manufacturing preclinical study and clinical trial materials;
- costs incurred in seeking regulatory approval of SGT-001, SGT-003 and our other product 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.

We expense research and development costs as incurred. We recognize costs for certain development activities, such as preclinical research and development and clinical trial costs, based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors, collaborators and third-party service providers. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our consolidated financial statements as prepaid or accrued research and development expenses.

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

The following table summarizes our research and development expenses by product candidates for the respective periods:

(In thousands)	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
SGT-001	\$ 5,088	\$ 8,628	\$ 17,995	\$ 24,408
SGT-003 and other product candidates	127	83	627	465
Unallocated research and development expenses				
Personnel related expenses	6,295	5,179	17,572	16,512
External expenses	2,915	2,155	7,950	7,773
Total unallocated research and development expenses	9,210	7,334	25,522	24,285
Total research and development expenses	\$ 14,425	\$ 16,045	\$ 44,144	\$ 49,158

We cannot determine with certainty the duration, costs and timing of clinical trials of SGT-001, SGT-003 and our other product candidates or if, when or to what extent we will generate revenue from the commercialization and sale of any of our product candidates for which we obtain marketing approval or our other research and development expenses. We may never succeed in obtaining marketing approval for any of our product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including:

- the scope, rate of progress, expense and results of any clinical trials of SGT-001, SGT-003 or other product 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.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those 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 increase for the foreseeable future as we proceed with clinical trials for SGT-001, initiate clinical trials for SGT-003 or any future product candidates and continue to identify and develop additional product candidates.

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, which include direct depreciation costs and allocated expenses for rent and maintenance of office facilities and other operating costs.

We expect that our general and administrative expenses will increase in the future as we increase our general and administrative personnel headcount to support our research and development activities and activities related to the clinical trials for and potential commercialization of SGT-001, SGT-003 and our other product candidates.

Restructuring charges

In January 2020, we implemented changes to our organizational structure to create a leaner company focused on advancing SGT-001. In connection with the restructuring, we made changes to our management team and reduced headcount by approximately 30%.

Other income (expense), net

Other income (expense), net consists of interest income earned on our cash, cash equivalents, available-for-sale securities, funding from charitable organizations, and financing leases interest expense.

Income taxes

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

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

Critical accounting policies and use of estimates

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

During the nine months ended September 30, 2021, there were no material changes to our critical accounting policies. 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, 2020 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:

- Revenue recognition;
- Accrued research and development expenses; and
- Equity-based compensation.

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, 2021 and 2020

The following table summarizes our results of operations for the three months ended September 30, 2021 and 2020:

(in thousands)	Three Months Ended September 30,		Increase (decrease)
	2021	2020	
Collaboration revenue - related party	\$ 3,537	\$ —	\$ 3,537
Operating expenses:			
Research and development	14,425	16,045	(1,620)
General and administrative	7,143	5,181	1,962
Restructuring charges	—	—	—
Total operating expenses	21,568	21,226	342
Loss from operations	(18,031)	(21,226)	3,195
Other income (expense), net	48	(20)	68
Net loss	\$ (17,983)	\$ (21,246)	\$ 3,263

Collaboration revenue

Collaboration revenue for the three months ended September 30, 2021 was \$3.5 million, compared to no collaboration revenue for the three months ended September 30, 2020. The increase in collaboration revenue was related to research services and cost reimbursement received under the Collaboration Agreement with Ultragenyx, which we entered into in the fourth quarter of 2020.

Research and development expenses

(in thousands)	Three Months Ended September 30,		Increase (decrease)
	2021	2020	
SGT-001	\$ 5,088	\$ 8,628	\$ (3,540)
SGT-003 and other product candidates	127	83	44
Unallocated research and development expenses			
Personnel related expenses	6,295	5,179	1,116
External expenses	2,915	2,155	760
Total unallocated research and development expenses	9,210	7,334	1,876
Total research and development expenses	\$ 14,425	\$ 16,045	\$ (1,620)

Research and development expenses for the three months ended September 30, 2021 were \$14.4 million, compared to \$16.0 million for the three months ended September 30, 2020. The decrease of \$1.6 million in research and development expenses was primarily due to a \$4.3 million decrease in manufacturing costs for our lead product candidate SGT-001, offset by an increase in unallocated research and development costs of \$1.9 million, primarily due to an increase in personnel related expenses of \$1.1 million and an increase in other R&D expenses of \$0.8 million, and an increase in clinical and non-clinical costs of \$0.8 million.

General and administrative expenses

General and administrative expenses were \$7.1 million for the three months ended September 30, 2021, compared to \$5.2 million for the three months ended September 30, 2020. The increase of \$2.0 million was due to an increase in corporate expenses of \$1.1 million primarily related to consulting and professional services and an increase in personnel related expenses of \$0.9 million.

Other income (expense), net

Other income (expense), net was less than \$0.1 million for the three months ended September 30, 2021, compared to other expense of less than \$0.1 million for the three months ended September 30, 2020. The activity was primarily related to the increase in interest income on available-for-sale securities included within our portfolio.

Comparison of the nine months ended September 30, 2021 and 2020

The following table summarizes our results of operations for the nine months ended September 30, 2021 and 2020:

(in thousands)	Nine Months Ended September 30,		Increase (decrease)
	2021	2020	
Collaboration revenue - related party	\$ 10,466	\$ —	\$ 10,466
Operating expenses:			
Research and development	44,144	49,158	(5,014)
General and administrative	19,924	15,957	3,967
Restructuring charges	—	1,944	(1,944)
Total operating expenses	64,068	67,059	(2,991)
Loss from operations	(53,602)	(67,059)	13,457
Other income (expense), net	24	132	(108)
Net loss	\$ (53,578)	\$ (66,927)	\$ 13,349

Collaboration revenue

Collaboration revenue for the nine months ended September 30, 2021 was \$10.5 million, compared to no collaboration revenue for the nine months ended September 30, 2020. The increase in collaboration revenue was related to research services and cost reimbursement received under the Collaboration Agreement with Ultragenyx, which we entered into in the fourth quarter of 2020.

Research and development expenses

(in thousands)	Nine Months Ended September 30,		Increase (decrease)
	2021	2020	
SGT-001	\$ 17,995	\$ 24,408	\$ (6,413)
SGT-003 and other product candidates	627	465	162
Unallocated research and development expenses			
Personnel related expenses	17,572	16,512	1,060
External expenses	7,950	7,773	177
Total unallocated research and development expenses	25,522	24,285	1,237
Total research and development expenses	\$ 44,144	\$ 49,158	\$ (5,014)

Research and development expenses for the nine months ended September 30, 2021 were \$44.1 million, compared to \$49.1 million for the nine months ended September 30, 2020. The decrease of \$5.0 million in research and development expenses was primarily due to a \$6.4 million decrease in costs related to our lead product candidate SGT-001, driven by lower manufacturing costs of \$7.4 million offset by an increase in clinical and non-clinical costs of \$1.0 million. The decrease in research and development expenses was also offset by a \$1.1 million increase in personnel related expenses. The decrease in research and development was offset by a \$0.3 million increase in costs related to SGT-003 and other product candidates.

General and administrative expenses

General and administrative expenses were \$19.9 million for the nine months ended September 30, 2021, compared to \$16.0 million for the nine months ended September 30, 2020. The increase of \$3.9 million was due to an increase in corporate expenses of \$2.2 million primarily related to consulting and professional services and an increase in personnel related expenses of \$1.7 million.

Other income (expense), net

Interest expense was less than \$0.1 million for the nine months ended September 30, 2021, compared to interest income of \$0.1 million for the nine months ended September 30, 2020. The decrease was primarily related to less interest income earned on the available-for-sale securities in our portfolio.

Liquidity and capital resources

Sources of liquidity

To date, we have financed our operations primarily through the sale of redeemable preferred units and member units, the sale of common stock and prefunded warrants to purchase shares of our common stock in private placements and the sale of common stock in

our initial public offering and a follow-on public offering. Through September 30, 2021, 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 \$471.3 million of net proceeds from the sale of our common stock through public offerings, including our IPO, private placements, our ATM Sales Agreement and pursuant to the stock purchase agreement with Ultragenyx, as detailed in the following paragraphs.

We completed our initial public offering on January 30, 2018, in which we sold 8,984,375 shares of common stock, including the underwriters' over-allotment option, at a public offering price of \$16.00 per share, resulting in net proceeds of \$129.1 million.

On July 30, 2019, we issued and sold in a private placement (i) 10,607,525 shares of our common stock at a price per share of \$4.65 and (ii) 2,295,699 pre-funded warrants to purchase shares of our common stock at a price per warrant of \$4.64. Each pre-funded warrant is exercisable for one share of common stock at an exercise price of \$0.01 and the pre-funded warrants have no expiration date. We received \$57.9 million of net proceeds from the private placement after deducting offering costs. On October 2, 2020, 137,460 of these pre-funded warrants were exercised.

On October 22, 2020, we entered into the Collaboration Agreement with Ultragenyx. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement with Ultragenyx, pursuant to which we issued and sold 7,825,797 shares of our common stock to Ultragenyx for an aggregate purchase price of approximately \$40 million.

On December 15, 2020, we issued and sold in a private placement 24,324,320 shares of our common stock at a price per share of \$3.70. We received \$86.2 million of net proceeds from the private placement after deducting offering costs.

On March 13, 2019, we entered into the ATM Sales Agreement under which we may offer and sell, from time to time, shares of our common stock having aggregate gross proceeds of up to \$50.0 million through Jefferies as sales agent. Any such sales being made by any method that is deemed an "at-the-market offering" as defined in Rule 415 promulgated under the Securities Act. We 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 nine months ended September 30, 2021, we did not sell any shares pursuant to the ATM Sales Agreement. During the year ended December 31, 2020, we sold 6,309,632 shares pursuant to the ATM Sales Agreement resulting in net proceeds of \$23.2 million.

On March 23, 2021, we issued and sold in a public offering 25,000,000 shares of our common stock at a price per share of \$5.75, including the full exercise by the underwriters of an option to purchase additional shares of common stock, or the March 2021 Offering. We received net proceeds of approximately \$134.9 million after deducting underwriter discounts and commissions and offering expenses.

As of September 30, 2021, we had cash, cash equivalents and available-for-sale securities of \$229.8 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,		Increase (decrease)
	2021	2020	
Cash used in operating activities	\$ (56,890)	\$ (57,906)	\$ 1,016
Cash (used in) provided by investing activities	(121,262)	6,660	(127,922)
Cash provided by financing activities	134,919	—	134,919
Net decrease in cash, cash equivalents and restricted cash	\$ (43,233)	\$ (51,246)	\$ 8,013

Operating activities

During the nine months ended September 30, 2021, operating activities used \$56.9 million of cash, primarily resulting from our net loss of \$53.6 million and cash used in changes in our operating assets and liabilities of 16.0 million offset by non-cash charges of \$12.7 million due to equity-based compensation of \$10.0 million, depreciation expense of \$2.2 million and amortization on available for sale securities of \$0.5 million. Net cash used in changes in our operating assets and liabilities during the nine months ended September 30, 2021 consisted primarily of a decrease in accrued expenses and other liabilities of \$4.8 million, a decrease in deferred revenue of \$9.6 million, an increase in accounts receivable of \$0.6 million and an increase prepaid expenses and other assets of \$1.6 million, offset by an increase in accounts payable of \$0.6 million.

During the nine months ended September 30, 2020, operating activities used \$57.9 million of cash, primarily resulting from our net loss of \$66.9 million and cash used in changes in our operating assets and liabilities of \$3.2 million offset by non-cash charges of

\$12.2 million due primarily to equity-based compensation of \$9.1 million and depreciation expense of \$3.1 million. Net cash used in changes in our operating assets and liabilities during the nine months ended September 30, 2020 consisted primarily of a decrease in accrued expenses and other liabilities of \$2.0 million and a decrease in accounts payable of \$2.6 million partially offset by a net decrease in prepaid expenses and other assets of \$1.5 million. These changes were primarily due to the timing of payments.

Investing activities

During the nine months ended September 30, 2021, investing activities used \$121.2 million of cash, resulting from purchases of available-for-sale securities and property and equipment.

During the nine months ended September 30, 2020, investing activities provided \$6.7 million of cash, consisting primarily of proceeds from maturities of available-for-sale securities partially offset by purchases of property and equipment, and available-for-sale securities.

Financing activities

During the nine months ended September 30, 2021, financing activities provided \$134.9 million of cash resulting from the March 2021 Offering.

During the nine months ended September 30, 2020, we received no cash from financing activities.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing development activities related to SGT-001, SGT-003 and our other product candidates. In addition, we expect to incur additional 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 IGNITE DMD and continue clinical development of SGT-001;
- move SGT-003 and other product candidates into clinical trials;
- continue research and preclinical development of SGT-003 and our other product candidates;
- seek to identify additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- arrange for manufacture of larger quantities of our product candidates for 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, technologies and intellectual property;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to the Collaboration Agreement; and
- add operational, financial and management information systems and personnel.

As of September 30, 2021, we had cash, cash equivalents and available-for-sale securities of \$229.8 million. We believe that our cash, cash equivalents and available-for-sale securities as of September 30, 2021 will be sufficient to fund our operating expenses and capital requirements into the second quarter of 2023. 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 SGT-001, SGT-003 and our other product candidates and programs and because the extent to which we may enter collaborations with third parties for development of our product candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements will depend on many factors, including:

- the progress and results of IGNITE DMD and future clinical trials of SGT-001, SGT-003 and our other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001, SGT-003 and our other product candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for SGT-003 and our other product candidates that we may pursue in the future, if any;
- the costs associated with our manufacturing process development and evaluation of third-party manufacturers;
- whether we decide to construct and validate our own manufacturing facility and the associated costs;
- revenue, if any, received from commercial sale of SGT-001, SGT-003 or our other product candidates, should any of our product 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 product candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to the COVID-19 pandemic and its collateral consequences.

We are supplying, and expect to continue to supply, our clinical development program for SGT-001 with drug product produced at a cGMP compliant facility located at one of our contract manufacturing organizations. We intend to establish the capability and capacity to supply SGT-001 at commercial scale from multiple sources.

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

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

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

Recently Issued Accounting Pronouncements

See Note 2 to the condensed consolidated financial statements included elsewhere in this quarterly report on Form 10-Q for information regarding recently adopted and issued accounting pronouncements. See also Note 2 to our consolidated financial statements included in our Annual Report on Form 10-K for the year ended December 31, 2020.

Emerging Growth Company Status

The Jumpstart Our Business Startups Act of 2012, or the JOBS Act, permits an “emerging growth company” such as us to take advantage of an extended transition period to comply with new or revised accounting standards applicable to public companies until those standards would otherwise apply to private companies. We have irrevocably elected to “opt out” of this provision and, as a result, we will comply with new or revised accounting standards when they are required to be adopted by public companies that are not emerging growth companies.

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 changes in interest rates. As of September 30, 2021, our cash equivalents consisted of money market accounts that have contractual maturities of less than 90 days from the date of acquisition. As of September 30, 2021, our investments consisted of corporate bond securities 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 interim 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, 2021. 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, 2021, our President and Chief Executive Officer and our interim 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

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2021 that has materially affected, or is 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.

RISK FACTOR SUMMARY

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

- We have incurred significant net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future and may never achieve or maintain profitability.
- We will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.
- Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in our 2020 Annual Report on Form 10-K.
- We have never generated revenue from product sales and do not expect to do so for the next several years, if ever.
- The ongoing COVID-19 pandemic may affect our ability to initiate and complete current or future preclinical studies or clinical trials, disrupt regulatory activities or have other adverse effects on our business and operations.
- In November 2019, the FDA placed IGNITE DMD on clinical hold after we reported a serious adverse event in the clinical trial. Even though the clinical hold was lifted in October 2020 and treatment of patients resumed in February 2021, we cannot guarantee that similar events will not happen in the future.
- SGT-001 is a gene transfer candidate based on a novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- Success in preclinical studies or early clinical trials, including our IGNITE DMD clinical trial, may not be indicative of results obtained in later trials.
- 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.
- Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize SGT-001, SGT-003 or our other product candidates and the approval may be for a more narrow indication than we seek.
- We face significant competition.
- We have limited gene transfer 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-001, SGT-003 or our other product candidates.

- Although we may establish our own manufacturing facility, 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.
- Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our SGT-001 gene transfer product candidate or other gene transfer product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for SGT-001 or other gene transfer product candidates.
- We rely heavily on our license agreements for rights to intellectual property granted to us by others to develop and commercialize SGT-001.
- If we are unable to obtain and maintain patent protection for our product 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 product candidates may be adversely affected.

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 \$53.6 million for the nine months ended September 30, 2021. Our net losses were \$88.3 million and \$117.2 million for the years ended December 31, 2020 and 2019, respectively. As of September 30, 2021, we had an accumulated deficit of \$458.1 million. To date, we have devoted substantially all of our efforts to research and development, including clinical development of our gene transfer product candidate, SGT-001, as well as to building out our management team and infrastructure. We expect that it could be several years, if ever, before we have a commercialized product. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if, and as, we:

- continue to enroll patients in IGNITE DMD and continue clinical development of SGT-001;
- move SGT-003 or our other product candidates into clinical trials;
- continue research and preclinical development of SGT-003 or our other product candidates;
- seek to identify additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- arrange for manufacture of larger quantities of our product candidates for 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, technologies and intellectual property;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to our collaboration and license agreement with Ultragenyx; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, and our expenses will increase substantially as we continue IGNITE DMD and complete future clinical trials of SGT-001, SGT-003 and our other product candidates, obtain marketing approval for SGT-001, SGT-003 or our other product candidate, develop and validate commercial-scale manufacturing processes, manufacture, market and sell any future product candidates for which we may obtain marketing approval and satisfy any post-marketing requirements. We may never succeed in any or all 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, SGT-001, SGT-003 and our other product candidates. In addition, if we obtain marketing approval for SGT-001, SGT-003 and our other product candidates, we expect to incur significant expenses related to product sales, marketing, manufacturing and distribution. We also 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, 2021 will be sufficient to fund our operating expenses and capital requirements into the second quarter of 2023, 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 SGT-001, SGT-003 and our other product candidates.

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

- the progress and results of IGNITE DMD and future clinical trials of SGT-001, SGT-003 and our other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001, SGT-003 and our other product candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for SGT-003 and our other product candidates that we may pursue in the future, if any;
- the costs associated with our manufacturing process development and evaluation of third-party manufacturers;
- whether we decide to construct and validate our own manufacturing facility and the associated costs;
- revenue, if any, received from commercial sale of SGT-001, SGT-003 or our other product candidates, should any of our product 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 product candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to the COVID-19 pandemic and its collateral consequences.

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

Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in our 2020 Annual Report on Form 10-K.

The report from our independent registered public accounting firm for the year ended December 31, 2020 included an explanatory paragraph stating that our losses from operations and required additional funding to finance our operations raise substantial doubt about our ability to continue as a going concern. See Note 1 to the consolidated financial statements included in our 2020 Annual Report on Form 10-K for additional information. Given our planned expenditures, our independent registered public accounting firm may conclude, in connection with the audit of our financial statements for the year ended December 31, 2021 or any other subsequent period, that there is substantial doubt regarding our ability to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited financial statements, and it is likely that investors will lose all or a part of their investment. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms or at all.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies, SGT-001, SGT-003 or our other product 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, SGT-001, SGT-003 or our other product 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 next several years, 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, SGT-001, SGT-003 and any other product candidates that we may pursue in the future. We do not anticipate generating revenue from product sales for the next several years, if ever. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing research and development of SGT-001, SGT-003 and our other product candidates in a timely and successful manner;
- seeking and obtaining regulatory and marketing approvals for any product candidates for which we complete clinical trials;
- launching and commercializing SGT-001, SGT-003 and any other product 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 process for SGT-001, SGT-003 and our other product candidates that is compliant with cGMPs;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the commercial demand for SGT-001, SGT-003 and our other product candidates, if approved;
- obtaining market acceptance, if and when approved, of SGT-001, SGT-003 or any other product candidate 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 SGT-001, SGT-003 and our other product 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.

We are a development-stage company founded in 2013. Our operations to date, with respect to the development of SGT-001 and other potential product candidates, have been limited to organizing and staffing our company, business planning, raising capital, acquiring rights to our technology, identifying SGT-001 as a potential gene transfer product candidate and undertaking preclinical studies and a clinical trial of that product candidate and establishing research and development and manufacturing collaborations. We have not yet demonstrated the ability to complete clinical trials of SGT-001 or any other product candidate, obtain marketing approvals, manufacture a commercial-scale product 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.

The ongoing COVID-19 pandemic may affect our ability to initiate and complete current or future preclinical studies or clinical trials, disrupt regulatory activities or have other adverse effects on our business and operations. In addition, this pandemic may continue to adversely impact economies worldwide, which could result in adverse effects on our business and operations.

The ongoing COVID-19 pandemic has caused many governments to implement measures to slow the spread of the outbreak through quarantines, travel restrictions, heightened border scrutiny, and other measures. The outbreak and government measures taken in response have also had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. The future progression of the outbreak and its effects on our business and operations are uncertain.

We and our third-party manufacturers for our SGT-001 supply and prospective contract research organizations, or CROs, may face disruptions 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 our product 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 IGNITE DMD or future clinical trials arising from delays in IND-enabling studies, manufacturing disruptions, and the ability to obtain necessary institutional review board or other necessary site approvals, as well as other delays at clinical trial sites.

We may also face difficulties recruiting or enrolling patients for our IGNITE DMD or future clinical trials if patients are affected by the COVID-19 virus or are fearful of visiting or traveling to clinical trial sites because of the outbreak. For example, we experienced a few missed or postponed patient visits due to site closures early in the COVID-19 pandemic. As trial sites and families have developed strategies for safety during the pandemic, we have not seen missed or postponed visits in recent months.

The response to the COVID-19 pandemic 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. For example, the FDA has announced that in order to bring new therapies to patients sick with COVID-19 as quickly as possible, it has redeployed medical and regulatory staff from other areas to work on COVID-19 therapies. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

We have modified our business practices, including implementing a work from home policy for all employees who are able to perform their duties remotely. We expect to continue to take actions as may be required or recommended by government authorities or as we determine are in the best interests of our employees, and other business partners in light of COVID-19. In the event of a continuation of shelter-in-place orders and/or other mandated local travel restrictions, our employees conducting research and development activities may not be able to access our research space, and our core activities may be significantly limited or curtailed, possibly for an extended period of time.

The pandemic has already caused significant disruptions in the financial markets, and may continue to cause such disruptions, which could impact our ability to raise additional funds through public offerings and may also impact the volatility of our stock price and trading in our stock. Moreover, it is possible the pandemic will significantly impact economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the COVID-19 pandemic will be on our business and it has the potential to adversely affect our business, financial condition, results of operations and prospects.

Finally, in response to the COVID-19 pandemic, the FDA issued guidance on March 18, 2020, and updated it on July 2, 2020, January 27, 2021, and August 30, 2021, to address the conduct of clinical trials during the pandemic. The guidance sets out a number of considerations for sponsors of clinical trials impacted by the pandemic, including the requirement to include in the clinical study report (or as a separate document) contingency measures implemented to manage the study, and any disruption of the study as a result of COVID-19; a list of all study participants affected by COVID-19-related study disruptions by a unique subject identifier and by investigational site, and a description of how the individual's participation was altered; and analyses and corresponding discussions that address the impact of implemented contingency measures (e.g., participant discontinuation from investigational product and/or study, alternative procedures used to collect critical safety and/or efficacy data) on the safety and efficacy results reported for the study. In its most recent update to this guidance, FDA addresses questions received during the past year from clinical practitioners who are adapting their operations in a pandemic environment. These questions focused on, among other things, when to suspend, continue or initiate a trial and how to submit changes to protocols for INDs and handle remote site monitoring visits. There is no assurance that this guidance governing clinical studies during the pandemic will remain in effect or, even if it does, that it will help address the risks and challenges enumerated above.

Risks related to the development of our product candidates

In November 2019, the FDA placed IGNITE DMD on clinical hold after we reported a serious adverse event in the clinical trial. Even though the clinical hold was lifted in October 2020 and treatment of patients resumed in February 2021, we cannot guarantee that similar events will not happen in the future.

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 October 2020, the FDA lifted the clinical hold placed on IGNITE DMD. In connection with the lifting of the clinical hold, we reduced the maximum weight of the next two patients dosed in IGNITE DMD to 18 kg per patient. Additionally, to mitigate the risk of serious drug-related adverse events, we amended the IGNITE DMD clinical protocol to include the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and increase the prednisone dose in the first month post dosing. In March 2021, we announced that a seventh patient was safely dosed under the amended protocol, with transient and manageable adverse events, none of which were serious. 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. This type of event is described in our Investigators Brochure and is not considered unexpected. Following dosing of these two patients with our second-generation manufacturing process and clinical strategy, we conducted an extensive review of all clinical data, resulting in a strengthened risk mitigation plan including new patient guidance, which has also been submitted to the FDA. We cannot guarantee that similar serious adverse events or clinical holds will not happen in the future.

Even though the FDA lifted the clinical hold in October 2020, additional preclinical studies or clinical trials involving SGT-001, further amendments to the SGT-001 enrollment criteria and/or clinical trial protocol or changes to our manufacturing process may be needed, which may prove difficult to implement and/or complete. In such instance, our progress in the development of SGT-001 may be significantly slowed or stopped and the associated costs may be significantly increased, adversely affecting our business, and our stock price would likely decline. Furthermore, even though the FDA lifted the clinical hold, we may nonetheless face difficulties in recruiting patients to enroll in, or retain patients in IGNITE DMD if patients or their caregivers are affected by the COVID-19 virus or are fearful of traveling to, or are unable to travel to, our clinical trial sites because of the COVID-19 pandemic.

In addition, we may not be able to obtain institutional review board committee, or IRB, or data safety monitoring board approvals for IGNITE DMD as a result of the now lifted clinical hold or any related risks, which could further delay our ability to open new trial sites and enroll patients into the clinical trial. Any delay in enrolling patients or inability to continue or complete our clinical trial of SGT-001, as a result of the now lifted clinical hold or otherwise, will delay or terminate our clinical development plans for SGT-001, may require us to incur additional clinical development costs and could impair our ability to ultimately obtain FDA approval for SGT-001. Delays in the completion of any clinical trial of SGT-001 or any other product candidate will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of SGT-001, SGT-003 or our other product candidates.

SGT-001 is a gene transfer candidate based on a 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 have concentrated our research and development efforts on SGT-001 for the treatment of Duchenne and our future success depends on our successful development of that product candidate. Our risk of failure is high. We have experienced, and may in the future experience, problems or delays in developing SGT-001. 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 SGT-001, the adeno-associated virus, or AAV, vector, toxicity or other issues 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, 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 SGT-001 in either the United States or the European Union. Approvals by the European Commission may not be indicative of what the FDA may require for approval and vice versa.

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

During the conduct of clinical trials, patients may experience changes in their health, including illnesses, injuries, discomforts or a fatal outcome. Often, it is not possible to determine whether the product candidate being studied caused these conditions. For instance, we reported a serious adverse event in IGNITE DMD, which resulted in a clinical hold in November 2019, which has since been resolved, and previously the FDA had placed IGNITE DMD on clinical hold after we reported another serious adverse event. In April 2021, the eighth patient treated with IGNITE DMD 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.

In addition, it is possible that as we test SGT-001, SGT-003 or our other product candidates in larger, longer and more extensive clinical programs, or as use of these product 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 III 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 SGT-001 or any other product candidate has side effects or causes serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked.

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 using other vectors. While new recombinant vectors 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. More recently, 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. Patients will create antibodies to the AAV vector and a second administration of gene transfer might not be successful. 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 vectors 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 candidate demonstrates a similar effect, we may decide or be required to halt or delay further clinical development of SGT-001.

As part of our preclinical program, we performed necessary good laboratory practices, or GLP, toxicology studies to establish the overall safety profile of SGT-001 in wild-type mice and non-human primates, or NHPs. The data and our conclusions from these studies were included in our IND submission to the FDA. Systemic administration of SGT-001 was generally well tolerated in both species. We observed no evidence of test-article-related toxicity for up to 13 weeks after systemic administration of SGT-001 in either species that would prevent us from initiating clinical trials. In the NHP study, test-article-related effects were self-limited, mild chemistry and hematology changes with no microscopic correlates at the end of the study. There was a transient and asymptomatic increase in liver function enzymes observed in NHPs starting on day 9, which returned to normal levels by day 21. We believe there were no other relevant test-article-related adverse events associated with SGT-001 administration in either GLP study. In the NHP toxicology study, a single animal from the high dose cohort was euthanized after it did not recover from an anesthetic procedure. We believe this event was attributed to procedural errors. However, AAV vector cannot be completely ruled out as a contributing factor to the toxicity that gave rise to the event.

In addition to side effects caused by SGT-001, SGT-003 and our other product candidates, the administration process or related procedures also can cause adverse side effects. For example, integration of AAV DNA into the host cell's genome has been reported to occur. Further, our AAV delivery system has not been validated in human clinical trials previously, and if such 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-001. In addition, the relatively high dosing requirements for SGT-001 may amplify the risk of adverse side effects relating to the AAV vector. When James M. Wilson, M.D., Ph.D., resigned from our Scientific Advisory Board in early 2018 he cited emerging concerns about the possible risks of high systemic dosing of AAV. If any such adverse side effects were to occur in the future and we are unable to demonstrate that they were not caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, SGT-001 or any other product candidate 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.

Additionally, if SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 or our other product candidates, several potentially significant negative consequences could result, including:

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

We have never completed a clinical trial, and may be unable to do so for any product candidates we may develop, including SGT-001.

We will need to successfully complete clinical trials in order to obtain FDA approval to market SGT-001, SGT-003 or our other product candidates. We have limited experience in preparing, submitting and prosecuting regulatory filings, and have not previously submitted a biologics license application, or BLA, for any product candidate. We cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin or to begin as proposed, or that, once begun, issues will not arise that suspend or terminate such clinical trials. Carrying out later-stage clinical trials and the submission of a successful BLA is a complicated process. This may be particularly true for design of a pivotal trial for the treatment of Duchenne as the FDA has not given clear guidance as to the necessary endpoints for approval of a treatment for Duchenne. In addition, we cannot be certain how many clinical trials of SGT-001, SGT-003 or our other product 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-001, SGT-003 or our other product candidates. We may require more time and incur greater costs than our competitors and may not succeed in obtaining regulatory approvals of product candidates that we develop. Failure to commence or complete, or delays in, clinical trials, could prevent us from or delay us in commercializing SGT-001, SGT-003 and our other product candidates.

In the past, we have made changes to the IGNITE DMD protocol, and these changes, and any other such changes that may be made in the future, may impact our development timeline and result in increased costs and expenses.

Success in preclinical studies or early clinical trials, including our IGNITE DMD clinical trial, may not be indicative of results obtained in later trials.

Results from preclinical studies or early clinical trials, including our IGNITE DMD clinical trial, are not necessarily predictive of future clinical trial results and are not necessarily indicative of final results. Our preclinical studies for SGT-001 in animals have been limited and we have only dosed a limited number of human subjects with SGT-001. There is a high failure rate for gene therapy and biologic products proceeding through clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. We also may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. SGT-001, SGT-003 or our other product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies. This failure would cause us to abandon SGT-001, SGT-003 or our other product candidates.

Preliminary or interim data that we announce or publish from time to time may change as more data become 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 become available. Additionally, preliminary or interim data are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become 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 same 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 SGT-001, SGT-003 or our other product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indications. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in 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 IGNITE DMD for SGT-001;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials, including because such trials may be placebo-controlled trials and patients are not guaranteed to receive treatment with our product candidates;
- 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 SGT-001, SGT-003 or our other product 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;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- delays as a result of the COVID-19 pandemic or from the outbreak of another pandemic or contagious disease or other global instability could delay IGNITE DMD, or the commencement or rate of completion of any other 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 SGT-001, SGT-003 or our other product candidates, we may:

- be delayed or fail in obtaining marketing approval for SGT-001, SGT-003 or our other product 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.

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 SGT-001, SGT-003 or our other product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. We do not know whether any of our preclinical studies or clinical trials will begin as planned, will need to be restructured or will be completed on schedule, or at all. We may also determine to change the design or protocol of one or more of our clinical trials, which could result in delays. Significant preclinical study or clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do and impair our ability to successfully commercialize our product candidates.

If our third-party clinical trial vendors fail to comply with strict regulations, the clinical trials for SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 or our other product candidates. For IGNITE DMD 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, the clinical trials for SGT-001, SGT-003 or our other product 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-001, SGT-003 or our other product candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-001, SGT-003 and our other product candidates is critical to our success. 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 product candidates, other approved gene therapies or the biotechnology or gene therapy fields, or due to competitive clinical trials for similar patient populations, clinical trials in products employing our vector or our platform or for other reasons, the timeline for recruiting patients, conducting clinical trials and obtaining regulatory approval of SGT-001 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 retaining patients in, IGNITE DMD if they or their caretakers are affected by the COVID-19 virus or are fearful of traveling to, or are unable to travel to, our clinical trial sites because of the COVID-19 pandemic. These delays could result in increased costs, delays in advancing SGT-001, SGT-003 or our other product candidates, delays in testing the effectiveness of SGT-001, SGT-003 and our other product 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 that some patients may have pre-existing antibodies to AAV vectors precluding them from being able to receive AAV-mediated gene transfer;
- restrictions on our ability to conduct clinical trials, including full and partial clinical holds on ongoing or planned clinical trials;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of gene therapy-based approaches to the treatment of diseases;
- release or disclosure of data from our completed or ongoing clinical trials;
- availability of competing therapies and clinical trials;
- severity of the disease;
- proximity and availability of clinical trial sites for prospective subjects;

- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians;
- ability to monitor subjects adequately during and after treatment; and
- in the case of pivotal trials, the risk that patients may opt not to enroll because they are not assured treatment with our product candidate.

In November 2019, the FDA placed our IGNITE DMD clinical trial of SGT-001 on clinical hold following our report of a serious adverse event in the clinical trial. In April 2020, we submitted a response to the FDA, that included changes to the clinical protocol designed to potentially enhance patient safety, as well as information related to improvements to our manufacturing process. The FDA responded by maintaining the clinical hold and requesting further data and analyses relating to this manufacturing process. In June 2020, we submitted a response to the FDA that provided data and analyses related to improvements to our manufacturing process. In July 2020, we announced that the FDA responded by maintaining the clinical hold and requesting further manufacturing information and updated safety and efficacy data for all patients dosed in the trial, as well as providing direction on the total viral load to be administered per patient. In October 2020, we announced that the FDA lifted the clinical hold placed on the IGNITE DMD clinical trial. Even though the FDA lifted the clinical hold, additional preclinical studies or clinical trials involving SGT-001, further amendments to the SGT-001 enrollment criteria and/or clinical trial protocol or changes to our manufacturing process may be needed from time to time, which may prove difficult to implement and/or complete, and we may face difficulties in recruiting and enrolling such patients. 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 SGT-001, SGT-003 or our other product candidates and the approval may be for a more narrow indication than we seek.

We cannot commercialize SGT-001, SGT-003 or our other product candidates until the appropriate regulatory authorities have reviewed and approved the product candidate. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state and local statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA advisory committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action or changes in regulatory authority policy during the period of product development, clinical trials and the regulatory review process.

Further, disruptions at the FDA and other agencies may prolong the time necessary for new drugs to be reviewed or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. The Trump Administration also took several executive actions that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities, but those Executive Orders were rescinded by President Biden.

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

Even if we obtain regulatory approval for a product candidate, our product candidates will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for SGT-001, SGT-003 or our other product candidates, we will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates may also be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or conditions of approval, or requirements for potentially costly post-marketing testing, including Phase IV clinical trials, and surveillance to monitor the quality, safety and efficacy of the 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.

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.

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.

Even if we obtain and maintain approval for SGT-001, SGT-003 or our other product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States, which would limit our market opportunities and adversely affect our business.

Even if we receive FDA approval of SGT-001, SGT-003 or our other product candidates in the United States, approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in

other foreign countries or by the FDA. Future sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approval. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. We intend to submit a marketing authorization application, or MAA, to the EMA for approval of SGT-001 in the European Union, but 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 SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 or our other product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced, and our ability to realize the full market potential of our product candidates will be harmed.

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

The FDA has established the Office of Tissues and Advanced Therapies, or the OTAT, within the Center for Biologics Evaluation and Research, or the CBER, to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, also are potentially subject to review by the Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC; however, the NIH announced that the RAC will only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct a clinical trial, that institution's institutional biosafety committee, or IBC, as well as its IRB would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of our product candidates.

The FDA has issued various guidance documents regarding gene therapies, including recent 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. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy product candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire.

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

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

of their products to the EMA, which provides an opinion regarding the MAA. The European Commission grants or refuses marketing authorization in light of the opinion delivered by the EMA.

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

As we advance our product candidates through clinical development, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. These regulatory review committees and advisory groups and any new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of SGT-001, SGT-003 or our other product candidates or lead to significant post-approval limitations or restrictions. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue.

We may not be able to benefit from orphan drug designation for SGT-001 or any of our product candidates.

The FDA and EMA granted SGT-001 orphan drug designation for the treatment of Duchenne in August 2016 and September 2016, respectively. The designation of SGT-001 as an orphan drug does not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan drug designation to product candidates of other companies that treat the same indications as our product candidate prior to our product candidate receiving exclusive marketing approval.

We may lose orphan drug exclusivity if the FDA or EMA determines that the request for designation was materially defective or if we cannot assure sufficient quantity of the applicable drug to meet the needs of patients with Duchenne.

Even if we maintain orphan drug exclusivity for SGT-001 or obtain orphan drug exclusivity for any other product candidate, the exclusivity may not effectively protect the product candidate from competition because regulatory authorities still may authorize different drugs for the same condition or the same drug for the same condition if it is determined by the FDA to be clinically superior to the product with orphan drug exclusivity. Moreover, the concept of what constitutes the “same drug” for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA issued draft guidance in January 2020 suggesting that it would not consider two gene therapy products to be different drugs solely based on minor differences in the transgenes or vectors.

We may seek a breakthrough therapy designation for SGT-001, SGT-003 or our other product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

We may seek a breakthrough therapy designation for SGT-001, SGT-003 or our other product candidates; however, we cannot assure our stockholders that SGT-001, SGT-003 or our other product candidates will meet the criteria for that designation. A breakthrough therapy is defined as a therapy that is intended, alone or in combination with one or more other therapies, to treat a serious condition, and preliminary clinical evidence indicates that the therapy may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For therapies and biologics that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Therapies designated as breakthrough therapies by the FDA may also be eligible for priority review if supported by clinical data at the time the new drug application is submitted to the FDA.

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

Accelerated approval by the FDA, even if granted for SGT-001, SGT-003 or our other product candidates, may not lead to a faster development or regulatory review or approval process and it does not increase the likelihood that our product candidates will receive marketing approval.

We may seek approval of SGT-001, SGT-003 or our other product candidates using the FDA's accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate an effect on a surrogate 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 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.

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. 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.

Even if we do receive accelerated approval, we may not experience a faster development or regulatory review or approval process and receiving accelerated approval does not provide assurance of ultimate FDA approval.

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

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

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

The FDA has granted Rare Pediatric Disease Designation, or RPDD, to SGT-001; however, a BLA for SGT-001 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.

For the purposes of this program, a “rare pediatric disease” is a (a) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (b) rare disease or conditions within the meaning of the Orphan Drug Act. The FDA has granted Rare Pediatric Disease designation to SGT-001. The FDA may determine, however, that a BLA for SGT-001, SGT-003 or our other product candidates does not meet the eligibility criteria for a priority review voucher upon approval.

The passage of the 21st Century Cures Act in December 2016 extended the Rare Pediatric Disease Priority Review Voucher Program, authorizing the FDA to award vouchers through September 30, 2022, for drugs with rare pediatric disease designation granted by September 30, 2020. On September 30, 2020, Congress provided a short-term extension of the Priority Review Voucher Program. On December 27, 2020, the Rare Pediatric Disease Priority Review Voucher Program was further extended. Under the current statutory sunset provisions, after September 30, 2024, FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has rare pediatric disease designation for the drug, and that designation was granted by September 30, 2024. After September 30, 2026, FDA may not award any rare pediatric disease priority review vouchers. If we do not obtain approval of a BLA by these dates, and if the Rare Pediatric Disease Priority Review Voucher Program is not further extended by congressional action, we may not receive a Priority Review Voucher.

The FDA has granted fast track designation for SGT-001. However, such designation may not actually lead to a faster development or regulatory review or approval process. We might not receive such designation for SGT-003 or our other product candidates.

If a therapy is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a drug sponsor may apply for FDA fast track designation. The FDA has granted fast track designation to SGT-001; however, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. 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. Fast track designation alone does not guarantee qualification for the FDA’s priority review procedures.

We may seek priority review designation for SGT-001, SGT-003 or our other product candidates, but we might not receive such designation, and even if we do, such designation may not lead to a faster development or regulatory review or approval process.

If the FDA determines that a product candidate offers a treatment for a serious condition and, if approved, the product would provide a significant improvement in safety or effectiveness, the FDA may designate the product candidate for priority review. A priority review designation means that the goal for the FDA to review an application is six months, rather than the standard review period of ten months. We may request priority review for our product candidates, however, we cannot assume that SGT-001, SGT-003 or our other product candidates will meet the criteria for that designation. The FDA has broad discretion with respect to whether or not to grant priority review status to a product candidate, so even if we believe a particular product candidate is eligible for such designation or status, the FDA may decide not to grant it. Moreover, a priority review designation does not necessarily mean a faster development or regulatory review or approval process or necessarily confer any advantage with respect to approval compared to conventional FDA procedures. Receiving priority review from the FDA does not guarantee approval within the six-month review cycle or at all.

Inadequate funding for the FDA, the SEC and other 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 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, over the last several years 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 FDA, SEC and other government employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. Further, future government shutdowns 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 successfully market or commercialize SGT-001, SGT-003 or our other product candidates.

We operate in a highly competitive segment of the biopharmaceutical market. We face competition from many different sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, government agencies and private and public research institutions. Our product candidates, if successfully developed and approved, will compete with established therapies as well as with new treatments that may be introduced by our competitors. There are a variety of product candidates, including gene therapies, in development for Duchenne. 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, as well as in acquiring technologies complementary to, or necessary for, our programs.

For example, we are aware of several companies and research institutions focused on developing systemic gene transfers for Duchenne, including Pfizer Inc. and Sarepta Therapeutics, Inc. with product candidates currently in Phase III clinical development, and Genethon with a product candidate currently in Phase I/II/III clinical trial development.

Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, have broader market acceptance, are more convenient or are less expensive than any product candidate that we may develop.

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 SGT-001 or any future gene therapy product candidates we develop.

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

The success of our business depends upon our ability to develop and commercialize SGT-001, SGT-003 and our other product candidates. Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential than SGT-001, SGT-003 or our other product candidates. For example, in January 2020, in connection with implementing our strategic plan to create a leaner company focused on advancing SGT-001, we curtailed certain activities supporting our other research and development programs.

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

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

Risks related to the manufacturing and commercialization of SGT-001, SGT-003 and our other product candidates

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

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

While we have retained all rights to and are developing on our own SGT-001, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to SGT-001, SGT-003 or future product 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 product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

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

- collaborators have significant discretion in determining the amount and timing of efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development of our product candidates or may elect not to continue or renew development programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;

- collaborators may not pursue commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew commercialization programs based on results of clinical trials or other studies, changes in the collaborators' strategic focus or available funding, or external factors, such as an acquisition, that may divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates on a discretionary basis;
- collaborators, including Ultragenyx, could develop products that compete directly or indirectly with our product candidates and products pursuant to the collaboration;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates and products if the collaborators believe that the competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates discovered in collaboration with us may be viewed by our collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator may fail to comply with applicable regulatory requirements regarding the development, manufacture, distribution or marketing of a product candidate or product;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over intellectual property or proprietary rights, contract interpretation or the preferred course of development, might cause delays or terminations of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly obtain, maintain, enforce, defend or protect our intellectual property or proprietary rights or may use our proprietary information in such a way as to potentially lead to disputes or legal proceedings that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership of intellectual property developed pursuant to our collaborations;
- collaborators may infringe, misappropriate or otherwise violate the intellectual property or proprietary rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated for the convenience of the collaborator, and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

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

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

We may not be successful in finding strategic collaborators for continuing development of SGT-001, SGT-003 or our other product candidates or successfully commercializing or competing in the market for certain indications.

We may seek to establish strategic partnerships for developing SGT-001, SGT-003 or our other product candidates due to capital costs required to develop, manufacture and commercialize our product candidates. We may not be successful in our efforts to establish such strategic partnerships or other alternative arrangements because, among other things, our research and development

pipeline may be insufficient, SGT-001 may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view SGT-001 as having the requisite potential to demonstrate safety and efficacy. We cannot be certain that, following a strategic transaction, we will achieve an economic or business benefit that justifies such transaction. If we seek to but are unable to reach agreements with suitable collaborators on a timely basis, on acceptable terms or at all, we may have to curtail, reduce or delay the development of a product candidate, delay its potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development, manufacturing or commercialization activities independently. If we elect to fund our own independent development or commercialization activities, we will need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development, manufacturing and commercialization activities, we may not be able to further develop SGT-001, SGT-003 or our other product candidates.

We have limited gene transfer 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-001, SGT-003 or our other product candidates.

The manufacturing process we use to produce SGT-001 is complex and has not been validated for commercial use. We have no experience manufacturing SGT-001, SGT-003 and our other product candidates. Building our own manufacturing facility, if we decide to do so in the future, would require substantial additional investment, would be time-consuming and may be subject to delays, including those resulting from compliance with regulatory requirements. In addition, building a manufacturing facility may cost more than we currently anticipate. Although we may establish our own manufacturing facility to support a commercial launch, if we are unable to do so or otherwise decide not to do so, we may be unable to produce commercial materials or meet demand, if any should develop, for SGT-001, SGT-003 and our other product candidates. Any such failure could delay or prevent our commercialization of SGT-001, SGT-003 or our other product candidates. The production of SGT-001 requires processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a gene transfer product 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 employ multiple steps to control our manufacturing process to assure that the process works and that SGT-001 is made strictly and consistently in compliance with the process. As a result of the limited number of FDA approvals for gene transfer products to date, the timeframe required for us to obtain approval for a cGMP gene therapy manufacturing facility in the United States is uncertain. We must supply all necessary documentation in support of a 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-001, SGT-003 and our other product candidates. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP requirements, and perform extensive audits of contract laboratories, manufacturers and suppliers.

We currently rely on third-party manufacturers for our SGT-001 supply. In order to produce sufficient quantities of SGT-001 for clinical trials and initial U.S. commercial demand, we continue to further optimize and increase the capacity of our manufacturing process at our third-party manufacturer, and potentially through our own commercial-scale manufacturing facility. We may need to change our current manufacturing process. We may not be able to produce sufficient quantities of SGT-001 due to several factors, including equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, a public health issue (for example, an outbreak of a contagious disease such as the COVID-19 pandemic), disruption in utility services, human error or disruptions in the operations of our suppliers. For example, through our contract manufacturer we have performed and released within specifications manufacturing runs of SGT-001 for clinical supply and have experienced variability with respect to the success and yield of these runs. We continue to engage in process development activities to improve the reproducibility, reliability, quality and consistency of yields of our manufacturing process. While we are able to produce for more than one patient from a single batch, additional manufacturing runs will be required to produce necessary or adequate supply for IGNITE DMD 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-001 could be delayed, and we could incur additional expense.

If supply from a manufacturing facility is interrupted, including as a result of equipment malfunctions, facility contamination, material shortages or contamination, natural disasters, the COVID-19 pandemic or another public health issue, disruption in utility services or human error, there could be a significant disruption in supply of SGT-001, SGT-003 or our other product candidates. In such instance, we may need to locate appropriate replacement third-party manufacturers, and we may not be able to enter into arrangements with such additional third-party manufacturers on favorable terms or at all. Use of new third-party manufacturers could increase the risk of delays in production or insufficient supplies of our product candidates as we transfer our manufacturing technology to these manufacturers and as they gain experience manufacturing our product candidates.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a

product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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

We also may encounter problems hiring and retaining the experienced specialist scientific, quality control and manufacturing personnel needed to operate our manufacturing 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 larger 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 SGT-001, SGT-003, our other product candidates or future product candidates.

Although we may establish our own manufacturing facility, 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.

Until such time, if ever, as we establish a manufacturing facility that has been properly validated to comply with FDA cGMP requirements, we will not be able to independently manufacture material for our current and future clinical programs. For clinical trials of SGT-001, we are utilizing, and expect to continue to utilize, and for clinical trials of SGT-003 and our other product candidates, we expect to utilize, materials manufactured by cGMP-compliant third-party suppliers. Even following our potential establishment of a validated cGMP manufacturing facility, we intend to utilize third-party manufacturing capabilities in order to provide multiple sources of supply. In the event that the establishment of our own manufacturing facility is delayed or not otherwise pursued and if these third-party manufacturers do not successfully carry out their contractual duties, meet expected deadlines or manufacture SGT-001, SGT-003 and our other product candidates in accordance with 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 SGT-001, SGT-003 and our other product candidates. In such instances, we may need to locate an appropriate replacement third-party relationship, which may not be readily available or on acceptable terms, which would cause additional delay or increased expense prior to the approval of our product candidates.

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

In addition, we do not currently have long-term supply or manufacturing arrangements in place for the production of SGT-001, SGT-003 and our other product candidates at commercial scale. Although we intend to establish additional sources for long-term supply, potentially including our own commercial-scale cGMP-compliant manufacturing facility and 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 SGT-001, SGT-003 or our other product candidates. We may experience difficulties in scaling up production beyond clinical batches. Furthermore, demand for third-party cGMP manufacturing facilities may grow at a faster rate than existing manufacturing capacity, which could disrupt our ability to find and retain third-party manufacturers capable of producing sufficient quantities of SGT-001, SGT-003 or our other product candidates for future clinical trials or to meet initial commercial demand in the United States. We currently rely, and expect to continue to rely, on additional third parties to manufacture materials for our product candidates and to perform quality testing. Even following the potential establishment of our own cGMP-compliant manufacturing capabilities, we intend to maintain third-party manufacturers for these materials, as well as to serve as additional sources of SGT-001, SGT-003 and our other product candidates, which will expose us to risks including:

- reduced control of manufacturing activities;

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

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize SGT-001, SGT-003 or our other product candidates. Some of these events could be the basis for FDA action, including injunction, recall, seizure or total or partial suspension of product manufacture.

If we are unable to establish sales, distribution and marketing capabilities or enter into agreements with third parties to market and sell SGT-001, SGT-003 and our other product candidates, we will be unable to generate any product revenue.

We currently have no sales, distribution or marketing organization. To successfully commercialize any product candidate that may result from our development programs, we will need to develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any product candidate we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaborations regarding SGT-001, SGT-003 and our other product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any future collaborators do not commit sufficient resources to commercialize our product candidates, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded sales, distribution and marketing operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of SGT-001, SGT-003 and our other product candidates. Without an internal team or the support of a third party to perform marketing and sales functions, we may 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 SGT-001, SGT-003 or our other product candidates.

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 SGT-001, SGT-003 and our other product candidates 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 SGT-001 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 Duchenne. Our understanding of the patient population with this disease is based on estimates in published literature and by Duchenne foundations. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our product candidate or patients may become increasingly difficult to identify and access.

Further, there are several factors that could contribute to making the actual number of patients who receive SGT-001 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 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 therapies for Duchenne, the exact Duchenne-wide seroprevalence is currently unknown and it varies by AAV serotype and age. We may not be able to address this potentially limiting factor for gene therapy as a treatment for certain patients.

The commercial success of any of our product candidates, including SGT-001, 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 SGT-001 will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and SGT-001 in particular, 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 these 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, SGT-001, if approved for commercial sale, will depend on multiple factors, including:

- the efficacy and safety of SGT-001 as demonstrated in clinical trials;
- the efficacy and potential and perceived advantages of SGT-001 over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which SGT-001 is approved by the FDA or the European Commission;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products to meet market demand;
- publicity concerning our product candidates or competing products and treatments;
- any restrictions on the use of our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

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

Our efforts to educate the medical community and third-party payors on the benefits of SGT-001, SGT-003 and our other product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential product candidates. If SGT-001, SGT-003 or our other product candidates are approved but fail to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenue from any such product.

Our gene transfer approach utilizes a vector 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 our SGT-001 gene transfer product candidate and other gene transfer product candidates and adversely affect our ability to conduct our business or obtain regulatory approvals for SGT-001 or other gene transfer product candidates.

Gene transfer remains a novel technology and public perception may be influenced by claims that gene transfer is unsafe, and gene transfer may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of Duchenne prescribing treatments that involve the use of SGT-001 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 of SGT-001 or demand for any product candidate we may develop. A public backlash developed against gene therapy following the death of a patient in 1999 during a gene therapy clinical trial of research subjects with ornithine transcarbamylase, or OTC, deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene. The death of the clinical trial subject was due to complications of adenovirus vector administration. Dr. James M. Wilson, former chair of our Scientific Advisory Board, was a co-investigator of the 1999 trial while he was Director of the Institute for Human Gene Therapy of the University of Pennsylvania. Serious adverse events in our

clinical trials, including the events that led to the previously-lifted clinical holds on IGNITE DMD or other clinical trials involving gene transfer products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of SGT-001, stricter labeling requirements for SGT-001 if approved and a decrease in demand for SGT-001.

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

Given the nature of biologics manufacturing, there is a risk of contamination in our manufacturing processes. Any contamination could materially adversely affect our ability to produce SGT-001 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 SGT-001 could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially and adversely affect our development timelines.

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

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We expect the cost of a single administration of gene transfer products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of SGT-001, if approved, will depend substantially, both domestically and abroad, on the extent to which the costs of SGT-001 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; 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 SGT-001, SGT-003 and our other product candidates. 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 SGT-001, SGT-003 and our other product candidates.

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

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union, Canada and other countries may put pricing pressure on us. In general, the prices of therapeutics outside the United States are substantially lower than in the United States. Other countries may allow companies to fix their own prices for therapeutics, but monitor and control company profits. Additional

foreign price controls or other changes in pricing regulations could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable product revenue.

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

If we obtain approval to commercialize SGT-001, SGT-003 and our other product candidates 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 SGT-001, SGT-003 and our other product candidates 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.

Additionally, we could face heightened risks with respect to seeking marketing approval in the United Kingdom as a result of the recent withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. The United Kingdom and European Union entered into a Trade and Cooperation Agreement in connection with Brexit that sets out certain procedures for approval and recognition of medical products in each jurisdiction. Since the regulatory framework for pharmaceutical products in the United Kingdom covering the quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of the Trade and Cooperation Agreement would prevent us from commercializing any product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for any product candidates, which could significantly and materially harm our business.

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

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

- increased operating expenses and cash requirements;

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

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

Risks related to our business operations

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

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

Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, also will be critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector 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 the associated workforce reduction announced in January 2020 could result in total costs and expenses that are greater than expected and could disrupt our business.

In January 2020, we announced a reduction in workforce by approximately one third as part of a strategic plan designed to create a leaner company focused on advancing SGT-001. We cannot guarantee that we will not have to undertake additional workforce reductions or restructuring activities in the future. Furthermore, our strategic restructuring plan may be disruptive to our operations. For example, our workforce reductions could yield unanticipated consequences, such as attrition beyond planned staff reductions, or increase difficulties in our day-to-day operations. Our workforce reductions could also harm our ability to attract and retain qualified management, scientific, clinical, manufacturing and sales and marketing personnel who are critical to our business. Any failure to attract or retain qualified personnel could prevent us from successfully developing and commercializing our product candidates in the future.

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

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development activities and, in the longer term, build a commercial infrastructure to support commercialization of SGT-001 and any other product candidate that is approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and any future product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of

talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

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

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

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

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

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

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

In addition to legislative changes resulting from the passage of the Health Care Reform Law, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 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 by one year, through 2030. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

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 ACA is an essential and inseparable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the Health Care Reform Law are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit court affirmed the lower court’s ruling that the individual mandate portion of the Health Care Reform Law is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the Health Care Reform Law. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On February 10, 2021, the Biden Administration withdrew the federal government’s support for overturning the Health Care Reform Law. On June 17, 2021, the U.S. Supreme Court rejected this challenge to the Health Care Reform Law after finding that 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.

The Trump Administration also took executive actions to undermine or delay implementation of the Health Care Reform Law, including directing federal agencies with authorities and responsibilities under the Health Care Reform Law to waive, defer, grant exemptions from, or delay the implementation of any provision of the Health Care Reform Law that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden issued a new Executive Order which directs federal agencies to reconsider rules and other policies that limit Americans’ access to health care, and consider actions that will protect and strengthen that access. Under this Executive Order, federal agencies are directed to re-examine policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the Health Care Reform Law that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the Health Care Reform Law; and policies that reduce affordability of coverage or financial assistance, including for dependents. This Executive Order also directs the U.S. Department of Health and Human Services to create a special enrollment period for the Health Insurance Marketplace in response to the COVID-19 pandemic.

Another provision of the Health Care Reform Law, generally referred to as the Physician Payment Sunshine Act or Open Payments Program, has imposed new reporting and disclosure requirements for pharmaceutical and medical device manufacturers and distributors with certain FDA-approved products, such as approved vaccines, with regard to payments or other transfers of value made to certain U.S. health care practitioners, such as physicians and academic medical centers, and with regard to certain ownership interests held by physicians in reporting entities. The CMS publishes information from these reports on a publicly available website, including amounts transferred and the physician and teaching hospital identities.

Under the Physician Payment Sunshine Act, we are required to collect and report detailed information regarding certain financial relationships we have with physicians and teaching hospitals if we obtain FDA approval for any of our products and receive reimbursement from the federal government. Our compliance with these rules may also impose additional costs.

With enactment of the Tax Cuts and Jobs Act of 2017, which was signed by President Trump on December 22, 2017, Congress repealed the “individual mandate.” The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the Health Care Reform Law-mandated “Cadillac” tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the Health Care Reform Law, effective January 1, 2019, to increase the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole.” The Congress may consider other legislation to replace elements of the Health Care Reform Law.

In addition, the CMS has proposed regulations that would give states greater flexibility in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the Health Care Reform Law for plans sold through such marketplaces. On November 30, 2018, CMS announced a proposed rule that would amend the Medicare Advantage and Medicare Part D prescription drug benefit regulations to reduce out-of-pocket costs for plan enrollees and allow Medicare plans to negotiate lower rates for certain drugs. Among other things, the proposed rule changes would allow Medicare Advantage plans to use preauthorization, or PA, and step therapy, or ST, for six protected classes of drugs, with certain exceptions; permit plans to implement PA and ST in Medicare Part B drugs; and change the definition of “negotiated prices” as well as add a definition of “price concession” in the regulations. It is unclear whether these proposed changes will be accepted, and if so, what effect such changes will have on our business.

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

The prices of prescription pharmaceuticals have been the subject of considerable discussion in the United States. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for products. To those ends, President Trump issued several executive orders intended to lower the costs of prescription drug products. Certain of these orders are reflected in recently promulgated regulations, including an interim final rule implementing President Trump's most favored nation model, but such final rule is subject to a nationwide preliminary injunction. The Biden Administration has frozen certain of the previous administration's measures to reform drug prices, pending further review. It remains to be seen how the Biden Administration will address this issue but, under Medicare Part D, the administration may seek to establish a ceiling for the launch prices of all branded, biologic, and certain generic drugs by referencing the average price of these drugs in other developed countries. At the same time, the administration may seek to limit Medicare Part D and public option drug prices through a tax penalty on manufacturers for increases in the cost of drugs and biologics above the general inflation rate. The Biden Administration has agreed to delay for a year the implementation of one of President Trump's signature drug pricing policies, from January 2022 to 2023. The policy at issue would have prevented drug makers and middlemen from negotiating rebates on prescription drugs.

More recently, on July 9, 2021, President Biden signed Executive Order 14063, which focuses on, among other things, the price of pharmaceuticals. To address these costs, the Order directs the Department of Health and Human Services, or HHS, to create a plan within 45 days to combat "excessive pricing of prescription drugs and enhance domestic pharmaceutical supply chains, to reduce the prices paid by the federal government for such drugs, and to address the recurrent problem of price gouging." Thereafter, on September 9, 2021, HHS released its plan to reduce drug prices. The key features of that plan are to: (a) make drug prices more affordable and equitable for all consumers and throughout the health care system by supporting drug price negotiations with manufacturers; (b) improve and promote competition throughout the prescription drug industry by supporting market changes that strengthen supply chains, promote biosimilars and generic drugs, and increase transparency; and (c) foster scientific innovation to promote better healthcare and improve health by supporting public and private research and making sure that market incentives promote discovery of valuable and accessible new treatments. Further, in August 2021, CMS issued a proposed rule to rescind the Trump Administration's interim final rule, following public notice and comment. With issuance of this proposal, CMS stated that it will carefully consider the comments it received on the November 2020 interim final rule as it explores all options to incorporate value into payments for Medicare Part B drugs and improve beneficiaries' access to evidence-based care.

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

The Drug Supply Chain Security Act imposes new obligations on manufacturers of pharmaceutical products related to product tracking and tracing. Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the current regulations, guidance or interpretations will be changed, or what the impact of such changes on our business, if any, may be.

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

- the demand for any product candidates for which we may obtain regulatory approval;
- our ability to set a price that we believe is fair for our products;
- our ability to obtain coverage and reimbursement approval for a product;

- our ability to generate revenue and achieve or maintain profitability; and
- the level of taxes that we are required to pay.

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

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

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

If we obtain FDA approval for SGT-001, SGT-003 or our other product candidates and begin commercializing 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 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.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the 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.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the General Data Protection Regulation, or GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and/or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Similar actions are either in place or under way in the United States. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act, which went into effect on January 1, 2020, is creating similar risks and obligations as those created by GDPR, though the Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects (the Common Rule). Many other states are considering similar legislation. A broad range of legislative measures also have been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data. 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 reputation and our business.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with such requirements is rigorous and time intensive and requires significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations. Similarly, failure to comply with federal and state laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. 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 reputation and our business.

Further, we cannot provide any assurances that our third-party service providers with access to our or our customers', suppliers', trial patients' and employees' personally identifiable and other sensitive or confidential information in relation to which we are responsible will not breach contractual obligations imposed by us, or that they will not experience data security breaches or attempts thereof, which could have a corresponding effect on our business, including putting us in breach of our obligations under privacy laws and regulations and/or which could in turn adversely affect our business, results of operations and financial condition. We cannot provide any assurances that our contractual measures and our own privacy and security-related safeguards will protect us from the risks associated with the third-party processing, storage and transmission of such information.

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

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

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

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

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

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

We face an inherent risk of product liability exposure related to the testing of SGT-001, SGT-003, our other product candidates and any future product candidate in preclinical studies and clinical trials and may face an even greater risk if we commercialize any product candidate that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

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

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

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

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

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

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

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

While we are not aware of any such material system failure, accident, cyber-attack or security breach to date, if such an event were to occur and cause interruptions in our or our collaborators', contractors' or consultants' operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from preclinical studies or clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of SGT-001, SGT-003 and our other product 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 SGT-001 and SGT-003 and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize SGT-001, SGT-003 and our other product candidates.

Our ability to develop and commercialize SGT-001, SGT-003 and other product candidates is heavily dependent on licenses to patent rights and other intellectual property granted to us by third parties. In particular, we have licensed certain patents and patent applications from the University of Michigan, the University of Missouri and the University of Washington that are important or necessary to the development of SGT-001, SGT-003 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 these agreements, we may be subject to damages, which may be significant, and the licensor may have the right to terminate the license, in which event we may not be able to develop or market product candidates or technologies covered by the license, including SGT-001 or SGT-003. 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 Michigan, 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 these patents and applications will be prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to maintain, enforce or defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights, including SGT-001 and SGT-003, could be adversely affected. For more information, see Part I, Item 1, "Business—Strategic partnerships and collaborations/licenses" of our 2020 Annual Report on Form 10-K.

Moreover, licenses to additional third-party intellectual property, technology and materials are required for our development programs but may not be available in the future or may not be available on commercially reasonable terms. For example, we are aware of certain third-party patents related to certain microdystrophin constructs, which, if in force at the time of SGT-001's commercialization, may be claimed by third parties to cover SGT-001. In addition, third parties may claim that the AAV vectors we are developing for use in SGT-001, SGT-003 or other 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 SGT-001, SGT-003, or other product candidates and such AAV vectors. 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 or 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 to any third-party intellectual property rights that are required for the development and commercialization of SGT-001, SGT-003 or any of our other product 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 SGT-001, SGT-003 or our other product candidates.

If we are unable to obtain and maintain patent protection for our product 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 product 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 SGT-001, SGT-003, our other product 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 SGT-001, SGT-003 and certain other product candidates that are important to our business. However, we cannot predict whether the patent applications we and our licensors are currently pursuing will issue as patents or whether the claims of any issued patents will provide us with a competitive advantage.

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

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

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

It is also currently unknown what claims may, if ever, issue from pending applications included in our patent rights. Additionally, certain of our in-licensed U.S. patent rights lack corresponding foreign patents or patent applications, and therefore we will be unable to obtain patent protection for our product candidates in certain jurisdictions. We or our licensors may not be able to obtain or maintain patent protection with respect to SGT-001, SGT-003 or our other product candidates.

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

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

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

Other parties have developed products that may be related or competitive to our own and such parties may have filed or may file patent applications, or may have received or may receive patents, claiming inventions that may overlap or conflict with those claimed in our patent applications or issued patents. We may not be aware of all third-party intellectual property rights potentially relating to SGT-001, SGT-003 or our other current or future product candidates. In addition, we cannot provide any assurances that any of the inventions disclosed in our patent applications will be found to be patentable, including over third-party or our own prior art patents, publications or other disclosures, or will issue as patents. Even if our patent applications issue as patents, we cannot provide any assurances that such patents will not be challenged or ultimately held to be invalid or unenforceable. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and in other jurisdictions are typically not published until 18 months after filing, or, in some cases, at all. Therefore, we cannot know with certainty whether the inventors of our licensed patents and applications were the first to make the inventions claimed in those patents or pending patent applications, or that they were the first to file for patent protection of such inventions. Similarly, should we own any issued patents or patent applications in the future, we may not be certain that we were the first to file for patent protection for the inventions claimed in such patents or patent applications. Furthermore, given the differences in patent laws in the United States, Europe and other foreign jurisdictions, for example, the availability of grace periods for filing patent applications and what can be considered as prior art, we cannot make any assurances that any claims in our pending and future patent applications in the United States or other jurisdictions will issue, or if they do issue, whether they will issue in a form that provides us with any meaningful competitive advantage. Similarly, we cannot make any assurances that if the patentability, validity, enforceability or scope of our pending or future patents and patent applications in the United States or foreign jurisdictions are challenged by any third party, that the claims of such pending or future patents and patent applications will survive any such challenge in a form that provides us with any meaningful competitive advantage. For example, we are aware of certain third-party patents and publications related to certain microdystrophin constructs. While we believe that our owned or in-licensed patents and patent applications claim novel and non-obvious features of microdystrophin constructs that are not described in such third-party patents or publications, such third-party patents and publications may have earlier priority or publication dates and may be asserted as prior art against our owned or in-licensed patents and applications. Any such challenge, if successful, could limit or eliminate patent protection for our products and product candidates or otherwise materially harm our business. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights cannot be predicted with any certainty.

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

The degree of patent protection we require to successfully compete in the marketplace may be unavailable. We cannot provide any assurances that any of the patents or patent applications included in our patent rights include or will include claims with a scope sufficient to protect SGT-001, SGT-003 and our other product candidates or otherwise provide any competitive advantage. In addition, the laws of foreign countries may not protect our proprietary rights to the same extent as the laws of the United States. Furthermore, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally twenty years after it is filed. Certain extensions may be available, however, the life of a patent, and the protection it affords, is limited. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our patent rights may not provide us with adequate and continuing patent protection sufficient to exclude others from commercializing products similar or identical to our product candidates, including biosimilar versions of such products.

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

Even if we acquire patent protection that we expect should enable us to maintain some competitive advantage, third parties, including competitors, may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. In litigation, a competitor could claim that our in-licensed patents or any patents we may own in the future are not valid or enforceable for a number of reasons. If a court agrees, we would lose our rights to those challenged patents. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such proceedings could result in the revocation or cancellation of or amendment to our licensed patents and any patents we may own in the future in such a way that they no longer cover SGT-001, SGT-003 or our other product candidates.

Even if issued, the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our current and future patent rights may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third-party submission of prior art to the U.S. Patent and Trademark Office, or USPTO, challenging the validity of one or more claims of patents included in our patent rights. Such submissions may also be made prior to a patent's issuance, precluding the granting of a patent based on one of the pending patent applications included in our patent rights. We may become involved in opposition, derivation, revocation, reexamination, post-grant and *inter partes* review, or interference proceedings challenging one or more patents included in our patent rights. For example, competitors may claim that they invented the inventions claimed in patents or patent applications included in our patent rights, such as the microdystrophin we use in SGT-001, prior to the inventors of such patents or patent applications, or may have filed one or more patent applications before the filing of the patents or patent applications included in our patent rights. A competitor who can establish an earlier filing or invention date may also assert that we are infringing their patents and that we therefore cannot practice our technology related to our product candidates as claimed in the patents or patent applications included in our patent rights. Competitors may also contest patents or patent applications included in our patent rights by showing that the claimed subject matter was not patent-eligible, was not novel or was obvious or that the patent claims failed any other requirement for patentability or enforceability. In addition, we may in the future be subject to claims by our or our licensors' current or former employees or consultants asserting an ownership right in the patents or patent applications included in our patent rights as an inventor or co-inventor, as a result of the work they performed.

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

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

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

We currently depend, and will continue to depend, on our license, collaboration and other similar agreements. Further development and commercialization of SGT-001, SGT-003 and our other current and future product candidates 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, or increase what we believe to be our financial or other obligations under the relevant agreement. Moreover, if disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on commercially acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

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

- lose our rights to develop and market SGT-001, SGT-003 or our other product candidates;
- lose patent protection for SGT-001, SGT-003 or our other product candidates;
- experience significant delays in the development or commercialization of SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 and our other current and future product 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 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 SGT-001, SGT-003 or other product candidates. It is possible that we may be unable to obtain additional 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 SGT-001, SGT-003, our other product 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 SGT-001, SGT-003 or our other product candidates. We cannot provide any assurances that third-party patents or other intellectual property rights do not exist that might be enforced against our manufacturing methods, product candidates or any technologies we may develop, resulting in either an injunction prohibiting our manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In each of our existing license agreements, and we expect in our future agreements, patent prosecution of our licensed technology is controlled solely by the licensor, and we may be required to reimburse the licensor for their costs of patent prosecution. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Further, in each of our license agreements our licensors have the first right to bring any actions against any third party for infringing on the patents we have licensed. Our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing product candidates. Disputes may arise regarding intellectual property subject to our licensing agreements, including:

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

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 and our other current and future product candidates without infringing, misappropriating or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We or our licensors may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 or any of our other product candidates does not infringe an existing patent or a patent that may be granted in the future. Many companies and institutions have filed, and continue to file, patent applications related to gene therapy and related manufacturing methods. Some of these patent applications have already been allowed or issued and others may issue in the future. Since this area is competitive and of strong interest to pharmaceutical and biotechnology companies, there will likely be additional patent applications filed and additional patents granted in the future, as well as additional research and development programs expected in the future. Furthermore, because patent applications can take many years to issue, may be confidential for 18 months or more after filing and can be revised before issuance, there may be applications now pending that may later result in issued patents that may be infringed by the manufacture, use, sale or importation of our product candidates and we may or may not be aware of such patents. If a patent holder believes the manufacture, use, sale or importation of one of our product candidates infringes its patent, the patent holder may sue us even if we have licensed other patent protection for our product candidates. Moreover, we may face patent infringement claims from non-practicing entities that have no relevant product revenue and against whom our licensed patent portfolio may therefore have no deterrent effect.

It is also possible that we have failed to identify relevant third-party patents or applications for which we may need a license to develop and commercialize SGT-001, SGT-003 and our other product candidates. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Moreover, it is difficult for industry participants, including us, to identify all third-party patent rights that may be relevant to our product candidates because patent searching is imperfect due to differences in terminology among patents, incomplete databases and the difficulty in assessing the meaning of patent claims. We may fail to identify relevant patents or patent applications or may identify pending patent applications of potential interest but incorrectly predict the likelihood that such patent applications may issue with claims of relevance to our product candidates. In addition, we may be unaware of one or more issued patents that would be infringed by the manufacture, sale or use of a current or future product candidate, or we may incorrectly conclude that a third-party patent is invalid, unenforceable or not infringed by our activities. Additionally, pending patent applications that have been published can, subject to certain limitations, be later amended in a manner that could cover our product candidates.

Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent or other intellectual property rights against us. For example, as discussed above, third parties may claim that the microdystrophin or the AAV vector we are developing for use in SGT-001 is 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 SGT-001, SGT-003 or our other product 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 SGT-001, SGT-003 or our other product candidates did not infringe a third-party patent.

Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. If we are found, or believe there is a risk that we may be found, to infringe, misappropriate or otherwise violate a third party's intellectual property rights, and we are unsuccessful in demonstrating that such intellectual property rights are invalid or unenforceable, we could be required or may choose to obtain a license from such third party to continue developing, manufacturing and marketing our product candidates. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease developing, manufacturing and commercializing the infringing product candidate, including SGT-001. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement, misappropriation or other violation of intellectual property rights, or claims that we have done so, could prevent us from manufacturing and commercializing our product candidates or force us to cease some or all of our business operations.

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

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

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

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

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

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

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

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

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

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

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

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of the discovery and development processes of SGT-001, SGT-003 and our other product candidates that involve proprietary know-how, information or technology that is not covered by patents. Our manufacturing process is protected by trade secrets. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

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

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

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

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

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

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

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

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and "gene patents" have been decided by the Supreme Court of the United States, or the Supreme Court. On March 20, 2012, the 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 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 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 (9th) edition of the MPEP (Manual for Patent Examination Procedure), last revised in January 2018. The current subject matter eligibility guideline instructs USPTO examiners to follow a two-part test, set forth in the U.S. Supreme Court decisions *Alice/Mayo*, as the only test that should be used to evaluate the eligibility of claims under examination, including claims directed to natural products and principles including all naturally occurring nucleic acids. Certain claims of our licensed patents and patent applications contain, and any future patents we may obtain may contain, claims that relate to specific recombinant DNA sequences that are naturally occurring at least in part and, therefore, could be the subject of future challenges made by third parties. In addition, the current USPTO subject matter eligibility guidance and the constantly evolving case law, together with contemplated congressional action, could all impact our ability to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure our stockholders that our efforts to seek patent protection for our product candidates will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the 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 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 SGT-001, SGT-003 or our other product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of SGT-001, SGT-003 and our other product 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", "SOLID GT" and "SOLID". 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 product 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.

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.

As of November 1, 2021, our executive officers and directors and principal stockholders, in the aggregate, beneficially owned 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 7,825,797 shares of our common stock to Ultragenyx. Following the expiration of an 18-month lock-up period and 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 and December 2020, 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. We have filed registration statements covering the resale of these shares by the purchasers in these private placements 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:

- results of or developments in preclinical studies and clinical trials of SGT-001, SGT-003 or our other product candidates or those of our competitors;
- the success of competitive products or technologies;
- the effect of the COVID-19 pandemic on both the healthcare system and the patient population;
- regulatory or legal developments in the United States, the European Union and other countries;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates, or our clinical development programs and our commercialization efforts;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in our development timelines;
- our ability to raise additional capital;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates;

- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of health care payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; 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, or at all.

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

We are an “emerging growth company,” or EGC, as defined in the Jumpstart our Business Startups Act of 2012, or the JOBS Act. We will remain an EGC until the earliest of: (i) the last day of the fiscal year in which we have total annual gross revenues of \$1.07 billion or more; (ii) December 31, 2023; (iii) the date on which we have issued more than \$1.0 billion in nonconvertible debt during the previous three years; and (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission, or the SEC. For so long as we remain an EGC, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, or Section 404;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements;
- being permitted to provide only two years of audited financial statements, in addition to any required unaudited interim financial statements, with correspondingly reduced “Management’s Discussion and Analysis of Financial Condition and Results of Operations” disclosure;
- reduced disclosure obligations regarding executive compensation; and
- an exemption from the requirement to seek nonbinding advisory votes on executive compensation or golden parachute arrangements.

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common stock held by non-affiliates is more than \$250 million measured on the last business day of our second fiscal quarter, or our annual revenues are less than \$100 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is more than \$700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, 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 Securities and Exchange Commission. In particular, we have not included all of the executive compensation information that would be required if we were not an EGC. 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, and particularly after we are no longer an EGC, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC or 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

On July 1, 2021, we granted a new employee an option to purchase 235,000 shares of our common stock as an inducement to employment in accordance with Nasdaq Listing Rule 5635(c)(4). The stock option is scheduled to become exercisable as to one-fourth of the shares underlying the option on each anniversary of the new employee's start date, subject to the recipient's continued service. The option has an exercise price of \$3.77 per share.

On August 16, 2021, we granted a new employee an option to purchase 118,000 shares of our common stock as an inducement to employment in accordance with Nasdaq Listing Rule 5635(c)(4). The stock option is scheduled to become exercisable as to one-fourth of the shares underlying the option on each anniversary of the new employee's start date, subject to the recipient's continued service. The option has an exercise price of \$2.51 per share.

No underwriters were involved in the foregoing issuance of securities. The securities were issued pursuant to Section 4(a)(2) under the Securities Act of 1933, as amended, relating to transactions by an issuer not involving any public offering. The recipients either received adequate information about us or had access, through other relationships, to such information.

Item 6. Exhibits.

Exhibit Number	Description
10.1*	2021 Employee Stock Purchase Plan
10.2*	Sales Agreement, dated March 13, 2019, by and between the Registrant and Jefferies LLC, as amended by Amendment No. 1 to the Sales Agreement, dated August 16, 2021, by and between the Registrant and Jefferies LLC
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
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)

* Filed 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 3, 2021

By: /s/ Ilan Ganot

Ilan Ganot
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 3, 2021

By: /s/ Stephen DiPalma

Stephen DiPalma
Interim Chief Financial Officer
(Principal Financial and Accounting Officer)

SOLID BIOSCIENCES INC.

2021 EMPLOYEE STOCK PURCHASE PLAN

The purpose of this 2021 Employee Stock Purchase Plan (this “Plan”) is to provide eligible employees of Solid Biosciences Inc. (the “Company”) and certain of its subsidiaries with opportunities to purchase shares of the Company’s common stock, \$0.001 par value per share (the “Common Stock”), commencing at such time and on such dates as the Board of Directors of the Company (the “Board”) shall determine. Subject to adjustment under Section 15 hereof, 1,102,885 shares of Common Stock have been approved for issuance under this Plan. This Plan is intended to qualify as an “employee stock purchase plan” as defined in Section 423 of the Internal Revenue Code of 1986, as amended (the “Code”), and the regulations issued thereunder, and shall be interpreted consistent therewith.

1. Administration. The Plan will be administered by the Board or by a committee appointed by the Board (the “Committee”). The Board or the Committee has authority to make rules and regulations for the administration of the Plan and its interpretation and decisions with regard thereto shall be final and conclusive.

2. Eligibility. All employees of the Company and all employees of any subsidiary of the Company (as defined in Section 424(f) of the Code) designated by the Board or the Committee from time to time (a “Designated Subsidiary”), are eligible to participate in any one or more of the offerings of Purchase Rights (as defined in Section 9) to purchase Common Stock under the Plan provided that:

(a) they are customarily employed by the Company or a Designated Subsidiary for more than 20 hours a week and for more than five months in a calendar year;

(b) they have been employed by the Company or a Designated Subsidiary for at least thirty days prior to enrolling in the Plan; and

(c) they are employees of the Company or a Designated Subsidiary on the first day of the applicable Plan Purchase Period (as defined below).

No employee may be granted a Purchase Right hereunder if such employee, immediately after the Purchase Right is granted, owns 5% or more of the total combined voting power or value of the stock of the Company or any subsidiary. For purposes of the preceding sentence, the attribution rules of Section 424(d) of the Code shall apply in determining the stock ownership of an employee, and all stock that the employee has a contractual right to purchase shall be treated as stock owned by the employee.

The Company retains the discretion to determine which eligible employees may participate in an offering pursuant to and consistent with Treasury Regulation Sections 1.423-2(e) and (f).

3. Offerings. The Company will make one or more offerings (“Offerings”) to employees to purchase stock under this Plan. Offerings will begin at such time and on such dates as the Board shall determine, or the first business day thereafter (such dates, the “Offering”).

Commencement Dates”). Each Offering Commencement Date will begin a six-month period (a “Plan Purchase Period”) during which payroll deductions will be made and held for the purchase of Common Stock at the end of the Plan Purchase Period. However, the Board or the Committee may, at its discretion, choose a different Purchase Period of not more than twelve (12) months for Offerings.

4. Participation. An employee eligible on the Offering Commencement Date of any Offering may participate in such Offering by completing and submitting the enrollment and payroll deduction authorization or other forms prescribed by the Committee in accordance with enrollment procedures prescribed by the Committee (which may include accessing the website designated by the Company and electronically enrolling and authorizing payroll deductions or completing other forms) prior to the applicable Offering Commencement Date. The forms will authorize a regular payroll deduction from the Compensation received by the employee during the Plan Purchase Period. Unless an employee changes his or her enrollment authorizations or withdraws from the Plan in accordance with the prescribed procedures, his or her deductions and purchases will continue at the same rate for future Offerings under the Plan as long as the Plan remains in effect. The term “Compensation” means the amount of money reportable on the employee’s Federal Income Tax Withholding Statement (or analogous non-U.S. statement), excluding overtime, shift premium, incentive or bonus awards, allowances and reimbursements for expenses such as relocation allowances for travel expenses, income or gains associated with the grant or vesting of restricted stock, income or gains on the exercise of Company stock options or stock appreciation rights, and similar items, whether or not shown or separately identified on the employee’s Federal Income Tax Withholding Statement (or analogous non-U.S. statement), but including, in the case of salespersons, sales commissions to the extent determined by the Board or the Committee.

5. Deductions. The Company will maintain payroll deduction accounts for all participating employees. With respect to any Offering made under this Plan, an employee may authorize a payroll deduction in any percentage amount (in whole percentages) up to a maximum of 15% of the Compensation he or she receives during the Plan Purchase Period or such shorter period during which deductions from payroll are made. The Board or the Committee may, at its discretion, designate a lower maximum contribution rate. The minimum payroll deduction is such percentage of Compensation as may be established from time to time by the Board or the Committee.

6. Deduction Changes. An employee may decrease or discontinue his or her payroll deduction once during any Plan Purchase Period, by filing either a written or electronic new payroll deduction authorization form, as determined by the Company. However, an employee may not increase his or her payroll deduction during a Plan Purchase Period. If an employee elects to discontinue his or her payroll deductions during a Plan Purchase Period but does not elect to withdraw his or her funds pursuant to Section 8 hereof, funds deducted prior to his or her election to discontinue will be applied to the purchase of Common Stock on the Purchase Exercise Date (as defined below).

7. Interest. Interest will not be paid on any employee accounts, except to the extent that the Board or the Committee, in its sole discretion, elects to credit employee accounts with interest at such rate as it may from time to time determine.

8. Withdrawal of Funds. An employee may at any time prior to the close of business on the fifteenth business day prior to the end of a Plan Purchase Period (or such other number of days as is determined by the Company) and for any reason permanently draw out the balance accumulated in the employee's account and thereby withdraw from participation in an Offering. Partial withdrawals are not permitted. The employee may not begin participation again during the remainder of the Plan Purchase Period during which the employee withdrew his or her balance. The employee may participate in any subsequent Offering in accordance with terms and conditions established by the Board or the Committee.

9. Purchase of Shares.

(a)Number of Shares.

(i) On the Offering Commencement Date for the applicable Plan Purchase Period, the Company will grant to each eligible employee who is then a participant in the Plan an option (a "Purchase Right") to purchase on the last business day of such Plan Purchase Period (the "Purchase Exercise Date") at the applicable purchase price (the "Purchase Right Price") up to that whole number of shares of Common Stock (the "Purchase Right Shares") determined by (A) dividing \$25,000 by 12, (B) multiplying the result by the number of full months in the Plan Purchase Period and (C) dividing the result by the closing price (as determined below) on the Offering Commencement Date; provided, however, that no employee may be granted a Purchase Right which permits his or her rights to purchase Common Stock under this Plan and any other employee stock purchase plan (as defined in Section 423(b) of the Code) of the Company and its subsidiaries, to accrue at a rate which exceeds \$25,000 of the fair market value of such Common Stock (determined at the date such Purchase Right is granted) for each calendar year in which the Purchase Right is outstanding at any time; and, provided, further, however, that the Committee may, in its discretion, set a fixed maximum number of shares of Common Stock that each eligible employee may purchase per Plan Purchase Period which number may not be greater than the number of shares of Common Stock determined by using the formula in the first clause of this Section 9(a) and which number shall be subject to the second clause of this Section 9(a).

(ii) In the event the length of the Plan Purchase Period exceeds twelve (12) months), the Purchase Right Shares shall, subject to the additional limitations in Section 9(a)(i), be determined by multiplying \$2,083 by the number of full months on the Plan Purchase Period and dividing the result by the closing price (as determined below) on the Offering Commencement Date.

(b)Purchase Right Price. The Board or the Committee shall determine the Purchase Right Price for each Plan Purchase Period, including whether such Purchase Right Price shall be determined based on the lesser of the closing price of the Common Stock on (i) the first business day of the Plan Purchase Period or (ii) the Purchase Exercise Date, or shall be based solely on the closing price of the Common Stock on the Purchase Exercise Date; provided, however, that such Purchase Right Price shall be at least 85% of the applicable closing price. In the absence of a determination by the Board or the Committee, the Purchase Right Price will be 85% of the lesser of the closing price of the Common Stock on (i) the first business day of the Plan Purchase Period or (ii) the Purchase Exercise Date. The closing price shall be (a) the closing price

(for the primary trading session) on any national securities exchange on which the Common Stock is listed or (b) the average of the closing bid and asked prices in the over-the-counter-market, whichever is applicable, as published in The Wall Street Journal or another source selected by the Board or the Committee. If no sales of Common Stock were made on such a day, the price of the Common Stock shall be the reported price for the last preceding day on which sales were made.

(c)Exercise of Purchase Right. Each employee who continues to be a participant in the Plan on the Purchase Exercise Date shall be deemed to have exercised his or her Purchase Right at the Purchase Right Price on such date and shall be deemed to have purchased from the Company the number of whole shares of Common Stock reserved for the purpose of the Plan that his or her accumulated payroll deductions on such date will pay for, but not in excess of the maximum numbers determined in the manner set forth above.

(d)Return of Unused Payroll Deductions. Any balance remaining in an employee's payroll deduction account at the end of a Plan Purchase Period will be automatically refunded to the employee, except that any balance that is less than the purchase price of one share of Common Stock will be carried forward into the employee's payroll deduction account for the following Offering, unless the employee elects not to participate in the following Offering under the Plan, in which case the balance in the employee's account shall be refunded.

10. Issuance of Shares.

(a) Certificates. Certificates representing shares of Common Stock purchased under the Plan may be issued only in the name of the employee, in the name of the employee and another person of legal age as joint tenants with rights of survivorship, or (in the Company's sole discretion) in the name of a brokerage firm, bank, or other nominee holder designated by the employee. The Company may, in its sole discretion and in compliance with applicable laws, authorize the use of book entry registration of shares in lieu of issuing stock certificates.

(b) ESPP Broker Account. The Company may require that the shares purchased on behalf of each participant shall be deposited directly into a brokerage account which the Company shall establish for the participant at a Company-designated brokerage firm. The account will be known as the ESPP Broker Account. Except as otherwise provided below, the deposited shares may not be transferred (either electronically or in certificate form) from the ESPP Broker Account until the **later** of the following two periods: (i) the end of the two (2)-year period measured from the date the participant first commences participation in the Offering Period in which the shares were purchased and (ii) the end of the one (1)-year period measured from the Purchase Exercise Date of those shares. Such limitation shall apply both to transfers to different accounts with the same ESPP broker and to transfers to other brokerage firms. Any shares held for the required holding period may thereafter be transferred (either electronically or in certificate form) to other accounts or to other brokerage firms.

The foregoing procedures shall not in any way limit when the Participant may sell his or her shares. Those procedures are designed solely to assure that any sale of shares prior to the satisfaction of the required holding period is made through the ESPP Broker Account. In addition, the participant may request a stock certificate or share transfer from his or her ESPP Broker Account prior to the satisfaction of the required holding period should the participant wish

to make a gift of any shares held in that account. However, shares may not be transferred (either electronically or in certificate form) from the ESPP Broker Account for use as collateral for a loan, unless those shares have been held for the required holding period.

The foregoing procedures shall apply to all shares purchased by each participant in the United States, whether or not that participant continues in employee status.

11. Rights on Retirement, Death or Termination of Employment. If a participating employee's employment ends before the last business day of a Plan Purchase Period, no payroll deduction shall be taken from any pay then due and owing to the employee and the balance in the employee's account shall be paid to the employee. In the event of the employee's death before the last business day of a Plan Purchase Period, the Company shall, upon notification of such death, pay the balance of the employee's account (a) to the executor or administrator of the employee's estate or (b) if no such executor or administrator has been appointed to the knowledge of the Company, to such other person(s) as the Company may, in its discretion, designate. If, before the last business day of the Plan Purchase Period, the Designated Subsidiary by which an employee is employed ceases to be a subsidiary of the Company, or if the employee is transferred to a subsidiary of the Company that is not a Designated Subsidiary, the employee shall be deemed to have terminated employment for the purposes of this Plan.

12. Purchase Right Holders Not Stockholders. Neither the granting of a Purchase Right to an employee nor the deductions from his or her pay shall make such employee a stockholder of the shares of Common Stock covered by a Purchase Right under this Plan until he or she has purchased and received such shares.

13. Purchase Rights Not Transferable. Purchase Rights under this Plan are not transferable by a participating employee other than by will or the laws of descent and distribution, and are exercisable during the employee's lifetime only by the employee.

14. Application of Funds. All funds received or held by the Company under this Plan may be combined with other corporate funds and may be used for any corporate purpose.

15. Adjustment for Changes in Common Stock and Certain Other Events.

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under this Plan, (ii) the share limitations set forth in Section 9, and (iii) the Purchase Right Price shall be equitably adjusted to the extent determined by the Board or the Committee.

(b) Reorganization Events.

(1) Definition. A "Reorganization Event" shall mean: (a) any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (b) any transfer or disposition of all of the Common Stock of the

Company for cash, securities or other property pursuant to a share exchange or other transaction or (c) any liquidation or dissolution of the Company.

(2)Consequences of a Reorganization Event on Purchase Rights. In connection with a Reorganization Event, the Board or the Committee may take any one or more of the following actions as to outstanding Purchase Rights on such terms as the Board or the Committee determines: (i) provide that Purchase Rights shall be assumed, or substantially equivalent Purchase Rights shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to employees, provide that all outstanding Purchase Rights will be terminated immediately prior to the consummation of such Reorganization Event and that all such outstanding Purchase Rights will become exercisable to the extent of accumulated payroll deductions as of a date specified by the Board or the Committee in such notice, which date shall not be less than ten (10) days preceding the effective date of the Reorganization Event, (iii) upon written notice to employees, provide that all outstanding Purchase Rights will be cancelled as of a date prior to the effective date of the Reorganization Event and that all accumulated payroll deductions will be returned to participating employees on such date, (iv) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the "Acquisition Price"), change the last day of the Plan Purchase Period to be the date of the consummation of the Reorganization Event and make or provide for a cash payment to each employee equal to (A) (1) the Acquisition Price times (2) the number of shares of Common Stock that the employee's accumulated payroll deductions as of immediately prior to the Reorganization Event could purchase at the Purchase Right Price, where the Acquisition Price is treated as the fair market value of the Common Stock on the last day of the applicable Plan Purchase Period for purposes of determining the Purchase Right Price under Section 9(b) hereof, and where the number of shares that could be purchased is subject to the limitations set forth in Section 9(a), minus (B) the result of multiplying such number of shares by such Purchase Right Price, (v) provide that, in connection with a liquidation or dissolution of the Company, Purchase Rights shall convert into the right to receive liquidation proceeds (net of the Purchase Right Price thereof) and (vi) any combination of the foregoing.

For purposes of clause (i) above, an Purchase Right shall be considered assumed if, following consummation of the Reorganization Event, the Purchase Right confers the right to purchase, for each share of Common Stock subject to the Purchase Right immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); provided, however, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise of Purchase Rights to consist solely of such number of shares of common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Board determines to be equivalent in value (as of the date of such determination or another date specified by the Board) to

the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

16. Amendment of the Plan. The Board may at any time, and from time to time, amend or suspend this Plan or any portion thereof, except that (a) if the approval of any such amendment by the shareholders of the Company is required by Section 423 of the Code, such amendment shall not be effected without such approval, and (b) in no event may any amendment be made that would cause the Plan to fail to comply with Section 423 of the Code.

17. Insufficient Shares. If the total number of shares of Common Stock specified in elections to be purchased under any Offering plus the number of shares purchased under previous Offerings under this Plan exceeds the maximum number of shares issuable under this Plan, the Board or the Committee will allot the shares then available on a pro-rata basis.

18. Termination of the Plan. This Plan may be terminated at any time by the Board. Upon termination of this Plan all amounts in the accounts of participating employees shall be promptly refunded.

19. Governmental Regulations. The Company's obligation to sell and deliver Common Stock under this Plan is subject to listing on a national stock exchange (to the extent the Common Stock is then so listed or quoted) and the approval of all governmental authorities required in connection with the authorization, issuance or sale of such stock.

20. Governing Law. The Plan shall be governed by Delaware law except to the extent that such law is preempted by federal law.

21. Issuance of Shares. Shares may be issued upon exercise of a Purchase Right from authorized but unissued Common Stock, from shares held in the treasury of the Company, or from any other proper source.

22. Notification upon Sale of Shares. Each employee agrees, by participating in the Plan, to promptly give the Company notice of any disposition of shares purchased under the Plan where such disposition occurs within two years after the date of grant of the Purchase Right pursuant to which such shares were purchased.

23. Grants to Employees in Foreign Jurisdictions. The Company may, to comply with the laws of a foreign jurisdiction, grant Purchase Rights to employees of the Company or a Designated Subsidiary who are citizens or residents of such foreign jurisdiction (without regard to whether they are also citizens of the United States or resident aliens (within the meaning of Section 7701(b)(1)(A) of the Code)) with terms that are less favorable (but not more favorable) than the terms of Purchase Rights granted under the Plan to employees of the Company or a Designated Subsidiary who are resident in the United States. Notwithstanding the preceding provisions of this Plan, employees of the Company or a Designated Subsidiary who are citizens or residents of a foreign jurisdiction (without regard to whether they are also citizens of the United States or resident aliens (within the meaning of Section 7701(b)(1)(A) of the Code)) may be excluded from eligibility under the Plan if (a) the grant of an Purchase Right under the Plan to a citizen or resident of the foreign jurisdiction is prohibited under the laws of such jurisdiction or (b) compliance with the laws of the foreign jurisdiction would cause the Plan to violate the requirements of Section 423

of the Code. The Company may add one or more appendices to this Plan describing the operation of the Plan in those foreign jurisdictions in which employees are excluded from participation or granted less favorable Purchase Rights.

24. Authorization of Sub-Plans. The Board may from time to time establish one or more sub-plans under the Plan with respect to one or more Designated Subsidiaries, provided that such sub-plan complies with Section 423 of the Code.

25. Withholding. If applicable tax laws impose a tax withholding obligation, each affected employee shall, no later than the date of the event creating the tax liability, make provision satisfactory to the Board for payment of any taxes required by law to be withheld in connection with any transaction related to Purchase Rights granted to or shares acquired by such employee pursuant to the Plan. The Company may, to the extent permitted by law, deduct any such taxes from any payment of any kind otherwise due to an employee.

26. Effective Date and Approval of Shareholders. The Plan shall take effect on the date the Plan is approved by the stockholders of the Company as required by Section 423 of the Code, which approval must occur within twelve months of the adoption of the Plan by the Board.

Adopted by the Board of Directors on
April 14, 2021

Approved by the stockholders on
June 16, 2021

SOLID BIOSCIENCES INC.
Shares of Common Stock
(par value \$0.001 per share)

SALES AGREEMENT

March 13, 2019

JEFFERIES LLC
520 Madison Avenue
New York, New York 10022

Ladies and Gentlemen:

Solid Biosciences Inc., a Delaware corporation (the “**Company**”), proposes, subject to the terms and conditions stated herein, to issue and sell from time to time through Jefferies LLC, as sales agent and/or principal (the “**Agent**”), shares of the Company’s common stock, par value \$0.001 per share (the “**Common Shares**”), having an aggregate offering price of up to \$50,000,000 on the terms set forth in this agreement (this “**Agreement**”).

Section 1. DEFINITIONS

(a) Certain Definitions. For purposes of this Agreement, capitalized terms used herein and not otherwise defined shall have the following respective meanings:

“**Actions**” has the meaning set forth in Section 2(t).

“**Affiliate**” of a Person means another Person that directly or indirectly, through one or more intermediaries, controls, is controlled by, or is under common control with, such first- mentioned Person. The term “control” (including the terms “controlling,” “controlled by” and “under common control with”) means the possession, direct or indirect, of the power to direct or cause the direction of the management and policies of a Person, whether through the ownership of voting securities, by contract or otherwise.

“**Agency Period**” means the period commencing on the date of this Agreement and expiring on the earliest to occur of (x) the date on which the Agent shall have placed the Maximum Program Amount pursuant to this Agreement, (y) the date this Agreement is terminated pursuant to Section 7 and (z) the third anniversary of the date of this Agreement.

“**Agent**” has the meaning set forth in the introductory paragraph of this Agreement.

“**Agreement**” has the meaning set forth in the introductory paragraph of this Agreement.

“**Anti-Money Laundering Laws**” has the meaning set forth in Section 2(jj).

“**Code**” has the meaning set forth in Section 2(k).

“**Commission**” means the U.S. Securities and Exchange Commission.

“**Common Shares**” has the meaning set forth in the introductory paragraph of this Agreement.

“**Company**” has the meaning set forth in the introductory paragraph of this Agreement.

“**Company Stock Plans**” has the meaning set forth in Section 2(k).

“**EDGAR**” has the meaning set forth in Section 2(b).

“**Environmental Laws**” has the meaning set forth in Section 2(cc).

“**ERISA**” has the meaning set forth in Section 2(dd).

“**Exchange Act**” means the Securities Exchange Act of 1934, as amended, and the rules and regulations of the Commission thereunder.

“**FINRA**” has the meaning set forth in Section 2(s).

“**Floor Price**” means the minimum price set by the Company in the Issuance Notice below which the Agent shall not sell Shares during the applicable Selling Period, which may be adjusted by the Company at any time during the Selling Period by delivering written notice of such change to the Agent and which in no event shall be less than \$1.00 without the prior written consent of the Agent, which may be withheld in the Agent’s sole discretion.

“**Free Writing Prospectus**” has the meaning set forth in Section 4(e).

“**GAAP**” has the meaning set forth in Section 2(g).

“**HMT**” has the meaning set forth in Section 2(kk).

“**Intellectual Property**” has the meaning set forth in Section 2(w).

“**Interim Prospectus Supplement**” has the meaning set forth in Section 4(a).

“**Investment Company Act**” has the meaning set forth in Section 2(y).

“**Issuance Amount**” means the aggregate Sales Price of the Shares to be sold by the Agent pursuant to any Issuance Notice.

“**Issuance Notice**” means a written notice delivered to the Agent by the Company in accordance with this Agreement in the form attached hereto as Exhibit A that is executed by its Chief Executive Officer, President or Chief Financial Officer.

“**Issuance Notice Date**” means any Trading Day during the Agency Period that an Issuance Notice is delivered pursuant to Section 3(b)(i).

“**Issuance Price**” means the Sales Price less the Selling Commission.

“**IT Systems and Data**” has the meaning set forth in Section 2(ii).

“**Material Adverse Effect**” has the meaning set forth in Section 2(i).

“**Maximum Program Amount**” means Common Shares with an aggregate Sales Price of the lesser of (1) \$50,000,000 and (2) the aggregate amount of Shares registered under the Registration Statement.

“**OFAC**” has the meaning set forth in Section 2(kk).

“**Original Registration Statement**” has the meaning set forth in Section 2(a).

“**Other Offering Amendment**” means an amendment or supplement to the Registration Statement or Prospectus relating solely to the issuance or offering of securities other than the Common Shares.

“**Person**” means an individual or a corporation, partnership, limited liability company, trust, incorporated or unincorporated association, joint venture, joint stock company, governmental authority or other entity of any kind.

“**Plan**” has the meaning set forth in Section 2(dd).

“**Principal Market**” means the Nasdaq Global Select Market or such other national securities exchange on which the Common Shares, including any Shares, are then listed.

“**Prospectus**” has the meaning set forth in Section 2(a).

“**Registration Statement**” has the meaning set forth in Section 2(a).

“**Regulation M**” has the meaning set forth in Section 2(oo).

“**Regulatory Authorities**” has the meaning set forth in Section 2(tt).

“**Related Judgment**” has the meaning set forth in Section 8(g).

“**Related Proceedings**” has the meaning set forth in Section 8(g).

“**Renewal Deadline**” has the meaning set forth in Section 4(x).

“**Representation Date**” has the meaning set forth in the introductory paragraph of Section 2.

“**Rule 102**” has the meaning set forth in Section 4(y).

“**Sales Agreement Prospectus**” has the meaning set forth in Section 2(a).

“**Sales Price**” means the actual sale execution price of each Share placed by the Agent pursuant to this Agreement.

“**Sarbanes-Oxley Act**” has the meaning set forth in Section 2(vv).

“**Securities Act**” means the Securities Act of 1933, as amended, and the rules and regulations of the Commission thereunder.

“**Selling Commission**” means, except as otherwise agreed, three percent (3.00%) of the gross proceeds.

“**Selling Period**” means the period set forth in the Issuance Notice.

“**Settlement Date**” means the second business day following each Trading Day during the Selling Period on which Shares are sold pursuant to this Agreement, when the Company shall deliver to the Agent the amount of Shares sold on such Trading Day and the Agent shall deliver to the Company the Issuance Price received on such sales.

“**Shares**” shall mean the Company’s Common Shares issued or issuable pursuant to this Agreement.

“**Specified Courts**” has the meaning set forth in Section 8(g).

“**Stock Options**” has the meaning set forth in Section 2(k).

“**Time of Sale**” has the meaning set forth in Section 3(b)(v).

“**Time of Sale Information**” has the meaning set forth in Section 2(b).

“**Trading Day**” means any day on which the Principal Market is open for trading.

“**Triggering Event Date**” has the meaning set forth in Section 4(o).

“**UNSC**” has the meaning set forth in Section 2(kk).

Section 2. REPRESENTATIONS AND WARRANTIES OF THE COMPANY

The Company represents and warrants to, and agrees with, the Agent that, unless such representation, warranty or agreement specifies otherwise, as of (1) the date of this Agreement, (2) each Issuance Notice Date, (3) each Settlement Date, (4) each Triggering Event Date and (5) as of each Time of Sale (each of the times referenced above is referred to herein as a “**Representation Date**”), except as may be disclosed in the Prospectus (including any documents incorporated by reference therein and any supplements thereto) on or before a Representation Date:

(a) Registration Statement. The Company has prepared and filed, or will file, with the Commission a shelf registration statement on Form S-3, including a base prospectus relating to certain securities, including the Common Shares to be issued from time to time by the Company, and a sales agreement prospectus (the “**Sales Agreement Prospectus**”) specifically relating to the Shares. Such registration statement registers the issuance and sale by the Company of the Shares

under the Securities Act. Such registration statement, including any information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, including all financial statements, exhibits and schedules thereto and all documents incorporated or deemed to be incorporated therein by reference pursuant to Item 12 of Form S-3 under the Securities Act as from time to time amended or supplemented, is herein referred to as the “**Registration Statement**,” and the Sales Agreement Prospectus, and any prospectus supplement filed with the Commission pursuant to Rule 424(b) under the Securities Act relating to a particular issuance of the Shares, including all documents incorporated or deemed to be incorporated therein by reference pursuant to Item 12 of Form S-3 under the Securities Act, in each case, as from time to time amended or supplemented, is referred to herein as the “**Prospectus**,” except that if any revised prospectus is provided to the Agent by the Company for use in connection with the offering of the Shares that is not required to be filed by the Company pursuant to Rule 424(b) under the Securities Act, the term “**Prospectus**” shall refer to such revised prospectus from and after the time it is first provided to the Agent for such use. The Registration Statement at the time it originally became effective is herein called the “**Original Registration Statement**.” As used in this Agreement, the terms “amendment” or “supplement” when applied to the Registration Statement or the Prospectus shall be deemed to include the filing by the Company with the Commission of any document under the Exchange Act after the date hereof that is or is deemed to be incorporated therein by reference.

All references in this Agreement to financial statements and schedules and other information which is “contained,” “included” or “stated” in the Registration Statement or the Prospectus (and all other references of like import) shall be deemed to mean and include all such financial statements and schedules and other information which is or is deemed to be incorporated by reference in or otherwise deemed under the Securities Act to be a part of or included in the Registration Statement or the Prospectus, as the case may be, as of any specified date; and all references in this Agreement to amendments or supplements to the Registration Statement or the Prospectus shall be deemed to mean and include, without limitation, the filing of any document under the Exchange Act which is or is deemed to be incorporated by reference in or otherwise deemed under the Securities Act to be a part of or included in the Registration Statement or the Prospectus, as the case may be, as of any specified date.

At the time the Registration Statement was originally declared effective, the Company met the then-applicable requirements for use of Form S-3 under the Securities Act. During the Agency Period, each time the Company files an annual report on Form 10-K the Company will meet the then-applicable requirements for use of Form S-3 under the Securities Act.

(b) Compliance with Registration Requirements. The Original Registration Statement will be declared effective prior to the first Issuance Notice Date. Any Rule 462(b) Registration Statement filed after the date hereof will be declared effective prior to any Issuance Notice Date following such filing. The Company has complied, or will comply, to the Commission’s satisfaction with all requests of the Commission for additional or supplemental information. No stop order suspending the effectiveness of the Registration Statement or any Rule 462(b) Registration Statement is in effect and no proceedings for such purpose have been instituted or are pending or, to the best knowledge of the Company, are contemplated or threatened by the Commission.

The Prospectus when filed complied, or will comply, in all material respects with the Securities Act and, if filed by electronic transmission pursuant to the SEC's Electronic Data Gathering, Analysis, and Retrieval system ("**EDGAR**") (except as may be permitted by Regulation S-T under the Securities Act), was identical to the copy thereof delivered to the Agent for use in connection with the issuance and sale of the Shares. Each of the Registration Statement, any Rule 462(b) Registration Statement and any post-effective amendment thereto, at the time it became effective and at all subsequent times, complied and will comply in all material respects with the Securities Act and did not and will not contain any untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein not misleading. As of the date of this Agreement, the Prospectus and any Free Writing Prospectus consented to by the Agent pursuant to Section 4(e), considered together (collectively, the "**Time of Sale Information**") did not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading. The Prospectus, as amended or supplemented, as of its date and at all subsequent times, did not and will not contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading. The representations and warranties set forth in the three immediately preceding sentences do not apply to statements in or omissions from the Registration Statement, any Rule 462(b) Registration Statement, or any post-effective amendment thereto, or the Prospectus, or any amendments or supplements thereto, made in reliance upon and in conformity with information relating to the Agent furnished to the Company in writing by the Agent expressly for use therein, it being understood and agreed that the only such information furnished by the Agent to the Company consists of the information described in Section 6 below. There are no contracts or other documents required to be described in the Prospectus or to be filed as exhibits to the Registration Statement which have not been described or filed as required. The Registration Statement and the offer and sale of the Shares as contemplated hereby meet the requirements of Rule 415 under the Securities Act and comply in all material respects with said Rule.

(c) Ineligible Issuer Status. The Company is not an "ineligible issuer" in connection with the offering of the Shares pursuant to Rules 164, 405 and 433 under the Securities Act. Any Free Writing Prospectus that the Company is required to file pursuant to Rule 433(d) under the Securities Act has been, or will be, filed with the Commission in accordance with the requirements of the Securities Act. Each Free Writing Prospectus that the Company has filed, or is required to file, pursuant to Rule 433(d) under the Securities Act or that was prepared by or behalf of or used or referred to by the Company complies or will comply in all material respects with the requirements of Rule 433 under the Securities Act including timely filing with the Commission or retention where required and legending, and each such Free Writing Prospectus, as of its issue date and at all subsequent times through the completion of the issuance and sale of the Shares did not, does not and will not include any information that conflicted, conflicts with or will conflict with the information contained in the Registration Statement or the Prospectus, including any document incorporated by reference therein. Except for the Free Writing Prospectuses, if any, and electronic road shows, if any, furnished to you before first use, the Company has not prepared, used or referred to, and will not, without your prior consent, prepare, use or refer to, any Free Writing Prospectus.

(d) Incorporated Documents. The documents incorporated or deemed to be incorporated by reference in the Registration Statement and the Prospectus, at the time they were filed with the Commission, complied in all material respects with the requirements of the Exchange Act, as applicable, and, when read together with the other information in the Prospectus, do not contain an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the statements therein, in light of the circumstances under which they were made, not misleading.

(e) S-3 Eligibility. The Company and the transactions contemplated by this Agreement meet the requirements for, and comply with the conditions for the use of, Form S-3 under the Act, including but not limited to Instruction I.B.1 of Form S-3. The Company is not a shell company (as defined in Rule 405 of the Act) and has not been a shell company for at least 12 calendar months.

(f) Exchange Act Compliance. The documents incorporated or deemed to be incorporated by reference in the Prospectus, at the time they were or hereafter are filed with the Commission, and any Free Writing Prospectus or amendment or supplement thereto complied and will comply in all material respects with the requirements of the Exchange Act, and, when read together with the other information in the Prospectus, at the time the Registration Statement and any amendments thereto become effective, will not contain an untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary to make the fact required to be stated therein or necessary to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(g) Financial Statements. The financial statements (including the related notes thereto) of the Company and its consolidated subsidiaries included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information comply in all material respects with the applicable requirements of the Securities Act and present fairly in all material respects the financial position of the Company and its consolidated subsidiaries as of the dates indicated and the results of their operations and the changes in their cash flows for the periods specified; such financial statements have been prepared in conformity with generally accepted accounting principles (“GAAP”) in the United States applied on a consistent basis throughout the periods covered thereby, except unaudited financial statements, which are subject to normal year-end adjustments and do not contain certain footnotes as permitted by the applicable rules of the Commission, and any supporting schedules included or incorporated by reference in the Registration Statement present fairly in all material respects the information required to be stated therein; and the other financial information included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information has been derived from the accounting records of the Company and its consolidated subsidiaries and presents fairly in all material respects the information shown thereby.

(h) No Material Adverse Change. Since the date of the most recent financial statements of the Company included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information, (i) there has not been any material change in the capital stock (other than the issuance of shares of Common Stock upon exercise of stock options and warrants described as outstanding in, and the grant of options and awards under existing equity incentive plans described in, the Registration Statement, the Prospectus and the Time of Sale

Information), short-term debt or long-term debt of the Company or any of its subsidiaries, or any dividend or distribution of any kind declared, set aside for payment, paid or made by the Company on any class of capital stock, or any material adverse change, or any development that would reasonably be expected to result in a material adverse change, in or affecting the business, properties, management, financial position, stockholders' equity, results of operations or prospects of the Company and its subsidiaries taken as a whole; (ii) neither the Company nor any of its subsidiaries has entered into any transaction or agreement (whether or not in the ordinary course of business) that is material to the Company and its subsidiaries taken as a whole or incurred any liability or obligation, direct or contingent, that is material to the Company and its subsidiaries taken as a whole; and (iii) neither the Company nor any of its subsidiaries has sustained any loss or interference with its business that is material to the Company and its subsidiaries taken as a whole and that is either from fire, explosion, flood or other calamity, whether or not covered by insurance, or from any labor disturbance or dispute or any action, order or decree of any court or arbitrator or governmental or regulatory authority, except in each case as otherwise disclosed in the Registration Statement, the Prospectus and the Time of Sale Information.

(i) Organization and Good Standing. The Company and each of its subsidiaries have been duly organized and are validly existing and in good standing under the laws of their respective jurisdictions of organization, are duly qualified to do business and are in good standing in each jurisdiction in which their respective ownership or lease of property or the conduct of their respective businesses requires such qualification, and have all power and authority necessary to own or hold their respective properties and to conduct the businesses in which they are engaged, except where the failure to be so qualified or in good standing or have such power or authority would not, individually or in the aggregate, reasonably be expected to have a material adverse effect on the business, properties, management, financial position, stockholders' equity, results of operations or prospects of the Company and its subsidiaries taken as a whole or on the performance by the Company of its obligations under this Agreement (a "**Material Adverse Effect**"). Except as otherwise disclosed to the Agent, the Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Exhibit 21.1 to the most recent annual report on Form 10-K filed by the Company and incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information.

(j) Capitalization. All the outstanding shares of capital stock of the Company have been duly and validly authorized and issued and are fully paid and non-assessable and are not subject to any preemptive or similar rights that have not been validly waived; except as described in or expressly contemplated by the Registration Statement, the Prospectus and the Time of Sale Information, there are no outstanding rights (including, without limitation, preemptive rights), warrants or options to acquire, or instruments convertible into or exchangeable for, any shares of capital stock or other equity interest in the Company or any of its subsidiaries, or any contract, commitment, agreement, understanding or arrangement of any kind relating to the issuance of any capital stock of the Company or any such subsidiary, any such convertible or exchangeable securities or any such rights, warrants or options; the capital stock of the Company conforms in all material respects to the description thereof contained in the Registration Statement and the Prospectus; and all the outstanding shares of capital stock or other equity interests of each subsidiary owned, directly or indirectly, by the Company have been duly and validly authorized and issued, are fully paid and non-assessable (except, as otherwise described in the Registration Statement, the Prospectus and the Time of Sale Information), and are owned directly or indirectly

by the Company, free and clear of any lien, charge, encumbrance, security interest, restriction on voting or transfer or any other claim of any third party.

(k) Stock Options. With respect to the stock options (the “**Stock Options**”) granted pursuant to the stock-based compensation plans of the Company and its subsidiaries (the “**Company Stock Plans**”), (i) to the Company’s knowledge, each Stock Option intended to qualify as an “incentive stock option” under Section 422 of the Internal Revenue Code of 1986, as amended (the “**Code**”) so qualifies, (ii) each grant of a Stock Option was duly authorized no later than the date on which the grant of such Stock Option was by its terms to be effective by all necessary corporate action, including, as applicable, approval by the board of directors of the Company (or a duly constituted and authorized committee thereof) and any required stockholder approval by the necessary number of votes or written consents, and the award agreement governing such grant (if any) was duly executed by the Company and delivered to the recipient, (iii) each such grant was made in accordance with the terms of the Company Stock Plans and all other applicable laws and regulatory rules or requirements, including the rules of the Principal Market and any other exchange on which Company securities are traded, and (iv) each such grant was properly accounted for in accordance with GAAP in the United States in the financial statements (including the related notes) of the Company.

(l) Due Authorization. The Company has the requisite power and authority to execute and deliver this Agreement and to perform its obligations hereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of this Agreement and the consummation by it of the transactions contemplated hereby has been duly and validly taken.

(m) Sales Agreement. This Agreement has been duly authorized, executed and delivered by the Company.

(n) The Shares. The Shares to be issued and sold by the Company hereunder, when issued and delivered and paid for as provided herein, will be duly authorized and validly issued, will be fully paid and nonassessable and will conform in all material respects to the descriptions thereof in the Registration Statement, the Prospectus and the Time of Sale Information; and the issuance of the Shares is not subject to any preemptive or similar rights that have not been duly waived or satisfied.

(o) Descriptions of the Sales Agreement. This Agreement conforms in all material respects to the description thereof contained in the Prospectus and the Time of Sale Information.

(p) Stock Exchange Listing. The Common Shares are registered pursuant to Section 12(b) of the Exchange Act and are listed on the Nasdaq Global Select Market, and the Company has taken no action designed to, or likely to have the effect of, terminating the registration of the Common Shares under the Exchange Act or delisting the Common Shares from the Nasdaq Global Select Market, nor has the Company received any notification that the Commission or the Nasdaq Global Select Market is contemplating terminating such registration or listing.

(q) No Violation or Default. Neither the Company nor any of its subsidiaries is (i) in violation of its charter or by-laws or similar organizational documents; (ii) in default, and no event has occurred that, with notice or lapse of time or both, would constitute such a default, in the due

performance or observance of any term, covenant or condition contained in any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which the Company or any of its subsidiaries is bound or to which any property or asset of the Company or any of its subsidiaries is subject; or (iii) in violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of clauses (ii) and (iii) above, for any such default or violation that would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(r) No Conflicts. The execution, delivery and performance by the Company of this Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by this Agreement will not (i) conflict with or result in a breach or violation of any of the terms or provisions of, or constitute a default under, result in the termination, modification or acceleration of, or result in the creation or imposition of any lien, charge or encumbrance upon any property, right or asset of the Company or any of its subsidiaries pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which the Company or any of its subsidiaries is bound or to which any property, right or asset of the Company or any of its subsidiaries is subject, (ii) result in any violation of the provisions of the charter or by-laws or similar organizational documents of the Company or any of its subsidiaries or (iii) result in the violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation, default, lien, charge or encumbrance that would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(s) No Consents Required. No consent, approval, authorization, order, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Company of this Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by this Agreement and by each Prospectus, except for the registration of the Shares under the Securities Act and such consents, approvals, authorizations, orders and registrations or qualifications as may be required by the Financial Industry Regulatory Authority, Inc. (“**FINRA**”), the Nasdaq Global Select Market or under applicable state securities laws in connection with the sale and distribution of the Shares.

(t) Legal Proceedings. Except as described in the Registration Statement, the Prospectus, and the Time of Sale Information, there are no legal, governmental or regulatory investigations, actions, demands, claims, suits, arbitrations, inquiries or proceedings (“**Actions**”) pending to which the Company or any of its subsidiaries is or may be a party or to which any property of the Company or any of its subsidiaries is or may be the subject that, individually or in the aggregate, if determined adversely to the Company or any of its subsidiaries, would reasonably be expected to have a Material Adverse Effect; to the knowledge of the Company, no such Actions are threatened or contemplated by any governmental or regulatory authority or threatened by others; and (i) there are no current or pending Actions that are required under the Securities Act to be described in the Registration Statement, the Prospectus, and the Time of Sale Information that are not so described in the Registration Statement, the Prospectus, and the Time of Sale Information and (ii) there are no statutes, regulations or contracts or other documents that are required under the Securities Act to be filed as exhibits to the Registration Statement or described

in the Registration Statement, the Prospectus, or the Time of Sale Information that are not so filed as exhibits to the Registration Statement or described in the Registration Statement, the Prospectus, and the Time of Sale Information.

(u) Independent Accountants. PricewaterhouseCoopers LLP, who have certified certain financial statements of the Company and its subsidiaries, is an independent registered public accounting firm with respect to the Company and its subsidiaries within the applicable rules and regulations adopted by the Commission and the Public Company Accounting Oversight Board (United States) and as required by the Securities Act.

(v) Title to Real and Personal Property. The Company and its subsidiaries have good and marketable title in fee simple to, or have valid rights to lease or otherwise use, all items of real and personal property that are material to the respective businesses of the Company and its subsidiaries taken as a whole, in each case free and clear of all liens, encumbrances, claims and defects and imperfections of title except those that (i) do not materially interfere with the use made and proposed to be made of such property by the Company and its subsidiaries or (ii) would not reasonably be expected, individually or in the aggregate, to have a Material Adverse Effect.

(w) Title to Intellectual Property. The Company and its subsidiaries own or license valid and enforceable rights to use all inventions, patent rights, trademarks, service marks, trade names, trade dress, domain names, copyrights, licenses, know-how, trade secrets and other unpatented and/or unpatentable proprietary or confidential information, systems or procedures and all similar intellectual property and proprietary rights (including all registrations and applications for registration of, and all goodwill associated with, any of the foregoing, as applicable) (collectively, “**Intellectual Property**”) described in the Registration Statement, the Prospectus and the Time of Sale Information as being owned by or licensed to the Company and its subsidiaries, and all other Intellectual Property used in or reasonably necessary for the conduct of their business as currently conducted and as proposed to be conducted in the Registration Statement, the Prospectus and the Time of Sale Information. The conduct of the business of the Company and its subsidiaries does not, and the proposed conduct of such business as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information will not, infringe, misappropriate or otherwise violate any Intellectual Property rights of any others. Except as described in the Registration Statement, the Prospectus and the Time of Sale Information, there is no pending or, to the Company’s knowledge, threatened action, suit, proceeding or claim by any others (i) that the Company or any of its subsidiaries infringes, misappropriates or otherwise violates the Intellectual Property of others or (ii) challenging the validity, enforceability, scope or ownership of any Intellectual Property owned by or licensed to the Company or any of its subsidiaries or their rights therein. To the knowledge of the Company, no third party has infringed, misappropriated or otherwise violated any Intellectual Property owned by or exclusively licensed to the Company or any of its subsidiaries. To the Company’s knowledge, none of the Intellectual Property used by the Company or any of its subsidiaries in the conduct of its business has been obtained or is being used by the Company or any of its subsidiaries in material violation of any contractual obligation binding on the Company or any of its subsidiaries. Except as set forth in the Registration Statement, the Prospectus and the Time of Sale Information, the Intellectual Property owned by the Company and its subsidiaries is all solely owned by the Company or its subsidiaries free and clear of any liens or encumbrances. To the Company’s knowledge, no trademark, issued patent, pending patent application (if issued), copyright, or trade secret that is described in the Registration

Statement, the Prospectus and the Time of Sale Information as being owned by or licensed to the Company or any of its subsidiaries is invalid or unenforceable. Neither the Company nor any of its subsidiaries is subject to any judgment, order, writ, injunction or decree of any court or any federal, state, local, foreign or other governmental department, commission, board, bureau, agency or instrumentality, domestic or foreign, or any arbitrator, nor has it entered into or is a party to any agreement made in settlement of any pending or threatened litigation, which materially restricts or impairs its use of any Intellectual Property. The Company and its subsidiaries have taken all commercially reasonable steps, in accordance with normal industry practice, necessary to maintain the confidentiality of all Intellectual Property the value of which to the Company or any of its subsidiaries is contingent upon maintaining the confidentiality thereof, except as would not reasonably be expected to have a Material Adverse Effect, and neither the Company nor any of its subsidiaries is aware of any material disclosure of such Intellectual Property other than to employees, representatives, independent contractors, collaborators, licensors, licensees, agents and advisors of the Company and its subsidiaries, all of whom are bound by written obligations to maintain the confidentiality thereof. All founders, key employees and any other employees involved in the development of Intellectual Property for the Company and its subsidiaries have signed confidentiality and invention assignment agreements or similar agreements for the transfer, assignment, and/or licensing of Intellectual Property with the Company and its subsidiaries pursuant to which the Company and its subsidiaries either (i) have obtained ownership of and are the exclusive owners of or (ii) have obtained a valid and unrestricted right to exploit, sufficient for the conduct of their business, such Intellectual Property.

(x) No Undisclosed Relationships. No relationship, direct or indirect, exists between or among the Company or any of its subsidiaries, on the one hand, and the directors, officers, stockholders, customers, suppliers or other affiliates of the Company or any of its subsidiaries, on the other, that is required by the Securities Act to be described in each of the Registration Statement, the Prospectus and the Time of Sale Information and that is not so described in such documents.

(y) Investment Company Act. The Company is not, and immediately after giving effect to the offering and sale of the Shares and the application of the proceeds thereof as described in the Prospectus will not be, an “investment company” or an entity “controlled” by an “investment company” within the meaning of the Investment Company Act of 1940, as amended, and the rules and regulations of the Commission thereunder (collectively, the “**Investment Company Act**”).

(z) Taxes. The Company and its subsidiaries have paid all federal, state, local and foreign taxes and filed all tax returns required to be paid or filed through the date hereof; and except as otherwise disclosed in each of the Registration Statement, the Prospectus and the Time of Sale Information, there is no tax deficiency that has been, or could reasonably be expected to be, asserted against the Company or any of its subsidiaries or any of their respective properties or assets that would reasonably be expected to have a Material Adverse Effect.

(aa) Licenses and Permits. The Company and its subsidiaries possess all licenses, sub-licenses, certificates, permits and other authorizations issued by, and have made all declarations and filings with, the appropriate federal, state, local or foreign governmental or regulatory authorities that are necessary for the ownership or lease of their respective properties or the conduct of their respective businesses as described in each of the Registration Statement, the Prospectus

and the Time of Sale Information, except where the failure to possess or make the same would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; and except as described in each of the Registration Statement, the Prospectus and the Time of Sale Information, neither the Company nor any of its subsidiaries has received notice of any revocation or material modification of any such license, sub-license, certificate, permit or authorization or has any reason to believe that any such license, sub-license, certificate, permit or authorization will not be renewed in the ordinary course.

(bb) No Labor Disputes. No labor disturbance by or dispute with employees of the Company or any of its subsidiaries exists or, to the knowledge of the Company, is contemplated or threatened, and the Company is not aware of any existing or imminent labor disturbance by, or dispute with, the employees of any of its or its subsidiaries' principal suppliers, contractors or customers, except as would not reasonably be expected to have a Material Adverse Effect. Neither the Company nor any of its subsidiaries has received any notice of cancellation or termination with respect to any collective bargaining agreement to which it is a party.

(cc) Certain Environmental Laws. (i) The Company and its subsidiaries (x) are in compliance with all, and have not violated any, applicable federal, state, local and foreign laws (including common law), rules, regulations, requirements, decisions, judgments, decrees, orders and other legally enforceable requirements relating to pollution or the protection of human health or safety, the environment, natural resources, hazardous or toxic substances or wastes, pollutants or contaminants (collectively, "**Environmental Laws**"); (y) have received and are in compliance with all, and have not violated any, permits, licenses, certificates or other authorizations or approvals required of them under any Environmental Laws to conduct their respective businesses; and (z) have not received notice of any actual or potential liability or obligation under or relating to, or any actual or potential violation of, any Environmental Laws, including for the investigation or remediation of any disposal or release of hazardous or toxic substances or wastes, pollutants or contaminants, and have no knowledge of any event or condition that would reasonably be expected to result in any such notice, and (ii) there are no costs or liabilities associated with Environmental Laws of or relating to the Company or its subsidiaries, except in the case of each of (i) and (ii) above, for any such matter as would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; and (iii) except as described in each of the Registration Statement, the Prospectus and the Time of Sale Information, (x) there is no proceeding that is pending, or that is, to the Company's knowledge, contemplated, against the Company or any of its subsidiaries under any Environmental Laws in which a governmental entity is also a party, other than such proceeding regarding which it is reasonably believed no monetary sanctions of \$100,000 or more will be imposed, (y) the Company and its subsidiaries are not aware of any facts or issues regarding compliance with Environmental Laws, or liabilities or other obligations under Environmental Laws or concerning hazardous or toxic substances or wastes, pollutants or contaminants, that could reasonably be expected to have a material effect on the capital expenditures, earnings or competitive position of the Company and its subsidiaries, and (z) none of the Company or its subsidiaries anticipates material increase in capital expenditures relating to any Environmental Laws.

(dd) Compliance with ERISA. (i) Each employee benefit plan, within the meaning of Section 3(3) of the Employee Retirement Income Security Act of 1974, as amended ("**ERISA**"), for which the Company or any member of its "Controlled Group" (defined as any entity, whether

or not incorporated, that is under common control with the Company within the meaning of Section 4001(a)(14) of ERISA or any entity that would be regarded as a single employer with the Company under Section 414(b), (c), (m) or (o) of the Code) would have any liability (each, a “**Plan**”) has been maintained in compliance with its terms and the requirements of any applicable statutes, orders, rules and regulations, including but not limited to ERISA and the Code, except for noncompliance that would not reasonably be expected to result in material liability to the Company; (ii) no prohibited transaction, within the meaning of Section 406 of ERISA or Section 4975 of the Code, has occurred with respect to any Plan, excluding transactions effected pursuant to a statutory or administrative exemption that would not reasonably be expected to result in material liability to the Company; (iii) for each Plan that is subject to the funding rules of Section 412 of the Code or Section 302 of ERISA, no Plan has failed (whether or not waived), or is reasonably expected to fail, to satisfy the minimum funding standards (within the meaning of Section 302 of ERISA or Section 412 of the Code) applicable to such Plan; (iv) to the knowledge of the Company, no Plan is, or is reasonably expected to be, in “at risk status” (within the meaning of Section 303(i) of ERISA) and no Plan that is a “multiemployer plan” within the meaning of Section 4001(a)(3) of ERISA is in “endangered status” or “critical status” (within the meaning of Sections 304 and 305 of ERISA) (v) the fair market value of the assets of each Plan exceeds the present value of all benefits accrued under such Plan (determined based on those assumptions used to fund such Plan); (vi) no “reportable event” (within the meaning of Section 4043(c) of ERISA and the regulations promulgated thereunder) has occurred or, to the knowledge of the Company, is reasonably expected to occur that either has resulted, or would reasonably be expected to result, in material liability to the Company; (vii) each Plan that is intended to be qualified under Section 401(a) of the Code is so qualified, and nothing has occurred, whether by action or by failure to act, which would reasonably be expected to cause the loss of such qualification; (viii) neither the Company nor any member of the Controlled Group has incurred, nor reasonably expects to incur, any liability under Title IV of ERISA (other than contributions to the Plan or premiums to the Pension Benefit Guarantee Corporation, in the ordinary course and without default) in respect of a Plan (including a “multiemployer plan” within the meaning of Section 4001(a)(3) of ERISA); and (ix) none of the following events has occurred or is reasonably likely to occur: (A) a material increase in the aggregate amount of contributions required to be made to all Plans by the Company or its Controlled Group affiliates in the current fiscal year of the Company and its Controlled Group affiliates compared to the amount of such contributions made in the Company’s and its Controlled Group affiliates’ most recently completed fiscal year; or (B) a material increase in the Company and its subsidiaries’ “accumulated post-retirement benefit obligations” (within the meaning of Accounting Standards Codification Topic 715-60) compared to the amount of such obligations in the Company and its subsidiaries’ most recently completed fiscal year, except in each case with respect to the events or conditions set forth in (i) through (ix) hereof, as would not, individually or in the aggregate, have a Material Adverse Effect.

(ee) Disclosure Controls. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, the Company and its subsidiaries maintain an effective system of “disclosure controls and procedures” (as defined in Rule 13a-15(e) of the Exchange Act that complies with the requirements of the Exchange Act and that has been designed to ensure that information required to be disclosed by the Company in reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the Commission’s rules and forms, including controls and procedures designed to

ensure that such information is accumulated and communicated to the Company's management as appropriate to allow timely decisions regarding required disclosure.

(ff) Accounting Controls. The Company and its subsidiaries maintain systems of "internal control over financial reporting" (as defined in Rule 13a-15(f) of the Exchange Act) that have been designed to comply with the requirements of the Exchange Act and have been designed by, or under the supervision of, their respective principal executive and principal financial officers, or persons performing similar functions, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP. The Company and its subsidiaries maintain internal accounting controls sufficient to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences and (v) interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information fairly presents the information called for in all material respects and is prepared in accordance with the Commission's rules and guidelines applicable thereto. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, there are no material weaknesses in the Company's internal controls. The Company's auditors and the Audit Committee of the Board of Directors of the Company have been advised of: (i) all significant deficiencies and material weaknesses, if any, in the design or operation of internal controls over financial reporting which have adversely affected or are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and (ii) to the knowledge of the Company, any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal controls over financial reporting.

(gg) Insurance. The Company and its subsidiaries have insurance covering their respective properties, operations, personnel and businesses, including business interruption insurance, which insurance is in amounts and insures against such losses and risks which the Company reasonably believes are adequate to protect the Company and its subsidiaries and their respective businesses; and neither the Company nor any of its subsidiaries has (i) received notice from any insurer or agent of such insurer that capital improvements or other expenditures are required or necessary to be made in order to continue such insurance or (ii) any reason to believe that it will not be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage at reasonable cost from similar insurers as may be necessary to continue its business.

(hh) No Unlawful Payments. Neither the Company nor any of its subsidiaries, nor any director, officer or employee of the Company or any of its subsidiaries nor, to the knowledge of the Company, any agent, affiliate or other person associated with or acting on behalf of the Company or any of its subsidiaries has (i) used any corporate funds for any unlawful contribution, gift, entertainment or other unlawful expense relating to political activity; (ii) made or taken an act in furtherance of an offer, promise or authorization of any direct or indirect unlawful payment or benefit to any foreign or domestic government official or employee, including of any government-

owned or controlled entity or of a public international organization, or any person acting in an official capacity for or on behalf of any of the foregoing, or any political party or party official or candidate for political office; (iii) violated or is in violation of any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable law or regulation implementing the OECD Convention on Combating Bribery of Foreign Public Officials in International Business Transactions, or committed an offence under the Bribery Act 2010 of the United Kingdom, or any other applicable anti-bribery or anti-corruption law; or (iv) made, offered, agreed, requested or taken an act in furtherance of any unlawful bribe or other unlawful benefit, including, without limitation, any rebate, payoff, influence payment, kickback or other unlawful or improper payment or benefit. The Company and its subsidiaries have instituted, maintain and enforce, and will continue to maintain and enforce policies and procedures designed to promote and ensure compliance with all applicable anti-bribery and anti-corruption laws.

(ii) Cybersecurity. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, (i)(x) there has been no security breach or other compromise of or relating to any of the Company's information technology and computer systems, networks, hardware, software, data (including the data of its customers, employees, suppliers, vendors and any third party data maintained by or on behalf of the Company), equipment or technology (collectively, "**IT Systems and Data**") and (y) the Company has not been notified of, and has no knowledge of any event or condition that would reasonably be expected to result in, any security breach or other compromise to its IT Systems and Data; (ii) the Company is presently in compliance with all applicable laws or statutes and all judgments, orders, rules and regulations of any court or arbitrator or governmental or regulatory authority, internal policies and contractual obligations relating to the privacy and security of IT Systems and Data and to the protection of such IT Systems and Data from unauthorized use, access, misappropriation or modification, except as would not, in the case of clause (i) and (ii), individually or in the aggregate, have a Material Adverse Effect; and (iii) the Company has implemented backup and disaster recovery technology consistent with industry standards and practices.

(jj) Compliance with Anti-Money Laundering Laws. The operations of the Company and its subsidiaries are and have been conducted at all times in compliance with applicable financial recordkeeping and reporting requirements, including those of the Currency and Foreign Transactions Reporting Act of 1970, as amended, the applicable money laundering statutes of all jurisdictions where the Company or any of its subsidiaries conducts business, the rules and regulations thereunder and any related or similar rules, regulations or guidelines, issued, administered or enforced by any governmental agency (collectively, the "**Anti-Money Laundering Laws**"), and no action, suit or proceeding by or before any court or governmental agency, authority or body or any arbitrator involving the Company or any of its subsidiaries with respect to the Anti-Money Laundering Laws is pending or, to the knowledge of the Company, threatened.

(kk) No Conflicts with Sanctions Laws. Neither the Company nor any of its subsidiaries, directors, officers or employees, nor, to the knowledge of the Company, any agent, affiliate or other person associated with or acting on behalf of the Company or any of its subsidiaries is currently the subject or the target of any sanctions administered or enforced by the U.S. government (including, without limitation, the Office of Foreign Assets Control of the U.S. Department of the Treasury ("**OFAC**") or the U.S. Department of State and including, without

limitation, the designation as a “specially designated national” or “blocked person”), the United Nations Security Council (“UNSC”), the European Union, Her Majesty’s Treasury (“HMT”) or other relevant sanctions authority (collectively, “Sanctions”), nor is the Company or any of its subsidiaries located, organized or resident in a country or territory that is the subject or target of Sanctions, including, without limitation, Cuba, Iran, North Korea, Syria and Crimea (each, a “Sanctioned Country”); and the Company will not directly or indirectly use the proceeds of the offering of the Shares hereunder, or lend, contribute or otherwise make available such proceeds to any subsidiary, joint venture partner or other person or entity (i) to fund or facilitate any activities of or business with any person that, at the time of such funding or facilitation, is the subject or target of Sanctions, (ii) to fund or facilitate any activities of or business in any Sanctioned Country or (iii) in any other manner that will result in a violation by any person (including any person participating in the transaction, whether as agent, advisor, investor or otherwise) of Sanctions. For the past five years, the Company and its subsidiaries have not knowingly engaged in and are not now knowingly engaged in any dealings or transactions with any person that at the time of the dealing or transaction is or was the subject or the target of Sanctions or with any Sanctioned Country.

(ll) No Restrictions on Subsidiaries. No subsidiary of the Company is currently prohibited, directly or indirectly, under any agreement or other instrument to which it is a party or is subject, from paying any dividends to the Company, from making any other distribution on such subsidiary’s capital stock or similar ownership interest, from repaying to the Company any loans or advances to such subsidiary from the Company or from transferring any of such subsidiary’s properties or assets to the Company or any other subsidiary of the Company.

(mm) No Broker’s Fees. Neither the Company nor any of its subsidiaries is a party to any contract, agreement or understanding with any person (other than this Agreement) that would give rise to a valid claim against any of them for a brokerage commission, finder’s fee or like payment in connection with the transactions contemplated by this Agreement or any Prospectus.

(nn) No Registration Rights. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, no person has the right to require the Company or any of its subsidiaries to register any securities for sale under the Securities Act by reason of the filing of the Registration Statement with the Commission or the issuance and sale of the Shares, other than rights that have been validly waived.

(oo) No Stabilization. Neither the Company nor any of its subsidiaries has taken, directly or indirectly, without given effect to activities by the Agent, any action designed to or that would reasonably be expected to cause or result in any stabilization or manipulation of the price of the Shares or any other “reference security” (as defined in Rule 100 of Regulation M under the Exchange Act (“Regulation M”)) whether to facilitate the sale or resale of the Shares or otherwise, and has taken no action which would directly or indirectly violate Regulation M. The Company acknowledges that the Agent may engage in passive market making transactions in the Shares on the Nasdaq Global Select Market in accordance with Regulation M.

(pp) Margin Rules. Neither the issuance, sale and delivery of the Shares nor the application of the proceeds thereof by the Company as described in each of the Registration Statement, the Prospectus and the Time of Sale Information will violate Regulation T, U or X of

the Board of Governors of the Federal Reserve System or any other regulation of such Board of Governors.

(qq) Forward-Looking Statements. No forward-looking statement (within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act) included or incorporated by reference in any of the Registration Statement, the Prospectus or the Time of Sale Information has been made or reaffirmed without a reasonable basis or has been disclosed other than in good faith.

(rr) Statistical and Market Data. Nothing has come to the attention of the Company that has caused the Company to believe that the statistical and market-related data included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information is not based on or derived from sources that are reliable and accurate in all material respects.

(ss) eXtensible Business Reporting Language. The interactive data in eXtensible Business Reporting Language included or incorporated by reference in the Registration Statement, the Prospectus and the Time of Sale Information fairly presents the information called for in all material respects and has been prepared in accordance with the Commission's rules and guidelines applicable thereto.

(tt) Preclinical Studies and Clinical Trials. (i) Except as described in the Registration Statement, the Prospectus and the Time of Sale Information, the preclinical studies and clinical trials conducted by or, to the knowledge of the Company, on behalf of or sponsored by the Company or its subsidiaries, or in which the Company or its subsidiaries have participated, that are described in the Registration Statement, the Prospectus and the Time of Sale Information, or the results of which are referred to in the Registration Statement, the Prospectus and the Time of Sale Information, as applicable, were, and if still pending are, being conducted in all material respects in accordance with standard medical and scientific research standards and procedures for products or product candidates comparable to those being developed by the Company and all applicable statutes and all applicable rules and regulations of the U.S. Food and Drug Administration and comparable regulatory agencies outside of the United States to which they are subject, including the European Medicines Agency (collectively, the "**Regulatory Authorities**") and Good Clinical Practice and Good Laboratory Practice requirements; (ii) the descriptions in the Registration Statement, the Prospectus and the Time of Sale Information of the results of such studies and trials are accurate and complete descriptions in all material respects and fairly present the data derived therefrom; (iii) the Company has no knowledge of any other studies or trials not described in the Registration Statement, the Prospectus and the Time of Sale Information, the results of which the Company believes are inconsistent with or reasonably call into question the results described or referred to in the Registration Statement, the Prospectus and the Time of Sale Information; (iv) the Company and its subsidiaries have operated at all times and are currently in compliance with all applicable statutes, rules and regulations of the Regulatory Authorities, except where such non-compliance would not, individually or in the aggregate, have a Material Adverse Effect; (v) the Company has provided the Agent with all substantive written notices, correspondence and summaries of all other communications from the Regulatory Authorities; and (vi) neither the Company nor any of its subsidiaries have received any written notices, correspondence or other communications from the Regulatory Authorities or any other

governmental agency requiring or threatening the termination, material modification or suspension of any preclinical studies or clinical trials that are described in the Registration Statement, the Prospectus and the Time of Sale Information or the results of which are referred to in the Registration Statement, the Prospectus and the Time of Sale Information, other than ordinary course communications with respect to modifications in connection with the design and implementation of such studies or trials, and, to the Company's knowledge, there are no reasonable grounds for the same.

(uu) Regulatory Filings. The Company has not failed to file with the Regulatory Authorities any required filing, declaration, listing, registration, report or submission with respect to the Company's product candidates that are described or referred to in the Registration Statement, the Prospectus and the Time of Sale Information; all such filings, declarations, listings, registrations, reports or submissions, as applicable, were in material compliance with applicable laws when filed; and no material deficiencies regarding compliance with applicable law have been asserted by any applicable regulatory authority with respect to any such filings, declarations, listings, registrations, reports or submissions.

(vv) Sarbanes-Oxley Act. There is and has been no material failure on the part of the Company or, to the Company's knowledge, any of the Company's directors or officers, in their capacities as such, to comply with any applicable provision of the Sarbanes-Oxley Act of 2002, as amended, and the rules and regulations promulgated in connection therewith (the "**Sarbanes-Oxley Act**"), including Section 402 related to loans and Sections 302 and 906 related to certifications.

Any certificate signed by any officer or representative of the Company or any of its subsidiaries and delivered to the Agent or counsel for the Agent in connection with an issuance of Shares shall be deemed a representation and warranty by the Company to the Agent as to the matters covered thereby on the date of such certificate.

The Company acknowledges that the Agent and, for purposes of the opinions to be delivered pursuant to Section 4(p) hereof, counsel to the Company and counsel to the Agent, will rely upon the accuracy and truthfulness of the foregoing representations and hereby consents to such reliance.

Section 3. ISSUANCE AND SALE OF COMMON SHARES

(a) (i) Sale of Securities. On the basis of the representations, warranties and agreements herein contained, but subject to the terms and conditions herein set forth, the Company and the Agent agree that the Company may from time to time seek to sell Shares through the Agent, acting as sales agent, or directly to the Agent, acting as principal, as follows, with an aggregate Sales Price of up to the Maximum Program Amount, based on and in accordance with Issuance Notices as the Company may deliver, during the Agency Period.

(b) Mechanics of Issuances.

(i) Issuance Notice. Upon the terms and subject to the conditions set forth herein, on any Trading Day during the Agency Period on which the conditions set forth in Section 5(a) and Section 5(b) shall have been satisfied, the Company may exercise its right to request an issuance o

f Shares by delivering to the Agent an Issuance Notice; *provided, however*, that (A) in no event may the Company deliver an Issuance Notice to the extent that (I) the sum of (x) the aggregate Sales Price of the requested Issuance Amount, plus (y) the aggregate Sales Price of all Shares issued under all previous Issuance Notices effected pursuant to this Agreement, would exceed the Maximum Program Amount; and (B) prior to delivery of any Issuance Notice, the Selling Period for any previous Issuance Notice shall have expired or been terminated. An Issuance Notice shall be considered delivered on the Trading Day that it is received by e-mail to the persons so identified in writing by the Agent and confirmed by the Company by telephone (including a voicemail message to the persons so identified), with the understanding that, with adequate prior written notice, the Agent may modify the list of such persons from time to time.

(ii) Agent Efforts. Upon the terms and subject to the conditions set forth in this Agreement, upon the receipt of an Issuance Notice, the Agent will use its commercially reasonable efforts consistent with its normal sales and trading practices to place the Shares with respect to which the Agent has agreed to act as sales agent, subject to, and in accordance with the information specified in, the Issuance Notice, unless the sale of the Shares described therein has been suspended, cancelled or otherwise terminated in accordance with the terms of this Agreement. For the avoidance of doubt, the parties to this Agreement may modify an Issuance Notice at any time provided they both agree in writing to any such modification.

(iii) Method of Offer and Sale. The Shares may be offered and sold (A) in privately negotiated transactions, (B) as block transactions or (C) by any other method or payment permitted by law deemed to be an “at the market” offering as defined in Rule 415 under the Securities Act, including sales made directly on the Principal Market, sales made into any other existing trading market or sales made to or through a market maker or through an electronic communications network. Nothing in this Agreement shall be deemed to require either party to agree to the method of offer and sale specified in the preceding sentence, and the method of placement of any Shares by the Agent shall be at the Agent’s discretion.

(iv) Confirmation to the Company. If acting as sales agent hereunder, the Agent will provide written confirmation to the Company no later than the opening of the Trading Day next following the Trading Day on which it has placed Shares hereunder setting forth the number of shares sold on such Trading Day, the corresponding Sales Price and the Issuance Price payable to the Company in respect thereof.

(v) Settlement. Each issuance of Shares will be settled on the applicable Settlement Date for such issuance of Shares and, subject to the provisions of Section 5, on or before each Settlement Date, the Company will, or will cause its transfer agent to, electronically transfer the Shares being sold by crediting the Agent or its designee’s account at The Depository Trust Company through its Deposit/Withdrawal At Custodian (DWAC) System, or by such other means of delivery as may be mutually agreed upon by the parties hereto and, upon receipt of such Shares, which in all cases shall be freely tradable, transferable, registered shares in good deliverable form, the Agent will deliver, by wire transfer of immediately available funds, the related Issuance Price in same day funds delivered to an account designated by the Company prior to the Settlement Date. The Company may sell Shares to the Agent as principal at a price agreed upon at each relevant time Shares are sold pursuant to this Agreement (each, a “**Time of Sale**”).

(vi) Suspension or Termination of Sales. Consistent with standard market settlement practices, the Company or the Agent may, upon notice to the other party hereto in writing or by telephone (confirmed immediately by verifiable email), suspend any sale of Shares, and the Selling Period shall immediately terminate; *provided, however*, that (A) such suspension and termination shall not affect or impair either party's obligations with respect to any Shares placed or sold hereunder prior to the receipt of such notice; (B) if the Company suspends or terminates any sale of Shares after the Agent confirms such sale to the Company, the Company shall still be obligated to comply with Section 3(b)(v) with respect to such Shares; and (C) if the Company defaults in its obligation to deliver Shares on a Settlement Date, the Company agrees that it will hold the Agent harmless against any loss, claim, damage or expense (including, without limitation, penalties, interest and reasonable legal fees and expenses), as incurred, arising out of or in connection with such default by the Company. The parties hereto acknowledge and agree that, in performing its obligations under this Agreement, the Agent may borrow Common Shares from stock lenders in the event that the Company has not delivered Shares to settle sales as required by subsection (v) above, and may use the Shares to settle or close out such borrowings. The Company agrees that no such notice shall be effective against the Agent unless it is made to the persons identified in writing by the Agent pursuant to Section 3(b)(i).

(vii) No Guarantee of Placement, Etc. The Company acknowledges and agrees that (A) there can be no assurance that the Agent will be successful in placing Shares, (B) the Agent will incur no liability or obligation to the Company or any other Person if it does not sell Shares and (C) the Agent shall be under no obligation to purchase Shares on a principal basis pursuant to this Agreement, except as otherwise specifically agreed by the Agent and the Company.

(viii) Material Non-Public Information. Notwithstanding any other provision of this Agreement, the Company and the Agent agree that the Company shall not deliver any Issuance Notice to the Agent, and the Agent shall not be obligated to place any Shares, during any period in which the Company is in possession of material non-public information.

(c) Fees. As compensation for services rendered, the Company shall pay to the Agent, on the applicable Settlement Date, the Selling Commission for the applicable Issuance Amount (including with respect to any suspended or terminated sale pursuant to Section 3(b)(vi)) by the Agent deducting the Selling Commission from the applicable Issuance Amount.

(d) Expenses. The Company agrees to pay all costs, fees and expenses incurred in connection with the performance of its obligations hereunder and in connection with the transactions contemplated hereby, including without limitation (i) all expenses incident to the issuance and delivery of the Shares (including all printing and engraving costs), (ii) all fees and expenses of the registrar and transfer agent of the Shares, (iii) all necessary issue, transfer and other stamp taxes in connection with the issuance and sale of the Shares, (iv) all fees and expenses of the Company's counsel, independent public or certified public accountants and other advisors, (v) all costs and expenses incurred in connection with the preparation, printing, filing, shipping and distribution of the Registration Statement (including financial statements, exhibits, schedules, consents and certificates of experts), the Prospectus, any Free Writing Prospectus (as defined below) prepared by or on behalf of, used by, or referred to by the Company, and all amendments and supplements thereto, and this Agreement, (vi) all filing fees, attorneys' fees and expenses incurred by the Company or the Agent in connection with qualifying or registering (or obtaining

exemptions from the qualification or registration of) all or any part of the Shares for offer and sale under the state securities or blue sky laws or the provincial securities laws of Canada, and, if requested by the Agent, preparing and printing a “**Blue Sky Survey**” or memorandum and a “Canadian wrapper”, and any supplements thereto, advising the Agent of such qualifications, registrations, determinations and exemptions, (vii) the reasonable fees and disbursements of the Agent’s counsel, including the reasonable fees and expenses of counsel for the Agent in connection with FINRA review, if any, and approval of the Agent’s participation in the offering and distribution of the Shares, (viii) the filing fees incident to FINRA review, if any, (ix) the costs and expenses of the Company relating to investor presentations on any “road show” undertaken in connection with the marketing of the offering of the Shares, including, without limitation, expenses associated with the preparation or dissemination of any electronic road show, expenses associated with the production of road show slides and graphics, fees and expenses of any consultants engaged in connection with the road show presentations with the prior approval of the Company, travel and lodging expenses of the representatives, employees and officers of the Company and of the Agent and any such consultants, and the cost of any aircraft chartered in connection with the road show and (x) the fees and expenses associated with listing the Shares on the Nasdaq Global Select Market.

Section 4. ADDITIONAL COVENANTS

The Company covenants and agrees with the Agent as follows, in addition to any other covenants and agreements made elsewhere in this Agreement:

(a) **Exchange Act Compliance.** During the Agency Period, the Company shall (i) file, on a timely basis, with the Commission all reports and documents required to be filed under Section 13, 14 or 15 of the Exchange Act in the manner and within the time periods required by the Exchange Act and (ii) either (A) include in its quarterly reports on Form 10-Q and its annual reports on Form 10-K, a summary detailing, for the relevant reporting period, (1) the number of Shares sold through the Agent pursuant to this Agreement and (2) the net proceeds received by the Company from such sales or (B) prepare a prospectus supplement or in such other filing permitted by the Securities Act or Exchange Act (each an “**Interim Prospectus Supplement**”) with such summary information and, at least once a quarter and subject to this Section 4, file such Interim Prospectus Supplement pursuant to Rule 424(b) under the Securities Act (and within the time periods required by Rule 424(b) and Rule 430B under the Securities Act)).

(b) **Securities Act Compliance.** After the date of this Agreement, the Company shall promptly advise the Agent in writing (i) of the receipt of any comments of, or requests for additional or supplemental information from, the Commission, (ii) of the time and date of any filing of any post-effective amendment to the Registration Statement, any Rule 462(b) Registration Statement or any amendment or supplement to the Prospectus, any Free Writing Prospectus, (iii) of the time and date that any post-effective amendment to the Registration Statement or any Rule 462(b) Registration Statement becomes effective and (iv) of the issuance by the Commission of any stop order suspending the effectiveness of the Registration Statement or any post-effective amendment thereto, any Rule 462(b) Registration Statement or any amendment or supplement to the Prospectus or of any order preventing or suspending the use of any Free Writing Prospectus or the Prospectus, or of any proceedings to remove, suspend or terminate from listing or quotation the Common Shares from any securities exchange upon which they are listed for trading or

included or designated for quotation, or of the threatening or initiation of any proceedings for any of such purposes. If the Commission shall enter any such stop order at any time, the Company will use its best efforts to obtain the lifting of such order at the earliest possible moment. Additionally, the Company agrees that it shall comply with the provisions of Rule 424(b) and Rule 433, as applicable, under the Securities Act and will use its reasonable efforts to confirm that any filings made by the Company under such Rule 424(b) or Rule 433 were received in a timely manner by the Commission.

(c) Amendments and Supplements to the Prospectus and Other Securities Act Matters. If any event shall occur or condition exist as a result of which it is necessary to amend or supplement the Prospectus so that the Prospectus does not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances when the Prospectus is delivered to a purchaser, not misleading, or if in the opinion of the Agent or counsel for the Agent it is otherwise necessary to amend or supplement the Prospectus to comply with applicable law, including the Securities Act, the Company agrees (subject to Section 3(b) and 3(c)) to promptly prepare, file with the Commission and furnish at its own expense to the Agent, amendments or supplements to the Prospectus so that the statements in the Prospectus as so amended or supplemented will not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances when the Prospectus is delivered to a purchaser, be misleading or so that the Prospectus, as amended or supplemented, will comply with applicable law including the Securities Act. Neither the Agent's consent to, or delivery of, any such amendment or supplement shall constitute a waiver of any of the Company's obligations under Sections 3(b) or (c).

(d) Agent's Review of Proposed Amendments and Supplements. Prior to amending or supplementing the Registration Statement (including any registration statement filed under Rule 462(b) under the Securities Act, but excluding (i) documents incorporated by reference filed under the Exchange Act that either (A) do not name Agent and do not relate to the transactions contemplated by this Agreement or (B) only include disclosure relating to periodic sales pursuant to this Agreement, and (ii) amendments or supplements that do not name Agent and do not relate to the transactions contemplated by this Agreement) or the Prospectus (excluding documents incorporated by reference filed under the Exchange Act that either (A) do not name Agent and do not relate to the transactions contemplated by this Agreement or (B) only include disclosure relating to periodic sales pursuant to this Agreement), the Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of each such proposed amendment or supplement, and the Company shall not file or use any such proposed amendment or supplement without the Agent's prior consent, and to file with the Commission within the applicable period specified in Rule 424(b) under the Securities Act any prospectus required to be filed pursuant to such Rule.

(e) Use of Free Writing Prospectus. Neither the Company nor the Agent has prepared, used, referred to or distributed, or will prepare, use, refer to or distribute, without the other party's prior written consent, any "written communication" that constitutes a "free writing prospectus" as such terms are defined in Rule 405 under the Securities Act with respect to the offering contemplated by this Agreement (any such free writing prospectus being referred to herein as a "**Free Writing Prospectus**").

(f) Free Writing Prospectuses. The Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of each proposed free writing prospectus or any amendment or supplement thereto to be prepared by or on behalf of, used by, or referred to by the Company and the Company shall not file, use or refer to any proposed free writing prospectus or any amendment or supplement thereto without the Agent's consent. The Company shall furnish to the Agent, without charge, as many copies of any free writing prospectus prepared by or on behalf of, or used by the Company, as the Agent may reasonably request. If at any time when a prospectus is required by the Securities Act (including, without limitation, pursuant to Rule 173(d)) to be delivered in connection with sales of the Shares (but in any event if at any time through and including the date of this Agreement) there occurred or occurs an event or development as a result of which any free writing prospectus prepared by or on behalf of, used by, or referred to by the Company conflicted or would conflict with the information contained in the Registration Statement or included or would include an untrue statement of a material fact or omitted or would omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances prevailing at that subsequent time, not misleading, the Company shall promptly amend or supplement such free writing prospectus to eliminate or correct such conflict or so that the statements in such free writing prospectus as so amended or supplemented will not include an untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances prevailing at such subsequent time, not misleading, as the case may be; *provided, however*, that prior to amending or supplementing any such free writing prospectus, the Company shall furnish to the Agent for review, a reasonable amount of time prior to the proposed time of filing or use thereof, a copy of such proposed amended or supplemented free writing prospectus and the Company shall not file, use or refer to any such amended or supplemented free writing prospectus without the Agent's consent.

(g) Filing of Agent Free Writing Prospectuses. The Company shall not take any action that would result in the Agent or the Company being required to file with the Commission pursuant to Rule 433(d) under the Securities Act a free writing prospectus prepared by or on behalf of the Agent that the Agent otherwise would not have been required to file thereunder.

(h) Copies of Registration Statement and Prospectus. After the date of this Agreement through the last time that a prospectus is required by the Securities Act (including, without limitation, pursuant to Rule 173(d)) to be delivered in connection with sales of the Shares, the Company agrees to furnish the Agent with copies (which may be electronic copies) of the Registration Statement and each amendment thereto, and with copies of the Prospectus and each amendment or supplement thereto in the form in which it is filed with the Commission pursuant to the Securities Act or Rule 424(b) under the Securities Act, both in such quantities as the Agent may reasonably request from time to time; and, if the delivery of a prospectus is required under the Securities Act or under the blue sky or securities laws of any jurisdiction at any time on or prior to the applicable Settlement Date for any Selling Period in connection with the offering or sale of the Shares and if at such time any event has occurred as a result of which the Prospectus as then amended or supplemented would include an untrue statement of a material fact or omit to state any material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made when such Prospectus is delivered, not misleading, or, if for any other reason it is necessary during such same period to amend or supplement the Prospectus or to file under the Exchange Act any document incorporated by reference in the

Prospectus in order to comply with the Securities Act or the Exchange Act, to notify the Agent and to request that the Agent suspend offers to sell Shares (and, if so notified, the Agent shall cease such offers as soon as practicable); and if the Company decides to amend or supplement the Registration Statement or the Prospectus as then amended or supplemented, to advise the Agent promptly by telephone (with confirmation in writing) and to prepare and cause to be filed promptly with the Commission an amendment or supplement to the Registration Statement or the Prospectus as then amended or supplemented that will correct such statement or omission or effect such compliance (it being acknowledged that the Company may delay the filing of any amendment or supplement, if, in the judgment of the Company, it is in the best interest of the Company); provided, however, that if during such same period the Agent is required to deliver a prospectus in respect of transactions in the Shares, the Company shall promptly prepare and file with the Commission such an amendment or supplement.

(i) Blue Sky Compliance. The Company shall cooperate with the Agent and counsel for the Agent to qualify or register the Shares for sale under (or obtain exemptions from the application of) the state securities or blue sky laws or Canadian provincial securities laws of those jurisdictions designated by the Agent, shall comply with such laws and shall continue such qualifications, registrations and exemptions in effect so long as required for the distribution of the Shares. The Company shall not be required to qualify as a foreign corporation or to take any action that would subject it to general service of process in any such jurisdiction where it is not presently qualified or where it would be subject to taxation as a foreign corporation. The Company will advise the Agent promptly of the suspension of the qualification or registration of (or any such exemption relating to) the Shares for offering, sale or trading in any jurisdiction or any initiation or threat of any proceeding for any such purpose, and in the event of the issuance of any order suspending such qualification, registration or exemption, the Company shall use its best efforts to obtain the withdrawal thereof at the earliest possible moment.

(j) Earnings Statement. As soon as practicable, the Company will make generally available to its security holders and to the Agent an earnings statement (which need not be audited) covering a period of at least twelve months beginning with the first fiscal quarter of the Company occurring after the date of this Agreement which shall satisfy the provisions of Section 11(a) of the Securities Act and Rule 158 under the Securities Act; provided that the Company will be deemed to have furnished such statements to its security holders and the Agent to the extent they are filed on EDGAR or any successor system.

(k) Listing; Reservation of Shares. (a) The Company will maintain the listing of the Shares on the Nasdaq Global Select Market, and (b) the Company will reserve and keep available at all times, free of preemptive rights, Shares for the purpose of enabling the Company to satisfy its obligations under this Agreement.

(l) Transfer Agent. The Company shall engage and maintain, at its expense, a registrar and transfer agent for the Shares.

(m) Due Diligence. During the term of this Agreement, the Company will reasonably cooperate with any reasonable due diligence review conducted by the Agent in connection with the transactions contemplated hereby, including, without limitation, providing information and

making available documents and senior corporate officers, during normal business hours and at the Company's principal offices, as the Agent may reasonably request from time to time.

(n) Representations and Warranties. The Company acknowledges that each delivery of an Issuance Notice and each delivery of Shares on a Settlement Date shall be deemed to be (i) an affirmation to the Agent that the representations and warranties of the Company contained in or made pursuant to this Agreement are true and correct as of the date of such Issuance Notice or of such Settlement Date, as the case may be, as though made at and as of each such date, except as may be disclosed in the Prospectus (including any documents incorporated by reference therein and any supplements thereto), and (ii) an undertaking that the Company will advise the Agent if any of such representations and warranties will not be true and correct as of the Settlement Date for the Shares relating to such Issuance Notice, as though made at and as of each such date (except that such representations and warranties shall be deemed to relate to the Registration Statement and the Prospectus as amended and supplemented relating to such Shares).

(o) Deliverables at Triggering Event Dates; Certificates. The Company agrees that on or prior to the date of the first Issuance Notice and, during the term of this Agreement after the date of the first Issuance Notice, upon:

(i) the filing of the amendment or supplement of any Registration Statement or Prospectus (other than a prospectus supplement relating solely to an offering of securities other than the Shares or a prospectus filed pursuant to Section 4(a)(ii)(B)), by means of a post-effective amendment, sticker or supplement, but not by means of incorporation of documents by reference into the Registration Statement or Prospectus;

(ii) the filing with the Commission of an annual report on Form 10-K or a quarterly report on Form 10-Q (including any Form 10-K/A or Form 10-Q/A containing amended financial information or a material amendment to the previously filed annual report on Form 10-K or quarterly report on Form 10-Q), in each case, of the Company; or

(iii) the filing with the Commission of a current report on Form 8-K of the Company containing amended financial information (other than information "furnished" pursuant to Item 2.02 or 7.01 of Form 8-K or to provide disclosure pursuant to Item 8.01 of Form 8-K relating to reclassification of certain properties as discontinued operations in accordance with Statement of Financial Accounting Standards No. 144) that is material to the offering of securities of the Company in the Agent's reasonable discretion;

(any such event, a "**Triggering Event Date**"), the Company shall furnish the Agent (but in the case of clause (iii) above only if the Agent reasonably determines that the information contained in such current report on Form 8-K of the Company is material) with a certificate as of the Triggering Event Date, in the form and substance satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel, modified, as necessary, to relate to the Registration Statement and the Prospectus as amended or supplemented, (A) confirming that the representations and warranties of the Company contained in this Agreement are true and correct, (B) that the Company has performed all of its obligations hereunder to be performed on or prior to the date of such certificate and as to the matters set forth in Section 5(a)(i) hereof, and (C) containing any other certification that the Agent shall reasonably

request. The requirement to provide a certificate under this Section 4(o) shall be waived for any Triggering Event Date occurring at a time when no Issuance Notice is pending or a suspension pursuant to Section 3(b)(vi) is in effect, which waiver shall continue until the earlier to occur of the date the Company delivers instructions for the sale of Shares hereunder (which for such calendar quarter shall be considered a Triggering Event Date) and the next occurring Triggering Event Date. Notwithstanding the foregoing, if the Company subsequently decides to sell Shares following a Triggering Event Date when a suspension was in effect and did not provide the Agent with a certificate under this Section 4(o), then before the Company delivers the instructions for the sale of Shares or the Agent sells any Shares pursuant to such instructions, the Company shall provide the Agent with a certificate in conformity with this Section 4(o) dated as of the date that the instructions for the sale of Shares are issued.

(p) Legal Opinions. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, a negative assurances letter and the written legal opinion of Wilmer Cutler Pickering Hale and Dorr LLP, counsel to the Company, to the effect set forth in Exhibit B hereto, and the written legal opinion of Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C., intellectual property counsel to the Company, to the effect set forth in Exhibit C hereto, each dated the date of delivery, in form and substance reasonably satisfactory to Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel, modified, as necessary, to relate to the Registration Statement and the Prospectus as then amended or supplemented; *provided* that the Company shall be required to furnish no more than one opinion from each of Company's counsel per fiscal quarter. In lieu of such opinions for subsequent periodic filings, in the discretion of the Agent, the Company may furnish a reliance letter from such counsel to the Agent, permitting the Agent to rely on a previously delivered opinion letter, modified as appropriate for any passage of time or Triggering Event Date (except that statements in such prior opinion shall be deemed to relate to the Registration Statement and the Prospectus as amended or supplemented as of such Triggering Event Date).

(q) Comfort Letter. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall cause PricewaterhouseCoopers LLP, the independent registered public accounting firm who has audited the financial statements included or incorporated by reference in the Registration Statement, to furnish the Agent a comfort letter, dated the date of delivery, in form and substance reasonably satisfactory to the Agent and its counsel, substantially similar to the form previously provided to the Agent and its counsel; *provided*, however, that any such comfort letter will only be required on the Triggering Event Date specified to the extent that it contains financial statements filed with the Commission under the Exchange Act and incorporated or deemed to be incorporated by reference into a Prospectus and *provided* further that the Company shall be required to furnish no more than one comfort letter per fiscal quarter. If requested by the Agent, the Company shall also cause a comfort letter to be furnished to the Agent within ten (10) Trading Days of the date of occurrence of any material transaction or event requiring the filing of a current report on Form 8-K containing material amended financial information of the Company, including the restatement of the Company's financial statements.

(r) Secretary's Certificate. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall furnish the Agent a certificate executed by the Secretary of the Company, signing in such capacity, dated the Applicable Time (i) certifying that attached thereto are true and complete copies of the resolutions duly adopted by the Board of Directors of the Company authorizing the execution and delivery of this Agreement and the consummation of the transactions contemplated hereby (including, without limitation, the issuance of the Shares pursuant to this Agreement), which authorization shall be in full force and effect on and as of the date of such certificate, (ii) certifying and attesting to the office, incumbency, due authority and specimen signatures of each Person who executed this Agreement for or on behalf of the Company, and (iii) containing any other certification that the Agent or its counsel shall reasonably request.

(s) Officer's Certificate. On or prior to the date of the first Issuance Notice and within five (5) Trading Days of each Triggering Event Date with respect to which the Company is obligated to deliver a certificate pursuant to Section 4(o) for which no waiver is applicable and excluding the date of this Agreement, the Company shall furnish the Agent a certificate executed by the Chief Executive Officer, President or Chief Financial Officer of the Company, signing in such capacity, dated the Applicable Time to the effect that all conditions to the delivery of the applicable Issuance Notice shall have been satisfied as at the date of such certificate (which certificate shall not be required if the foregoing representations shall be set forth in the Issuance Notice).

(t) Agent's Own Account; Clients' Account. The Company consents to the Agent trading, in compliance with applicable law, in the Common Shares for the Agent's own account and for the account of its clients at the same time as sales of the Shares occur pursuant to this Agreement.

(u) Investment Limitation. The Company shall not invest, or otherwise use the proceeds received by the Company from its sale of the Shares in such a manner as would require the Company or any of its subsidiaries to register as an investment company under the Investment Company Act.

(v) Market Activities. The Company will not take, directly or indirectly, any action designed to or that might be reasonably expected to cause or result in stabilization or manipulation of the price of the Shares or any other reference security, whether to facilitate the sale or resale of the Shares or otherwise, and the Company will, and shall cause each of its affiliates to, comply with all applicable provisions of Regulation M. If the limitations of Rule 102 of Regulation M ("**Rule 102**") do not apply with respect to the Shares or any other reference security pursuant to any exception set forth in Section (d) of Rule 102, then promptly upon notice from the Agent (or, if later, at the time stated in the notice), the Company will, and shall cause each of its affiliates to, comply with Rule 102 as though such exception were not available but the other provisions of Rule 102 (as interpreted by the Commission) did apply. If the exemptive provisions set forth in Rule 101(c)(1) of Regulation M under the 1934 Act are not satisfied with respect to the Company or the Shares, the Company shall promptly notify the Agent.

(w) Notice of Other Sale. Without the written consent of the Agent, the Company will not, directly or indirectly, offer to sell, sell, contract to sell, grant any option to sell or otherwise dispose of any Common Shares or securities convertible into or exchangeable for Common Shares (other than Shares hereunder), warrants or any rights to purchase or acquire Common Shares, during the period beginning on the third Trading Day immediately prior to the date on which any Issuance Notice is delivered to the Agent hereunder and ending on the third Trading Day immediately following the Settlement Date with respect to Shares sold pursuant to such Issuance Notice; provided, however, that such restriction will not be required in connection with (i) the Company's issuance or sale of Common Shares, options to purchase Common Shares or Common Shares issuable upon the exercise of options granted under Company Stock Plans or warrants described as outstanding in the Registration Statement, the Prospectus and the Time of Sale Information, (ii) any options and other awards granted under Company Stock Plans described in the Registration Statement, the Prospectus and the Time of Sale Information, and (iii) shares of Stock or other securities issued in connection with a transaction with an unaffiliated third party that includes a bona fide commercial relationship (including joint ventures, marketing or distribution arrangements, collaboration agreements or intellectual property license agreements) or any acquisition of assets or acquisition of not less than a majority or controlling portion of the equity of another entity.

(x) If immediately prior to the third anniversary (the "**Renewal Deadline**") of the initial effective date of the Registration Statement, the Company has not sold the Maximum Program Amount and this Agreement has not expired or been terminated, the Company will, prior to the Renewal Deadline, file, if it has not already done so and is eligible to do so, a new automatic shelf registration statement relating to the Shares, in a form satisfactory to the Agent. The Company will, prior to the Renewal Deadline, if it has not already done so, file a new shelf registration statement relating to the Shares, in a form satisfactory to the Agent, and will use its best efforts to cause such registration statement to be declared effective within 60 days after the Renewal Deadline. The Company will take all other action necessary or appropriate to permit the issuance and sale of the Shares to continue as contemplated in the expired registration statement relating to the Shares. References herein to the Registration Statement shall include such new automatic shelf registration statement or such new shelf registration statement, as the case may be.

Section 5. CONDITIONS TO DELIVERY OF ISSUANCE NOTICES AND TO SETTLEMENT

(a) Conditions Precedent to the Right of the Company to Deliver an Issuance Notice and the Obligation of the Agent to Sell Shares During the Selling Period(s). The right of the Company to deliver an Issuance Notice hereunder is subject to the satisfaction, on the date of delivery of such Issuance Notice, and the obligation of the Agent to use its commercially reasonable efforts to place Shares during the applicable Selling Period is subject to the satisfaction, on each Trading Day during the Selling Period, of each of the following conditions:

(i) Accuracy of the Company's Representations and Warranties; Performance by the Company. The Company shall have delivered the certificate required to be delivered pursuant to Section 4(o) on or before the date on which delivery of such certificate is required pursuant to Section 4(o). The Company shall have performed, satisfied and complied with all covenants, agreements and conditions required by this

Agreement to be performed, satisfied or complied with by the Company at or prior to such date, including, but not limited to, the covenants contained in Section 4(p), Section 4(q) and Section 4(r).

(ii) No Injunction. No statute, rule, regulation, executive order, decree, ruling or injunction shall have been enacted, entered, promulgated or endorsed by any court or governmental authority of competent jurisdiction or any self-regulatory organization having authority over the matters contemplated hereby that prohibits or directly and materially adversely affects any of the transactions contemplated by this Agreement, and no proceeding shall have been commenced that may have the effect of prohibiting or materially adversely affecting any of the transactions contemplated by this Agreement.

(iii) Material Adverse Changes. Except as disclosed in the Registration Statement, the Prospectus and the Time of Sale Information, (a) in the judgment of the Agent there shall not have occurred any Material Adverse Change; and (b) there shall not have occurred any downgrading, nor shall any notice have been given of any intended or potential downgrading or of any review for a possible change that does not indicate the direction of the possible change, in the rating accorded any securities of the Company or any of its subsidiaries by any “nationally recognized statistical rating organization” as such term is defined for purposes of Section 3(a)(62) of the Exchange Act.

(iv) No Suspension of Trading in or Delisting of Common Shares; Other Events. The trading of the Common Shares (including without limitation the Shares) shall not have been suspended by the Commission, the Principal Market or FINRA and the Common Shares (including without limitation the Shares) shall have been approved for listing or quotation on and shall not have been delisted from the Principal Market. There shall not have occurred (and be continuing in the case of occurrences under clauses (i), (ii) and (iii) below) any of the following: (i) trading generally shall have been suspended or materially limited on or by any of the New York Stock Exchange or the Principal Market; (ii) trading of any securities issued or guaranteed by the Company shall have been suspended on any exchange or in any over-the-counter market; (iii) a general moratorium on commercial banking activities shall have been declared by federal or New York State authorities; or (iv) there shall have occurred any outbreak or escalation of hostilities or any change in financial markets or any calamity or crisis, either within or outside the United States, that, in the judgment of the Agent is material and adverse and makes it impracticable or inadvisable to market the Shares in the manner and on the terms contemplated by this Agreement and described in the Prospectus.

(b) Documents Required to be Delivered on each Issuance Notice Date. The Agent’s obligation to use its commercially reasonable efforts to place Shares hereunder shall additionally be conditioned upon the delivery to the Agent on or before the Issuance Notice Date of:

(i) a certificate in form and substance reasonably satisfactory to the Agent, executed by the Chief Executive Officer, President or Chief Financial Officer of the Company, to the effect that all conditions to the delivery of such Issuance Notice shall have been satisfied as at the date of such certificate as required to be delivered pursuant to

Section 4(s) (which certificate shall not be required if the foregoing representations shall be set forth in the Issuance Notice);

(ii) a negative assurances letter and the written legal opinion of counsel to the Company required to be delivered pursuant to Section 4(p);

(iii) the written legal opinion of intellectual property counsel to the Company required to be delivered pursuant to Section 4(p);

(iv) a negative assurances letter and the written legal opinion of counsel to the Agent, such opinion or opinions to be delivered on or before the date on which the delivery of the opinion by counsel to the Company is required pursuant to Section 4(p), with respect to such matters as Agent may reasonably require, and the Company shall have furnished to such counsel such documents as they request for enabling them to pass upon such matters;

(v) the comfort letter required to be delivered pursuant to Section 4(q); and

(vi) the certificate of the Secretary of the Company to be delivered pursuant to Section 4(r).

(c) No Misstatement or Material Omission. Agent shall not have advised the Company that the Registration Statement, Prospectus or the Time of Sale Information, or any amendment or supplement thereto, contains an untrue statement of fact that in the Agent's reasonable opinion is material, or omits to state a fact that in the Agent's reasonable opinion is material and is required to be stated therein or is necessary to make the statements therein not misleading.

Section 6. INDEMNIFICATION AND CONTRIBUTION

(a) Indemnification of the Agent. The Company agrees to indemnify and hold harmless the Agent, its officers and employees, and each person, if any, who controls the Agent within the meaning of the Securities Act or the Exchange Act against any loss, claim, damage, liability or expense, as incurred, to which the Agent or such officer, employee or controlling person may become subject, under the Securities Act, the Exchange Act, other federal or state statutory law or regulation, or the laws or regulations of foreign jurisdictions where Shares have been offered or sold or at common law or otherwise (including in settlement of any litigation), insofar as such loss, claim, damage, liability or expense (or actions in respect thereof as contemplated below) arises out of or is based upon (i) any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement, or any amendment thereto, including any information deemed to be a part thereof pursuant to Rule 430B under the Securities Act, or the omission or alleged omission therefrom of a material fact required to be stated therein or necessary to make the statements therein not misleading; or (ii) any untrue statement or alleged untrue statement of a material fact contained in any Free Writing Prospectus that the Company has used, referred to or filed, or is required to file, pursuant to Rule 433(d) of the Securities Act or the Prospectus (or any amendment or supplement thereto), or the omission or alleged omission therefrom of a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; and to reimburse the Agent and each such officer, employee and controlling person for any and all expenses (including the fees and disbursements of counsel chosen by the Agent) as such expenses are reasonably incurred by the Agent or such

officer, employee or controlling person in connection with investigating, defending, settling, compromising or paying any such loss, claim, damage, liability, expense or action; provided, however, that the foregoing indemnity agreement shall not apply to any loss, claim, damage, liability or expense to the extent, but only to the extent, arising out of or based upon any untrue statement or alleged untrue statement or omission or alleged omission made in reliance upon and in conformity with written information furnished to the Company by the Agent expressly for use in the Registration Statement, any such Free Writing Prospectus or the Prospectus (or any amendment or supplement thereto), it being understood and agreed that the only such information furnished by the Agent to the Company consists of: (i) the Agent's name. The indemnity agreement set forth in this Section 6(a) shall be in addition to any liabilities that the Company may otherwise have.

(b) Notifications and Other Indemnification Procedures. Promptly after receipt by an indemnified party under this Section 6 of notice of the commencement of any action, such indemnified party will, if a claim in respect thereof is to be made against an indemnifying party under this Section 6, notify the indemnifying party in writing of the commencement thereof, but the omission so to notify the indemnifying party will not relieve it from any liability which it may have to any indemnified party for contribution or otherwise than under the indemnity agreement contained in this Section 6 or to the extent it is not prejudiced as a proximate result of such failure. In case any such action is brought against any indemnified party and such indemnified party seeks or intends to seek indemnity from an indemnifying party, the indemnifying party will be entitled to participate in, and, to the extent that it shall elect, jointly with all other indemnifying parties similarly notified, by written notice delivered to the indemnified party promptly after receiving the aforesaid notice from such indemnified party, to assume the defense thereof with counsel reasonably satisfactory to such indemnified party; provided, however, if the defendants in any such action include both the indemnified party and the indemnifying party and the indemnified party shall have reasonably concluded that a conflict may arise between the positions of the indemnifying party and the indemnified party in conducting the defense of any such action or that there may be legal defenses available to it and/or other indemnified parties which are different from or additional to those available to the indemnifying party, the indemnified party or parties shall have the right to select separate counsel to assume such legal defenses and to otherwise participate in the defense of such action on behalf of such indemnified party or parties. Upon receipt of notice from the indemnifying party to such indemnified party of such indemnifying party's election so to assume the defense of such action and approval by the indemnified party of counsel, the indemnifying party will not be liable to such indemnified party under this Section 6 for any legal or other expenses subsequently incurred by such indemnified party in connection with the defense thereof unless (i) the indemnified party shall have employed separate counsel in accordance with the proviso to the preceding sentence (it being understood, however, that the indemnifying party shall not be liable for the fees and expenses of more than one separate counsel (together with local counsel), representing the indemnified parties who are parties to such action), which counsel (together with any local counsel) for the indemnified parties shall be selected by the Agent (in the case of counsel for the indemnified parties referred to in Section 6(a) above), (ii) the indemnifying party shall not have employed counsel satisfactory to the indemnified party to represent the indemnified party within a reasonable time after notice of commencement of the action or (iii) the indemnifying party has authorized in writing the employment of counsel for the indemnified party at the expense of the indemnifying party, in each of which cases the fees and

expenses of counsel shall be at the expense of the indemnifying party and shall be paid as they are incurred.

(c) Settlements. The indemnifying party under this Section 6 shall not be liable for any settlement of any proceeding effected without its written consent, but if settled with such consent or if there be a final judgment for the plaintiff, the indemnifying party agrees to indemnify the indemnified party against any loss, claim, damage, liability or expense by reason of such settlement or judgment. Notwithstanding the foregoing sentence, if at any time an indemnified party shall have requested an indemnifying party to reimburse the indemnified party for fees and expenses of counsel as contemplated by Section 6(b) hereof, the indemnifying party agrees that it shall be liable for any settlement of any proceeding effected without its written consent if (i) such settlement is entered into more than 45 days after receipt by such indemnifying party of the aforesaid request and (ii) such indemnifying party shall not have reimbursed the indemnified party in accordance with such request prior to the date of such settlement. No indemnifying party shall, without the prior written consent of the indemnified party, effect any settlement, compromise or consent to the entry of judgment in any pending or threatened action, suit or proceeding in respect of which any indemnified party is or could have been a party and indemnity was or could have been sought hereunder by such indemnified party, unless such settlement, compromise or consent includes an unconditional release of such indemnified party from all liability on claims that are the subject matter of such action, suit or proceeding.

(d) Contribution. If the indemnification provided for in this Section 6 is for any reason held to be unavailable to or otherwise insufficient to hold harmless an indemnified party in respect of any losses, claims, damages, liabilities or expenses referred to therein, then each indemnifying party shall contribute to the aggregate amount paid or payable by such indemnified party, as incurred, as a result of any losses, claims, damages, liabilities or expenses referred to therein (i) in such proportion as is appropriate to reflect the relative benefits received by the Company, on the one hand, and the Agent, on the other hand, from the offering of the Shares pursuant to this Agreement or (ii) if the allocation provided by clause (i) above is not permitted by applicable law, in such proportion as is appropriate to reflect not only the relative benefits referred to in clause (i) above but also the relative fault of the Company, on the one hand, and the Agent, on the other hand, in connection with the statements or omissions which resulted in such losses, claims, damages, liabilities or expenses, as well as any other relevant equitable considerations. The relative benefits received by the Company, on the one hand, and the Agent, on the other hand, in connection with the offering of the Shares pursuant to this Agreement shall be deemed to be in the same respective proportions as the total gross proceeds from the offering of the Shares (before deducting expenses) received by the Company bear to the total commissions received by the Agent. The relative fault of the Company, on the one hand, and the Agent, on the other hand, shall be determined by reference to, among other things, whether any such untrue or alleged untrue statement of a material fact or omission or alleged omission to state a material fact relates to information supplied by the Company, on the one hand, or the Agent, on the other hand, and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

The amount paid or payable by a party as a result of the losses, claims, damages, liabilities and expenses referred to above shall be deemed to include, subject to the limitations set forth in Section 6(b), any legal or other fees or expenses reasonably incurred by such party in connection

with investigating or defending any action or claim. The provisions set forth in Section 6(b) with respect to notice of commencement of any action shall apply if a claim for contribution is to be made under this Section 6(d); *provided, however*, that no additional notice shall be required with respect to any action for which notice has been given under Section 6(b) for purposes of indemnification.

The Company and the Agent agree that it would not be just and equitable if contribution pursuant to this Section 6(d) were determined by pro rata allocation or by any other method of allocation which does not take account of the equitable considerations referred to in this Section 6(d).

Notwithstanding the provisions of this Section 6(d), the Agent shall not be required to contribute any amount in excess of the agent fees received by the Agent in connection with the offering contemplated hereby. No person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any person who was not guilty of such fraudulent misrepresentation. For purposes of this Section 6(d), each officer and employee of the Agent and each person, if any, who controls the Agent within the meaning of the Securities Act or the Exchange Act shall have the same rights to contribution as the Agent, and each director of the Company, each officer of the Company who signed the Registration Statement, and each person, if any, who controls the Company with the meaning of the Securities Act and the Exchange Act shall have the same rights to contribution as the Company.

Section 7. TERMINATION & SURVIVAL

(a) Term. Subject to the provisions of this Section 7, the term of this Agreement shall continue from the date of this Agreement until the end of the Agency Period, unless earlier terminated by the parties to this Agreement pursuant to this Section 7.

(b) Termination; Survival Following Termination. (i) Either party may terminate this Agreement prior to the end of the Agency Period, by giving written notice as required by this Agreement, upon five Trading Days' notice to the other party; provided that, (A) if the Company terminates this Agreement after the Agent confirms to the Company any sale of Shares, the Company shall remain obligated to comply with Section 3(b)(v) with respect to such Shares and (B) Section 2, Section 6, Section 7 and Section 8 shall survive termination of this Agreement. If termination shall occur prior to the Settlement Date for any sale of Shares, such sale shall nevertheless settle in accordance with the terms of this Agreement. Upon termination of this Agreement, the Company shall not have any liability to the Agent for any discount, commission or other compensation with respect to any Shares not otherwise sold by the Agent under this Agreement.

(ii) In addition to the survival provision of Section 7(b)(i), the respective indemnities, agreements, representations, warranties and other statements of the Company, of its officers and of the Agent set forth in or made pursuant to this Agreement will remain in full force and effect, regardless of any investigation made by or on behalf of the Agent or the Company or any of its or their partners, officers or directors or any controlling person, as the case may be, and, anything herein to the contrary notwithstanding, will survive delivery of and payment for the Shares sold hereunder and any termination of this Agreement.

Section 8. MISCELLANEOUS

(a) Press Releases and Disclosure. The Company may issue a press release describing the material terms of the transactions contemplated hereby as soon as practicable following the date of this Agreement, and may file with the Commission a Current Report on Form 8-K, with this Agreement attached as an exhibit thereto, describing the material terms of the transactions contemplated hereby, and the Company shall consult with the Agent prior to making such disclosures, and the parties hereto shall use all commercially reasonable efforts, acting in good faith, to agree upon a text for such disclosures that is reasonably satisfactory to all parties hereto. Notwithstanding anything to the contrary herein, the Company shall not issue any press release or make any other public disclosure or public statement (other than disclosure, including related to periodic sales pursuant to this Agreement, the Company believes is required to be included in any reports filed with the Commission pursuant to the Securities Act or Exchange Act) related to this Agreement, or any of the transactions contemplated hereby or naming the Agent without the Agent's prior written approval.

(b) No Advisory or Fiduciary Relationship. The Company acknowledges and agrees that (i) the transactions contemplated by this Agreement, including the determination of any fees, are arm's-length commercial transactions between the Company and the Agent, (ii) when acting as a principal under this Agreement, the Agent is and has been acting solely as a principal is not the agent or fiduciary of the Company, or its stockholders, creditors, employees or any other party, (iii) the Agent has not assumed nor will assume an advisory or fiduciary responsibility in favor of the Company with respect to the transactions contemplated hereby or the process leading thereto (irrespective of whether the Agent has advised or is currently advising the Company on other matters) and the Agent does not have any obligation to the Company with respect to the transactions contemplated hereby except the obligations expressly set forth in this Agreement, (iv) the Agent and its respective affiliates may be engaged in a broad range of transactions that involve interests that differ from those of the Company, and (v) the Agent has not provided any legal, accounting, regulatory or tax advice with respect to the transactions contemplated hereby and the Company has consulted its own legal, accounting, regulatory and tax advisors to the extent it deemed appropriate.

(c) Research Analyst Independence. The Company acknowledges that the Agent's research analysts and research departments are required to and should be independent from their respective investment banking divisions and are subject to certain regulations and internal policies, and as such the Agent's research analysts may hold views and make statements or investment recommendations and/or publish research reports with respect to the Company or the offering that differ from the views of their respective investment banking divisions. The Company understands that the Agent is a full service securities firm and as such from time to time, subject to applicable securities laws, may effect transactions for its own account or the account of its customers and hold long or short positions in debt or equity securities of the companies that may be the subject of the transactions contemplated by this Agreement.

(d) Notices. All communications hereunder shall be in writing and shall be mailed, hand delivered or telecopied and confirmed to the parties hereto as follows:

If to the Agent:

Jefferies LLC
520 Madison Avenue
New York, NY 10022
Attention: General Counsel

with a copy to:

Davis Polk & Wardwell LLP
450 Lexington Avenue
New York, NY 10017
Facsimile: (212) 701-5135
Attention: Deanna Kirkpatrick, Esq.

If to the Company:

Solid Biosciences Inc.
141 Portland Street, Fifth Floor
Cambridge, MA 02139
Attention: Chief Legal Officer

with a copy to:

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Lia Der Marderosian.

Any party hereto may change the address for receipt of communications by giving written notice to the others in accordance with this Section 8(d).

(e) Successors. This Agreement will inure to the benefit of and be binding upon the parties hereto, and to the benefit of the employees, officers and directors and controlling persons referred to in Section 6, and in each case their respective successors, and no other person will have any right or obligation hereunder. The term “**successors**” shall not include any purchaser of the Shares as such from the Agent merely by reason of such purchase.

(f) Partial Unenforceability. The invalidity or unenforceability of any Article, Section, paragraph or provision of this Agreement shall not affect the validity or enforceability of any other Article, Section, paragraph or provision hereof. If any Article, Section, paragraph or provision of this Agreement is for any reason determined to be invalid or unenforceable, there shall be deemed to be made such minor changes (and only such minor changes) as are necessary to make it valid and enforceable.

(g) Governing Law Provisions. This Agreement, and any claim, controversy or dispute of any kind or nature whatsoever arising out of or in any way relating to this Agreement, shall be governed by and construed in accordance with the internal laws of the State of New York

applicable to agreements made and to be performed in such state. Any legal suit, action or proceeding arising out of or based upon this Agreement or the transactions contemplated hereby (“**Related Proceedings**”) may be instituted in the federal courts of the United States of America located in the Borough of Manhattan in the City of New York or the courts of the State of New York in each case located in the Borough of Manhattan in the City of New York (collectively, the “**Specified Courts**”), and each party irrevocably submits to the exclusive jurisdiction (except for proceedings instituted in regard to the enforcement of a judgment of any such court (a “**Related Judgment**”), as to which such jurisdiction is non-exclusive) of such courts in any such suit, action or proceeding. Service of any process, summons, notice or document by mail to such party’s address set forth above shall be effective service of process for any suit, action or other proceeding brought in any such court. The parties irrevocably and unconditionally waive any objection to the laying of venue of any suit, action or other proceeding in the Specified Courts and irrevocably and unconditionally waive and agree not to plead or claim in any such court that any such suit, action or other proceeding brought in any such court has been brought in an inconvenient forum.

(h) General Provisions. This Agreement constitutes the entire agreement of the parties to this Agreement and supersedes all prior written or oral and all contemporaneous oral agreements, understandings and negotiations with respect to the subject matter hereof. This Agreement may be executed in two or more counterparts, each one of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument, and may be delivered by facsimile transmission or by electronic delivery of a portable document format (PDF) file. This Agreement may not be amended or modified unless in writing by all of the parties hereto, and no condition herein (express or implied) may be waived unless waived in writing by each party whom the condition is meant to benefit. The Article and Section headings herein are for the convenience of the parties only and shall not affect the construction or interpretation of this Agreement.

[Signature Page Immediately Follows]

If the foregoing is in accordance with your understanding of our agreement, kindly sign and return to the Company the enclosed copies hereof, whereupon this instrument, along with all counterparts hereof, shall become a binding agreement in accordance with its terms

Very truly yours,

SOLID BIOSCIENCES INC.

By:

/s/

Ilan
Ganot

Name: Ilan Ganot
Title: Chief Executive

Officer

The foregoing Agreement is hereby confirmed and accepted by the Agent in New York, New York as of the date first above written.

JEFFERIES LLC

By: /s/ Michael Magarro

Name: Michael Magarro

Title: Managing Director

EXHIBIT A
ISSUANCE NOTICE

[Date]

Jefferies LLC
520 Madison Avenue
New York, New York 10022

Attn: [_____]

Reference is made to the Sales Agreement between Solid Biosciences Inc. (the “**Company**”) and Jefferies LLC (the “**Agent**”) dated as of March 13, 2019. The Company confirms that all conditions to the delivery of this Issuance Notice are satisfied as of the date hereof.

Date of Delivery of Issuance Notice (determined pursuant to Section 3(b)(i)): _____

Issuance Amount (equal to the total Sales Price for such Shares):

\$

Number of Days in Selling Period:

First Date of Selling Period:

Last Date of Selling Period:

Settlement Date(s) if other than standard T+2 settlement:

Floor Price Limitation (in no event less than \$1.00 without the prior written consent of the Agent, which consent may be withheld in the Agent’s sole discretion): \$ ____ per share

Comments:

SOLID BIOSCIENCES INC.

By:

Name:
Title:

EXHIBIT B

Form of Opinion of Wilmer Cutler Pickering Hale and Dorr LLP, Counsel for the Company

B-1

EXHIBIT C

Form of Opinion of Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C., Intellectual Property Counsel for the Company

C-1

AMENDMENT NO. 1 TO THE SALES AGREEMENT

August 16, 2021

JEFFERIES LLC
520 Madison Avenue
New York, New York 10022

Ladies and Gentlemen:

This Amendment No. 1 to the Sales Agreement (this “**Amendment**”) is entered into as of the date first written above by Solid Biosciences Inc., a Delaware corporation (the “**Company**”), and Jefferies LLC (“**Agent**”), that are parties to that certain Sales Agreement, dated March 13, 2019 (the “**Original Agreement**”) relating to the offering of up to \$50,000,000 of the Company’s Common Shares (as defined below).

This Amendment, among other items, increases the aggregate offering price to \$75,000,000 and amends the Original Agreement in connection with the New Registration Statement (as defined below). All capitalized terms not defined herein shall have the meanings ascribed to them in the Original Agreement. The parties, intending to be legally bound, hereby amend the Original Agreement as follows:

1. The preamble to the Original Agreement is hereby deleted in its entirety and replaced with the following:

“Solid Biosciences Inc., a Delaware corporation (the “**Company**”), proposes, subject to the terms and conditions stated herein, to issue and sell from time to time through Jefferies LLC, as sales agent and/or principal (the “**Agent**”), shares of the Company’s common stock, par value \$0.001 per share (the “**Common Shares**”), having an aggregate offering price of up to \$75,000,000 on the terms set forth in this agreement (this “**Agreement**”).”

2. Section 1(a) of the Original Agreement is hereby amended by deleting in its entirety the definition of “Agency Period” and replacing it with the following:

““**Agency Period**” means the period commencing on the date of this Agreement and expiring on the earliest to occur of (x) the date on which the Agent shall have placed the Maximum Program Amount pursuant to this Agreement, (y) the date this Agreement is terminated pursuant to Section 7 and (z) the third anniversary of August 16, 2021.”

3. Section 1(a) of the Original Agreement is hereby further amended by deleting in its entirety the definition of “Maximum Program Amount” and replacing it with the following:

““**Maximum Program Amount**” means Common Shares with an aggregate Sales Price of the lesser of (1) \$75,000,000 and (2) the aggregate amount of Shares registered under the Registration Statement.”

4. Section 4(p) of the Original Agreement is hereby amended by replacing references to “Mintz, Levin, Cohn, Ferris, Glovsky and Popeo, P.C.” with “McCarter & English, LLP.”

5. The title of Exhibit C of the Original Agreement is hereby amended by deleting in its entirety and replacing it with the following:

“Form of Opinion of McCarter & English, LLP, Intellectual Property Counsel for the Company”

6. The Company represents and warrants to, and agrees with the Agent that: (a) this Amendment has been duly authorized, executed and delivered by, and is a valid and binding agreement of, the Company, enforceable in accordance with its terms, except as rights to indemnification hereunder may be limited by applicable law and except as the enforcement hereof may be limited by bankruptcy, insolvency, reorganization, moratorium or other similar laws relating to or affecting the rights and remedies of creditors or by general equitable principles; (b) on the date hereof, the Company will file a registration statement on Form S-3 (the “**New Registration Statement**”) with the Commission; and (c) the first delivery of an Issuance Notice after the effectiveness of the New Registration Statement will constitute a Triggering Event Date.

7. The increase in the amount of Common Shares that may be sold under the Original Agreement pursuant to Section 1 of this Amendment shall be effective at such time that the Commission declares the New Registration Statement effective. For the avoidance of doubt, any reference to “Original Registration Statement” and “Registration Statement” in the Original Agreement shall be deemed to include the New Registration Statement.

8. The Original Agreement, as amended by this Amendment, constitutes the entire agreement of the parties hereto and supersedes all prior written or oral and all contemporaneous oral agreements, understandings and negotiations with respect to the subject matter hereof. The invalidity or unenforceability of any section, paragraph or provision of this Amendment shall not affect the validity or enforceability of any other section, paragraph or provision hereof. If any section, paragraph or provision of this Amendment is for any reason determined to be invalid or unenforceable, there shall be deemed to be made such minor changes (and only such minor changes) as are necessary to make it valid and enforceable. All references in the Original Agreement to the “Agreement” shall mean the Original Agreement as amended by this Amendment; *provided, however*, that all references to “date of this Agreement” in the Original Agreement shall continue to refer to the date of the Original Agreement.

9. This Amendment, and any claim, controversy or dispute of any kind or nature whatsoever arising out of or in any way relating to this Amendment, shall be governed by and construed in accordance with the internal laws of the State of New York applicable to agreements made and to be performed in such state. Any legal suit, action or proceeding arising out of or based upon this Amendment or the transactions contemplated hereby may be instituted in the federal courts of the United States of America located in the Borough of Manhattan in the City of New York or the courts of the State of New York in each case located in the Borough of Manhattan in the City of New York (collectively, the “**Specified Courts**”), and each party irrevocably submits to the exclusive jurisdiction (except for proceedings instituted in regard to the enforcement of a judgment of any such court, as to which such jurisdiction is non-exclusive) of such courts in any such suit, action or proceeding. Service of any process, summons, notice or document by mail to such party’s address set forth in the Original Agreement shall be effective service of process for any suit, action or other proceeding brought in any such court. The parties irrevocably and unconditionally waive any objection to the laying of venue of any suit, action or other proceeding in the Specified Courts and irrevocably and unconditionally waive and agree not to plead or claim in any such court that any such suit, action or other proceeding brought in any such court has been brought in an inconvenient forum.

10. This Amendment may be executed in two or more counterparts, each one of which shall be an original, with the same effect as if the signatures thereto and hereto were upon the same instrument, and may be delivered by facsimile transmission or by electronic delivery of a portable document format (PDF) file. This Amendment may not be amended or modified unless in writing by all of the parties hereto, and no condition herein (express or implied) may be waived unless waived in writing by each party whom the condition is meant to benefit.

[Remainder of Page Intentionally Blank]

If the foregoing correctly sets forth the understanding between the Company and the Agent, please so indicate in the space provided below for that purpose, whereupon this letter shall constitute a binding amendment to the Original Agreement between the Company and the Agent.

Very truly yours,

JEFFERIES LLC

By: /s/ Michael Magarro

Name: Michael Magarro

Title: Managing Director

ACCEPTED as of the date first-above written:

SOLID BIOSCIENCES INC.

By: /s/ Ilan Ganot

Name: Ilan Ganot

Title: President and Chief Executive Officer

[Signature Page to Amendment No. 1 to the Sales Agreement]

**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, Ilan Ganot, 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/ Ilan Ganot

Ilan Ganot

President and Chief Executive Officer

(Principal Executive Officer)

Dated: November 3, 2021

**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, Stephen DiPalma, 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/ Stephen DiPalma

Stephen DiPalma

Interim Chief Financial Officer

(Principal Financial and Accounting Officer)

Dated: November 3, 2021

**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, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Ilan Ganot, 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 3, 2021

/s/ Ilan Ganot

Ilan Ganot
President and Chief Executive Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

**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, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Stephen DiPalma, Interim 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 3, 2021

/s/ Stephen DiPalma

Stephen DiPalma

Interim Chief Financial Officer

(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.