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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 June 30, 2018

OR

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

For the transition period from _____ to _____

Commission File Number: 001-38360

Solid Biosciences Inc.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**90-0943402**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
141 Portland Street, Fifth Floor Cambridge, MA	**02139**
(Address of principal executive offices)	**(Zip Code)**

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

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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted 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 and post 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	☐	Accelerated filer	☐
Non-accelerated filer	☒ (Do not check if a smaller reporting company)	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 August 1, 2018, the registrant had 35,432,460 shares of common stock, $0.001 par value per share, outstanding.

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PART I—FINANCIAL INFORMATION

Item 1. Condensed Consolidated Financial Statements (unaudited)

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

	June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 145,824	$ 52,080
Available-for-sale-securities	16,935	17,014
Prepaid expenses and other current assets	1,714	1,499
Restricted cash	—	65
Total current assets	164,473	70,658
Property and equipment, net	6,551	2,429
Other non-current assets	209	—
Restricted cash	237	—
Deferred offering costs	—	3,106
Total assets	$ 171,470	$ 76,193
Liabilities, Preferred Units and Stockholders' / Members' Equity / (Deficit)		
Current liabilities:		
Accounts payable	$ 3,741	$ 5,066
Accrued expenses and other current liabilities	4,938	6,205
Total current liabilities	8,679	11,271
Other non-current liabilities	499	—
Total liabilities	9,178	11,271
Commitments and contingencies (Note 10)		
Series 2 Senior Preferred Units, no units authorized at June 30, 2018 and 4,886,000 units authorized at December 31, 2017; no units issued and outstanding at June 30, 2018 and 4,886,000 units issued and outstanding at December 31, 2017	—	55,002
Series 1 Senior Preferred Units, no units authorized at June 30, 2018 and 2,500,000 units authorized at December 31, 2017; no units issued and outstanding at June 30, 2018 and 2,500,000 units issued and outstanding at December 31, 2017	—	25,000
Junior Preferred Units, no units authorized at June 30, 2018 and 4,414,356 units authorized at December 31, 2017; no units issued and outstanding at June 30, 2018 and 4,414,356 units issued and outstanding at December 31, 2017	—	44,177
Stockholders' / Members' equity / (deficit):		
Series A, B, C and D Common Units, no units authorized at June 30, 2018 and 20,189,509 units authorized at December 31, 2017; no units issued and outstanding at June 30, 2018 and 19,438,552 units issued and outstanding at December 31, 2017	—	65,014
Preferred stock, $0.001 par value; 10,000,000 shares authorized at June 30, 2018 and no shares authorized at December 31, 2017; no shares issued and outstanding at June 30, 2018 and December 31, 2017	—	—
Common stock, $0.001 par value; 300,000,000 shares authorized at June 30, 2018 and no shares authorized at December 31, 2017; 35,444,792 shares issued and outstanding at June 30, 2018 and no shares issued and outstanding at December 31, 2017	35	—
Additional paid-in capital	320,382	—
Accumulated other comprehensive loss	(10)	(13)
Accumulated deficit	(158,115)	(124,258)
Total stockholders'/members' equity/(deficit)	162,292	(59,257)
Total liabilities, preferred units and stockholders'/members' equity	$ 171,470	$ 76,193

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	13,594	8,607	25,523	17,340
General and administrative	4,584	3,280	8,628	8,660
Total operating expenses	18,178	11,887	34,151	26,000
Loss from operations	(18,178)	(11,887)	(34,151)	(26,000)
Other income (expense):				
Revaluation of preferred unit tranche rights	—	20	—	20
Interest income	80	52	145	114
Other income	118	504	149	680
Total other income (expense), net	198	576	294	814
Net loss	(17,980)	(11,311)	(33,857)	(25,186)
Net loss attributable to non-controlling interest	—	—	—	(1,060)
Net loss attributable to Solid Biosciences Inc.	(17,980)	(11,311)	(33,857)	(24,126)
Accretion of preferred units to redemption value	—	—	—	(959)
Redemption of preferred units	—	—	—	15,685
Redemption of redeemable interest from non-controlling interest in Solid GT	—	—	—	(1,925)
Net loss attributable to common stockholders	$ (17,980)	$ (11,311)	$ (33,857)	$ (11,325)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.52)	$ (0.66)	$ (1.06)	$ (1.12)
Weighted average shares of common stock outstanding, basic and diluted	34,449,758	17,041,311	31,916,295	10,083,502

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Net loss	$(17,980)	$(11,311)	$(33,857)	$(25,186)
Other comprehensive loss:				
Unrealized gain (loss) on available-for-sale securities	13	(2)	3	(25)
Comprehensive loss	(17,967)	(11,313)	(33,854)	(25,211)
Comprehensive loss attributable to non-controlling interest	—	—	—	(1,060)
Comprehensive loss attributable to Solid Biosciences Inc.	$(17,967)	$(11,313)	$(33,854)	$(24,151)

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

SOLID BIOSCIENCES INC.
CONDENSED CONSOLIDATED STATEMENTS OF PREFERRED UNITS AND STOCKHOLDERS'/MEMBERS' EQUITY/(DEFICIT)
(unaudited, in thousands, except share / unit data)

	Redeemable Preferred Units	Amount	Series 2 Senior Preferred Units	Amount	Series 1 Senior Preferred Units	Amount	Junior Preferred Units	Amount	Series A, B, C and D Common Units	Amount	Common Stock	Amount	Additional paid in capital	Accumulated other comprehensive income (loss)	Accumulated Deficit	Total Members'/ Stockholders' Equity (Deficit)	Non-controlling Interest	Total Equity (Deficit)
Balance at December 31, 2016	17,100,000	$ 71,649	—	—	—	—	—	—	5,123,917	$ 558	—	—	—	$ 23	$ (84,941)	$ (84,360)	$ 46,474	$ (37,886)
Issuance of Series 1 senior preferred units, net of issuance costs of $500 and tranche right of $459	—	—	—	—	2,500,000	$ 24,041	—	—	—	—	—	—	—	—	—	—	—	—
Accretion of Series 1 senior preferred units to redemption value	—	—	—	—	—	959	—	—	—	—	—	—	—	—	(959)	(959)	—	(959)
Redemption of preferred units	—	(15,685)	—	—	—	—	—	—	—	—	—	—	—	—	15,685	15,685	—	15,685
Equity-based compensation	—	—	—	—	—	—	—	—	—	5,030	—	—	—	—	—	5,030	300	5,330
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(52,118)	(52,118)	(1,060)	(53,178)
Issuance of Series B common units in exchange for Series A common units	—	—	—	—	—	—	—	—	(1,301,520)	—	—	—	—	—	—	—	—	—
Issuance of Series D common units in exchange for Series A common units	—	—	—	—	—	—	—	—	(160,954)	—	—	—	—	—	—	—	—	—
Issuance of Series A common units in exchange for redeemable preferred units	(17,100,000)	(55,964)	—	—	—	—	—	—	12,219,299	55,964	—	—	—	—	—	55,964	—	55,964
Issuance of junior preferred units in redemption of Class D non-controlling interest in Solid GT	—	—	—	—	—	—	4,414,356	$ 44,177	—	—	—	—	—	—	(1,925)	(1,925)	(42,252)	(44,177)
Issuance of Series C common units in exchange for Class B non-controlling interest in Solid GT	—	—	—	—	—	—	—	—	1,635,916	2,053	—	—	—	—	—	2,053	(2,053)	—
Issuance of Series D common units in exchange for Class C non-controlling interest in Solid GT	—	—	—	—	—	—	—	—	1,083,205	1,409	—	—	—	—	—	1,409	(1,409)	—
Issuance of Series D common units	—	—	—	—	—	—	—	—	838,689	—	—	—	—	—	—	—	—	—
Issuance of Series 2 senior preferred units,			4,886,000	$ 55,002														
Unrealized loss on available for sale securities	—	—			—	—	—	—	—	—				(36)	—	(36)	—	(36)
Balance at December 31, 2017	—	—	4,886,000	$ 55,002	2,500,000	$ 25,000	4,414,356	$ 44,177	19,438,552	$ 65,014	—	$ —	$ —	$ (13)	$ (124,258)	$ (59,257)	—	$ (59,257)
Conversion of units into shares of common stock	—	—	(4,886,000)	(55,002)	(2,500,000)	(25,000)	(4,414,356)	(44,177)	(19,429,620)	(65,180)	26,498,559	26	189,333	—	—	124,179	—	124,179
Issuance of common stock upon initial public offering, net of issuance costs of $4,592	—	—	—	—	—	—	—	—	—	—	8,984,375	$ 9	$ 129,087	—	—	129,096	—	129,096
Equity-based compensation	—	—	—	—	—	—	—	—	—	166			1,962	—	—	2,128	—	2,128
Net loss	—	—	—	—	—	—	—	—	—	—				—	(33,857)	(33,857)	—	(33,857)
Repurchase of common units/ shares of common stock	—	—	—	—	—	—	—	—	(8,932)	—	(38,142)	—	—	—	—	—	—	—
Unrealized gain on available for sale securities	—	—	—	—	—	—	—	—	—	—	—	—	—	3	—	3	—	3
Balance at June 30, 2018	—	—	—	$ —	—	$ —	—	$ —	—	$ —	35,444,792	$ 35	$ 320,382	$ (10)	$ (158,115)	$ 162,292	$ —	$162,292

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)

	Six Months Ended June 30,	
	2018	**2017**
Cash flows from operating activities:		
Net loss	$ (33,857)	$(25,186)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of (discount)/premium on available-for-sale securities	(34)	163
Equity-based compensation expense	2,128	3,717
Depreciation expense	540	115
(Gain) / loss on sale of property and equipment	6	—
Revaluation of tranche liability	—	(20)
Changes in operating assets and liabilities:		
Prepaid expenses and other current and non-current assets	(424)	1,606
Accounts payable	(1,057)	(1,264)
Accrued expenses and other current and non-current liabilities	(805)	1,066
Net cash used in operating activities	(33,503)	(19,803)
Cash flows from investing activities:		
Purchases of property and equipment	(4,217)	(287)
Proceeds from sale and maturities of available-for-sale securities	13,440	21,428
Purchases of available-for-sale securities	(13,324)	(568)
Net cash (used in) provided by investing activities	(4,101)	20,573
Cash flows from financing activities:		
Proceeds from issuance of Series 1 Senior Preferred units	—	24,500
Proceeds from initial public offering of common stock, net of commissions and underwriting discounts	133,688	—
Payment of deferred offering costs	(2,168)	(236)
Net cash provided by financing activities	131,520	24,264
Net increase in cash, cash equivalents and restricted cash	93,916	25,034
Cash, cash equivalents, and restricted cash at beginning of period	52,145	7,843
Cash, cash equivalents, and restricted cash at end of period	$146,061	$ 32,877
Supplemental disclosure of non-cash investing and financing activities:		
Conversion of Series A, B, C and D common units into shares of common stock	$ 65,180	—
Conversion of Series 2 Senior Preferred units into shares of common stock	$ 55,002	—
Conversion of Series 1 Senior Preferred units into shares of common stock	$ 25,000	—
Conversion of Junior Preferred units into shares of common stock	$ 44,177	—
Accretion to redemption value for redeemable preferred units	—	$ (959)
Redemption of preferred units	—	$ 15,685
Redemption of redeemable interest from non-controlling interest in Solid GT	—	$ (1,925)
Deferred offering costs included in accounts payable and accruals	—	$ 655
Property and equipment included in accounts payable and accruals	$ 715	319
Issuance of Series D common units in exchange for Series A common units	$ —	638
Issuance of Series A common units in exchange for Redeemable preferred units	$ —	55,964
Issuance of Junior preferred units upon redemption of Class D non-controlling interest in Solid GT	$ —	44,177
Issuance of Series C common units in exchange for Class B non-controlling interest in Solid GT	—	$ 2,053
Issuance of Series D common units in exchange for Class C non-controlling interest in Solid GT	—	$ 1,409

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

SOLID BIOSCIENCES INC.
NOTES TO CONDENSED CONSOLIDATED FINANCIAL STATEMENTS
(Amounts in thousands, except share / unit and per share / unit 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. In October 2013, the Company changed its name to Solid Ventures, LLC and in June 2015, the Company changed its name to Solid Biosciences, LLC.

The Company operated as a Delaware limited liability company under the name Solid Biosciences, LLC 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 (the "Corporate Conversion") and changed its name to Solid Biosciences Inc. (the "Company"). As a result of the Corporate Conversion, all of the Series 1 and 2 Senior Preferred Units, Junior Preferred Units, Series A, B, C and D Common Units of Solid Biosciences, LLC converted into shares of common stock of Solid Biosciences Inc. on a one-for-0.8485 basis and all of the unit holders of Solid Biosciences, LLC became holders of common stock of Solid Biosciences Inc.

The Company's mission is to cure Duchenne muscular dystrophy ("DMD"), 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 under development to restore functional dystrophin protein expression in patients' muscles. SGT-001 has been granted Rare Pediatric Disease 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, or 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 called IGNITE DMD. In November 2017, the FDA notified the Company that it was not permitted to dose patients in the higher-dose group of IGNITE DMD due to a partial clinical hold. The partial clinical hold related to the number of vials and manufacturing lots utilized per patient, as well as manufacturing processes to support the higher-dose group. The Company has since submitted a response to the FDA and was notified that the partial clinical hold has been resolved.

In March 2018, the Company announced that IGNITE DMD was placed on full clinical hold following an unexpected serious adverse event reported in the first patient dosed in the clinical trial. In its clinical hold letter, the FDA requested additional information, including an assessment of the etiology of the event, the patient's clinical status and laboratory parameters, and any additional measures to address patient safety. The Company submitted a comprehensive response to the FDA that included the requested information, and in June 2018, the FDA lifted the clinical hold. The Company has since resumed screening patients for IGNITE DMD.

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 pre-clinical 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.

Initial Public Offering in January 2018

On January 30, 2018, the Company completed its initial public offering with the sale of 8,984,375 shares of common stock, including shares of common stock issued upon the exercise in full of the underwriters' over-allotment option, at a public offering price of $16.00 per share, resulting in net proceeds of $129,096, after deducting underwriting discounts and commissions and offering expenses.

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 June 30, 2018, the Company has funded its operations primarily with the proceeds from the private placements of preferred units and member units and its recently completed initial public offering. The Company has incurred recurring losses from operations since its inception, including a net loss of $17,980 and $11,311 for the three months ended June 30, 2018 and 2017, respectively, and $33,857 and $25,186 for the six months ended June 30, 2018 and 2017, respectively. In addition, as of June 30, 2018, the Company had an accumulated deficit of $158,115. The Company expects to continue to generate operating losses for the foreseeable future.

As of June 30, 2018, the Company had cash, cash equivalents and available-for-sale securities of $162,759. The Company believes that its cash, cash equivalents and available-for-sale securities as of June 30, 2018 will enable it to fund its operating expenses and capital expenditure requirements through at least the next 12 months from the issuance of these financial statements.

To execute its business plans, the Company will need substantial funding to support its continuing operations and pursue its growth strategy. Until the Company can generate significant revenue from product sales, if ever, it expects to finance its operations through the sale of public or private equity, debt financings or other capital sources, potentially including collaborations with other companies or other strategic transactions. The Company may not be able to obtain financing on acceptable terms, or at all. Even if the Company is able to secure financing, the terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain funding, the Company could be forced to delay, reduce or eliminate some or all of its research and development programs, pre-clinical and clinical testing or commercialization efforts, which could adversely affect its business prospects. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient financing on terms acceptable to the Company to fund continuing operations, if at all.

Merger and Recapitalization in March 2017

The Company had historically owned 100% of the voting units of its wholly owned subsidiary, Solid GT, LLC ("Solid GT"), and the results of Solid GT were included in the Company's condensed consolidated financial statements. In November 2015, Solid GT issued voting units to new investors which decreased the Company's voting ownership in Solid GT to 77%. The Company continued to consolidate the results of Solid GT into its financial statements as the Company owned a majority voting interest in Solid GT and directed the activities of Solid GT. However, because the Company controlled but owned less than 100% of Solid GT, the Company recorded a non-controlling ownership interest at its fair value at inception and recognizes the net loss or profit attributable to non-controlling interests in the condensed consolidated statements of operations based on a profit and loss sharing arrangement between the Company and the non-controlling interests. The Company also presented the change in equity related to equity-based compensation issued to Solid GT employees by Solid GT in non-controlling interest.

On March 29, 2017, the Company merged the operations of Solid GT into the Company and Solid GT ceased to exist as a legal entity. The proportionate share of the loss attributed to the non-controlling interest amounted to $0 and $1,060 for the three and six months ended June 30, 2017, respectively. There was no loss attributed to the non-controlling interest for the three and six months ended June 30, 2018 since the merger was completed on March 29, 2017. See Note 3, *Merger and Recapitalization*, for additional information.

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 the Company 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 (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, 2017. 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 six months ended June 30, 2018 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

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

Cash Equivalents

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

Restricted Cash

The Company held restricted cash of $237 in a separate restricted bank account as a security deposit for the lease of the Company's facilities as of June 30, 2018. The Company has included restricted cash of $237 as a non-current asset as of June 30, 2018.

The Company held restricted cash of $65 in a separate restricted bank account as a security deposit for the lease of the Company's facility as of December 31, 2017. The Company has included the restricted cash of $65 as a current asset as of December 31, 2017.

Available-for-Sale Securities

Available-for-sale securities consist of investments with original maturities greater than 90 days at acquisition date. The Company has classified its investments with maturities beyond one year as short term, based on their highly liquid nature and because such available-for-sale securities represent the investment of cash that is available for current operations.

The Company classifies all of its investments as available-for-sale securities. The Company's investments are measured and reported at fair value using quoted prices in active markets for similar securities. Unrealized gains and losses on available-for-sale debt securities are reported as a separate component of stockholders' equity. The cost of securities sold is determined on a specific identification basis, and realized gains and losses are included in other income (expense) within the condensed consolidated statement of operations. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the condensed consolidated statement of operations. No such adjustments were necessary during the periods presented.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. Periodically, the Company maintains deposits in accredited financial institutions in excess of federally insured limits. The Company maintains each of its cash balances with high-quality and accredited financial institutions and accordingly, such funds are not exposed to significant credit risk. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

The Company is dependent on third-party manufacturers to supply products for research and development activities of its programs, including clinical and pre-clinical testing. These programs could be adversely affected by a significant interruption in the supply of such drug substance products.

Fair Value Measurements

Certain assets and liabilities are carried at fair value under GAAP. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. Financial assets and liabilities carried at fair value are to be classified and disclosed in one of the following three levels of the fair value hierarchy, of which the first two are considered observable and the last is considered unobservable:

- Level 1—Quoted prices in active markets for identical assets or liabilities.
- Level 2—Observable inputs (other than Level 1 quoted prices), such as quoted prices in active markets for similar assets or liabilities, quoted prices in markets that are not active for identical or similar assets or liabilities or other inputs that are observable or can be corroborated by observable market data.
- Level 3—Unobservable inputs that are supported by little or no market activity and that are significant to determining the fair value of the assets or liabilities, including pricing models, discounted cash flow methodologies and similar techniques.

The Company's cash equivalents and available-for-sale securities are carried at fair value, determined according to the fair value hierarchy described above. See Note 4, *Fair Value of Financial Assets and Liabilities*, for additional information. The carrying values of the Company's accounts payable and accrued expenses and other current liabilities approximate their fair value due to the short-term nature of these liabilities.

Equity-Based Compensation

In connection with the completion of the Company's initial public offering, the Company adopted the 2018 Omnibus Incentive Plan, which provides for the issuance of share-based awards, including options to purchase common stock. The 2018 Omnibus Incentive Plan provides for the awarding of up to 5,001,000 shares of common stock for equity awards.

The Company measures all stock options and other stock-based awards granted to employees and directors based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company applies the straight-line method of expense recognition to all awards with only service-based vesting conditions. The Company has not issued any awards with performance-based vesting conditions.

For stock-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed. At the end of each financial reporting period prior to the completion of the service, the fair value of these awards is remeasured using the then-current fair value of the Company's common stock and updated assumption inputs in the Black-Scholes option-pricing model.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. The expected term of stock options granted to non-employees is equal to the contractual term of the option award. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

Through January 25, 2018, the Company granted restricted common units to employees, directors and non-employees. In connection with the Company's Corporate Conversion on January 25, 2018, all restricted common units were converted to restricted shares of common stock.

The Company measures restricted common stock granted to employees and directors based on the fair value on the date of grant and recognizes compensation expense of those awards over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company generally issued restricted common stock with only service-based vesting conditions and records the expense for these awards using the straight-line method. The Company has not issued any awards with performance-based vesting conditions.

The Company measured restricted common stock awards granted to consultants and non-employees based on the fair value of the award on the date of grant. Compensation expense is recognized over the period during which services are rendered by such consultants and non-employees until completed. At the end of each financial reporting period prior to completion of the service, the fair value of unvested awards is remeasured using the then-current fair value of the Company's common stock.

The Company classifies stock-based compensation expense in its condensed consolidated statement of operations and comprehensive loss in the same manner in which the award recipient's payroll costs are classified or in which the award recipient's service payments are classified.

Income Taxes

Income taxes are accounted for under the asset and liability method. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. The Company records valuation allowances to reduce deferred income tax assets to the amount that is more likely than not to be realized. The Company determines whether it is more likely than not that a tax position will be sustained upon examination. If it is not more likely than not that a position will be sustained, no amount of benefit attributable to the position is recognized. The tax benefit to be recognized of any tax position that meets the more likely than not recognition threshold is calculated as the largest amount that is more than 50% likely of being realized upon resolution of the contingency.

Prior to January 25, 2018, the Company had not been subject to U.S. federal income taxes as the Company was organized as a limited liability company. As such, the taxable income or loss was passed through to and included in the tax returns of the members. Since January 25, 2018, the Company's income has since been subject to U.S. federal, state, local, and foreign income taxes and taxed at the prevailing corporate tax rates.

Segment Data

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

Comprehensive Loss

Comprehensive loss includes net loss, as well as other changes in stockholders'/members' equity/(deficit) that result from transactions and economic events other than those with stockholders and members. The Company's only element of other comprehensive income (loss) in all periods presented was unrealized gains (losses) from available-for-sale securities.

Net Loss per Share

The Company follows the two-class method when computing net loss per share, as the Company has issued shares that meet the definition of participating securities. The two-class method determines net loss per share for each class of common and participating securities according to dividends declared or accumulated and participation rights in undistributed earnings. The two-class method requires income available to common stockholders for the period to be allocated between common and participating securities based upon their respective rights to receive dividends as if all income for the period had been distributed.

Basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period. Diluted net loss attributable to common stockholders is computed by adjusting net loss attributable to common stockholders to reallocate undistributed earnings based on the potential impact of dilutive securities. Diluted net loss per share attributable to common stockholders is computed by dividing the diluted net loss attributable to common stockholders by the weighted average number of shares of common stock outstanding for the period, including potential dilutive shares of common stock assuming the dilutive effect of common stock equivalents.

The Company's preferred stock contractually entitles the holders of such shares to participate in dividends but does not contractually require the holders of such shares to participate in losses of the Company. Accordingly, in periods in which the Company reports a net loss, such losses are not allocated to such participating securities. In periods in which the Company reports a net loss attributable to common stockholders, diluted net loss per share attributable to common stockholders is the same as basic net loss per share attributable to common stockholders, since dilutive shares of common stock are not assumed to have been issued if their effect is anti-dilutive.

Contingencies

Loss contingency provisions are recorded if the potential loss from any claim, asserted or unasserted, or legal proceeding is considered probable and the amount can be reasonably estimated or a range of loss can be determined. These accruals represent the Company's best estimate of probable loss. Disclosure also is provided when it is reasonably possible that a loss will be incurred or when it is reasonably possible that the amount of a loss will exceed the recorded provision. The Company reviews the status of each significant matter and assesses its potential financial exposure. Significant judgment is required in both the determination of probability and the determination as to whether an exposure is reasonably estimable. Because of uncertainties related to these matters, accruals are based only on the best information available at the time. As additional information becomes available, the Company reassesses the potential liability related to pending claims and may change its estimates. These changes in the estimates of the potential liabilities could have a material impact on the Company's condensed consolidated results of operations and financial position.

Recently Adopted Accounting Pronouncements

In May 2017, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting* ("ASU 2017-09"). ASC 2017-09 provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The Company adopted this standard in the first quarter of 2018 and its adoption did not have any impact on the Company's condensed consolidated financial statements.

In November 2016, the FASB issued ASU 2016-18, *Statement of Cash Flows* ("ASU 2016-18"), which requires that amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents. The Company adopted this standard in the first quarter of 2018 and its adoption resulted in the inclusion of restricted cash in total cash and cash equivalents in the determination of changes in cash and cash equivalents in the Company's condensed consolidated statements of cash flows. The presentation of restricted cash on the condensed consolidated balance sheet remains the same.

A reconciliation of the amounts from the cash flow statement to the balance sheet is as follows:

	June 30, 2018	June 30, 2017
Cash and cash equivalents as presented on balance sheet	$145,824	$32,716
Restricted cash, current, as presented on balance sheet	—	—
Restricted cash, non-current, as presented on balance sheet	237	161
Cash and cash equivalents and restricted cash as presented on cash flow statement	$146,061	$32,877

In August 2016, the FASB issued ASU 2016-15, *Statement of Cash Flows (Topic 230): Classification of Certain Cash Receipts and Cash Payments* ("ASU 2016-15"). ASU 2016-15 reduces diversity in practice by providing guidance on the classification of certain cash receipts and payments in the statement of cash flows. ASU 2016-15 clarifies that when cash receipts and cash payments have aspects of more than one class of cash flows and cannot be separated, classification will depend on the predominant source or use. ASU 2016-15 is effective on a retrospective basis. The Company adopted this standard in the first quarter of 2018 and the adoption of this standard did not have a material impact on the Company's condensed consolidated statements of cash flows.

Recently Issued Accounting Pronouncements

In February 2016, the FASB issued ASU 2016-02, *Leases (Topic 842)* ("ASU 2016-02"), which sets out the principles for the recognition, measurement, presentation and disclosure of leases for both parties to a contract (i.e., lessees and lessors). The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of their classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases today. ASU 2016-02 supersedes the previous leases standard, ASC 840, *Leases*. The standard is effective for public entities for annual periods beginning after December 15, 2018 and for interim periods within those fiscal years. The Company plans to adopt ASU 2016-02 effective January 1, 2019. The Company is currently evaluating the impact that the adoption of ASU 2016-02 will have on its condensed consolidated financial statements.

3. Merger and Recapitalization

On March 29, 2017, Solid Biosciences, LLC completed a series of transactions, which included the issuance of Series 1 Senior Preferred Units pursuant to the Senior Preferred Unit Purchase Agreement (the "Senior Preferred Unit Purchase Agreement") and the merger of Solid GT into Solid Biosciences, LLC pursuant to the merger agreement between Solid Biosciences, LLC and Solid GT (the "Merger Agreement"), collectively referred to as the "Merger and Recapitalization." As part of the Merger and Recapitalization, Solid Biosciences, LLC (a) issued 2,500,000 Series 1 Senior Preferred Units to new investors at $10.00 per unit resulting in gross proceeds to Solid Biosciences, LLC of $25,000, (b) merged operations of Solid GT into Solid Biosciences, LLC, effected through the exchange of Solid GT units held by non-controlling interests of Solid Biosciences, LLC into new classes Solid Biosciences, LLC units, and (c) exchanged existing Redeemable Preferred Units and Series A Common Units of Solid Biosciences, LLC into new units. The details of each component of the Merger and Recapitalization are as follows:

(a) Issuance of Series 1 Senior Preferred Units

Pursuant to the Senior Preferred Unit Purchase Agreement, Solid Biosciences, LLC issued 2,500,000 Series 1 Senior Preferred Units to new investors at $10.00 per unit resulting in gross proceeds to Solid Biosciences, LLC of $25,000.

(b) Merger of Solid GT into Solid Biosciences, LLC

Prior to the Merger and Recapitalization, Solid Biosciences, LLC issued Class B Non-Voting and Class D Voting Units of Solid GT to holders which represent non-controlling interests of Solid Biosciences, LLC. On March 29, 2017, in connection with the Merger and Recapitalization, the non-controlling interests were eliminated as follows:

- 50,000 Class B Non-Voting Units of Solid GT ("Solid GT Class B Units") were exchanged for 1,635,916 Series C Common Units of Solid Biosciences, LLC; and
- 134,920 Class D Voting Units of Solid GT ("Solid GT Class D Units") were exchanged for 4,414,356 Junior Preferred Units of Solid Biosciences, LLC.

In addition, the Class C Non-Voting Units of Solid GT ("Solid GT Class C Restricted Units") were exchanged for Series D Common Units of Solid Biosciences, LLC. The Solid GT Class C Restricted Units were held by employees and consultants of Solid GT.

Since there was no change in control in connection with the Solid GT merger, the exchange of Solid GT Class B Units, Class C Restricted Units and Class D Units was accounted for as an equity transaction. In addition, because Solid GT Class D Units represented preferred units with preference over the other classes of Solid GT Units, the difference between the carrying value of the Solid GT Class D Units and the fair value of Junior Preferred Units was recorded as a deemed dividend in members' deficit, which impacted net loss attributable to common unitholders.

(c) Exchange of Solid Biosciences, LLC existing Redeemable Preferred Units and Series A Common Units

In connection with the Merger and Recapitalization, Solid Biosciences, LLC exchanged its existing Redeemable Preferred Units and Series A Common Units as follows:

- 17,100,000 Redeemable Preferred Units of Solid Biosciences, LLC were exchanged for 12,219,299 Series A Common Units of Solid Biosciences, LLC.
- 4,560,000 Series A Common Units of Solid Biosciences, LLC were exchanged for 3,258,480 Series B Common Units of Solid Biosciences, LLC.
- 563,917 Series A Common Units of Solid Biosciences, LLC were exchanged for 402,963 Series D Common Units of Solid Biosciences, LLC.

The table below displays the pre-merger and post-merger capitalization structure of Solid Biosciences, LLC:

Pre-Merger and Recapitalization			Post-Merger and Recapitalization		
Entity	**Class**	**Issued**	**Entity**	**Class**	**Issued**
Company	Redeemable Preferred	17,100,000	**Company**	Series A Common	12,219,299
Company	Series A Common (Founders)	4,560,000	**Company**	Series B Common	3,258,480
Company	Series A Common (Others)	563,917	**Company**	Series D Common	402,963
Solid GT	Class A Voting	450,000		Ceased to exist	
Solid GT	Class B Non-Voting	50,000	**Company**	Series C Common	1,635,916
Solid GT	Class C Non-Voting	33,107	**Company**	Series D Common	1,083,205
Solid GT	Class D Voting	134,920	**Company**	Junior Preferred	4,414,356
Company (Total)	Common Units (Series A)	5,123,917	**Company (Total)**	Common Units (Series A, B, C and D)	18,599,863

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 June 30, 2018 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Cash equivalents	$ —	$ 749	$ —	$ 749
Available-for-sale securities	—	16,935	—	16,935
	$ —	$17,684	$ —	$17,684

	Fair Value Measurements as of December 31, 2017 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Available-for-sale securities	$ —	$17,014	$ —	$17,014

As of June 30, 2018 and December 31, 2017, the fair values of the Company's available-for-sale debt securities, which consisted of U.S. government agency securities and corporate bond securities, were determined using Level 2 inputs. During the six months ended June 30, 2018 and the year ended December 31, 2017, 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 June 30, 2018 and December 31, 2017, the fair value of available-for-sale securities by type of security was as follows:

	June 30, 2018			
	Amortized Cost	**Gross Unrealized Gain**	**Gross Unrealized Loss**	**Fair Value**
Investments:				
U.S. government agency securities	$ 9,286	$ —	$ (4)	$ 9,282
Corporate bond securities	7,659	—	(6)	7,653
	$ 16,945	$ —	$ (10)	$16,935

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
U.S. government agency securities	$ 9,473	$ —	$ (7)	$ 9,466
Corporate bond securities	7,554	—	(6)	7,548
	$ 17,027	$ —	$ (13)	$17,014

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

	June 30, 2018		December 31, 2017	
	Amortized Cost	Fair Value	Amortized Cost	Fair Value
Due in one year or less	$ 16,945	$16,935	$ 17,027	$17,014
Total available-for-sale securities	$ 16,945	$16,935	$ 17,027	$17,014

The weighted average maturity of the Company's available-for-sale securities as of June 30, 2018 and December 31, 2017 was approximately 0.4 years.

6. Property and Equipment

Property and equipment consists of the following:

	June 30, 2018	December 31, 2017
Furniture and fixtures	$ 66	$ 61
Laboratory equipment	3,670	2,338
Leasehold improvements	3,009	68
Computer equipment	166	77
Computer software	36	23
Construction in process	578	366
	7,525	2,933
Less accumulated depreciation	974	504
	$ 6,551	$ 2,429

Depreciation expense was $313 and $540 for the three and six months ended June 30, 2018, respectively, and $64 and $115 for the three and six months ended June 30, 2017, respectively.

7. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following:

	June 30, 2018	December 31, 2017
Accrued research and development	$ 1,394	$ 1,855
Accrued compensation	1,829	2,033
Deferred funding from charitable organizations	190	233
Accrued other	1,525	2,084
	$ 4,938	$ 6,205

8. Equity-Based Compensation

2018 Omnibus Incentive Plan

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. The following table summarizes the Company's stock option activity for the six months ended June 30, 2018:

	Number of Options	Weighted Average Exercise Price
Outstanding at December 31, 2017	—	$ —
Granted	758,182	24.78
Forfeitures	(5,409)	12.80
Outstanding at June 30, 2018	752,773	$ 24.87
Exercisable at June 30, 2018	—	$ —

At June 30, 2018, the Company had an aggregate of $11,787 of unrecognized equity-based compensation cost related to stock options, which is expected to be recognized over a weighted average period of 3.7 years.

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

	Six Months Ended June 30, 2018
Expected volatility	73.8% - 81.6%
Expected dividends	0.00%
Expected term (in years)	6.25
Risk-free rate	2.77% - 2.98%

The weighted average fair value of options to purchase shares of common stock granted during the six months ended June 30, 2018 was $16.91.

Restricted Common Stock

In connection with the Company's Corporate Conversion on January 25, 2018, all restricted Series B and D common units were converted to restricted shares of common stock. The following table summarizes the Company's unvested restricted shares of common stock activity from December 31, 2017 through June 30, 2018:

	Units / Shares	Weighted-Average Grant Date Fair Value
Unvested restricted Series D Common Units at December 31, 2017	1,404,265	$ 4.83
Vested units prior to Corporate Conversion	(66,019)	3.46
Forfeited units prior to Corporate Conversion	(8,932)	2.99
Unvested Series D Common Units at January 25, 2018 prior to Corporate Conversion	1,329,314	4.91
Conversion to restricted shares of common stock upon Corporate Conversion	1,128,182	5.79
Vested shares of common stock	(138,974)	3.78
Forfeited	(38,142)	5.36
Unvested restricted shares of common stock at June 30, 2018	951,066	$ 6.08

At June 30, 2018, the Company had an aggregate of $5,921 of unrecognized equity-based compensation cost related to restricted shares of common stock, which is expected to be recognized over a weighted average period of 1.6 years.

The Company recorded equity-based compensation expense related to all of its share and unit-based awards to employees and non-employees in the following captions within its condensed consolidated statements of operations for the three and six months ended June 30:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Research and development	$1,084	$111	$1,716	$ 504
General and administrative	225	376	412	3,213
Total	$1,309	$487	$2,128	$3,717

9. Income Taxes

The Company recorded no tax benefit for the three or six months ended June 30, 2018 for the net operating losses incurred due to its uncertainty of realizing a benefit from those items. The Company was not subject to federal and state income taxes for the three or six months ended June 30, 2017 as it was organized as a limited liability company which was taxed as a partnership for U.S. tax purposes.

A reconciliation of income taxes computed using the U.S. federal statutory rate to that reflected in operations as of June 30, 2018 is as follows:

	June 30, 2018
Income tax computed at federal statutory tax rate	21.0%
State taxes, net of federal benefit	4.3%
Permanent differences	(2.8)%
Tax credits	9.5%
Loss taxed as a partnership	(3.8)%
Conversion to a C-Corporation	1.1%
Other	(0.1)%
Valuation allowance	(29.2)%
	0.0%

The Company established deferred tax assets and liabilities on identified book to tax temporary differences as of the date of conversion to a C-corporation. Deferred income taxes reflect the net tax effects of these temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's net deferred tax assets as of June 30, 2018 is as follows:

	June 30, 2018
Deferred tax assets:	
Tax loss carryforwards	$ 6,128
Tax credit carryforwards	3,198
Intangible assets	106
Deferred revenues	52
Deferred expenses	139
Accrued expenses	460
Stock Compensation	139
Other	20
Total deferred tax assets	10,242
Depreciation	(234)
Valuation allowance	(10,008)
Net deferred taxes	$ —

As of June 30, 2018, the Company has federal net operating loss carryforwards of $21,861 and tax credits of $2,947 which may be used to offset future federal income and tax liability, respectively. In addition, the Company has state net operating loss carryforwards of approximately $21,886 and tax credits of $319 which may be used to offset future state income and tax liability, respectively. The Company's ability to utilize these federal and state carryforwards may be limited in the future if the Company experiences an ownership change pursuant to Internal Revenue Code Section 382. Ownership changes, as defined in the Internal Revenue Code,

including those resulting from the issuance of common stock in connection with the Company's public offerings, may limit the amount of net operating loss and tax credit carryforwards that can be utilized to offset future taxable income or tax liability. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards would be subject to an annual limitation under Section 382. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization. Further, until a study is completed and any limitation is known, no amounts are being presented as an uncertain tax position.

A valuation allowance is recorded against deferred tax assets if it is more likely than not that some or all of the deferred tax assets will not be realized. The Company has evaluated the positive and negative evidence bearing upon the realizability of the deferred tax assets. The Company concluded, in accordance with the applicable accounting standards, that it is more likely than not that the Company will not realize the benefit of its deferred tax assets. Accordingly, the Company has recorded a full valuation allowance against its deferred tax assets. The Company had approximately $10,008 in valuation allowances recorded against its deferred tax assets as of June 30, 2018.

As of June 30, 2018, the Company did not have unrecognized tax benefits. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of June 30, 2018, no interest and penalties have been recorded.

10. Commitments and Contingencies

Contingencies

In the first quarter of 2017, the Company terminated the development, manufacturing and testing agreement (the "Agreement") it had entered into in January 2016 with a third-party. The Company and the third-party were in dispute regarding the remaining amounts owed by the Company to the third-party under the Agreement. The Company settled the matter in April 2018 for $1,320.

Legal Proceedings

On March 27, 2018, a purported stockholder of the Company, filed a putative class action complaint alleging violations of the federal securities laws, in the United States District Court for the District of Massachusetts (Case No. 18-10587), against the Company and certain of the Company's current executive officers and underwriters in the Company's initial public offering. The plaintiff claims to represent purchasers of the Company's common stock during the period from January 25, 2018 to March 14, 2018 and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to the Company's lead product candidate. On May 29, 2018, the plaintiff and another purported stockholder filed separate motions each seeking his appointment as lead plaintiff and approval of his selected lead counsel. On June 5, 2018, the plaintiff withdrew his motion. The court has not yet issued an order appointing a lead plaintiff or approving lead counsel. Pursuant to a stipulation filed by the parties on April 16, 2018 and allowed by the court on April 30, 2018, the lead plaintiff will file an amended complaint within sixty (60) days after entry of the order appointing the lead plaintiff and approving lead counsel. Defendants will then answer, move, or otherwise respond to the amended complaint no later than sixty (60) days after the filing of that amended complaint. Lead plaintiff will have forty-five (45) days within which to file an opposition to any motion to dismiss filed by defendants, and defendants shall file a reply to lead plaintiff's opposition no later than forty-five (45) days after the filing of the opposition.

On March 28, 2018, a purported stockholder of the Company, filed a putative class action complaint alleging violations of the federal securities laws, in the Business Litigation Section of the Superior Court of the Commonwealth of Massachusetts (Civil Action No. 1884-00984), against the Company, Ilan Ganot, Jennifer Ziolkowski, the Company's directors and certain of the underwriters in the Company's initial public offering. The plaintiff in this suit claims to represent purchasers of the Company's common stock in or traceable to the Company's January 25, 2018 initial public offering and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to the Company's lead product candidate. On April 30, 2018, all defendants including the Company moved to stay the proceedings in favor of the prior-filed federal court securities class action. The plaintiff filed his opposition to this motion on May 14, 2018, and defendants filed a reply in support of their motion on May 24, 2018. After oral argument on June 13, 2018, the court issued an order on June 22, 2018 allowing the motion to stay and directing the parties to advise the court of the status of the federal court action every six months.

On April 3, 2018, a purported stockholder of the Company, filed a putative class action complaint alleging violations of the federal securities laws, in the United States District Court for the District of Massachusetts (Case No. 18-10639), against the Company, Ilan Ganot and Jennifer Ziolkowski. The plaintiff in this suit claims to represent purchasers of the Company's common stock during the period from January 25, 2018 to March 14, 2018 and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to the Company's lead product candidate. On June 6, 2018, the plaintiff voluntarily dismissed the action, without prejudice, as to all defendants.

While the Company is vigorously defending against all claims asserted, this litigation could result in substantial costs to the Company and a diversion of the Company's management's attention and resources, which could harm its business. In addition, the uncertainty of the pending lawsuits or potential filing of additional lawsuits could lead to more volatility and a reduction in the Company's stock price. Given the early stage of the litigation, at this time the Company is unable to reasonably estimate possible losses or form a judgment that an unfavorable outcome is either probable or remote. It is not currently possible to assess whether or not the outcome of these proceedings may have a material adverse effect on the Company.

Leases

In January 2018, the Company executed a lease agreement for lab space in Cambridge, Massachusetts. The lease consists of approximately 9,500 square feet with an initial term of five years with the option to extend the term for one additional two year term. The future minimum rent commitment for the initial five-year term is approximately $3,700. In addition to rent, the lease requires the Company to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

In January 2018, the Company executed a lease agreement for office space in Cambridge, Massachusetts. The space serves as the Company's corporate headquarters and consists of approximately 16,000 square feet. The term of the lease runs through February 2022. The future minimum rent commitment for the lease term is approximately $4,400. In addition to rent, the lease requires the Company to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

Future minimum lease payments for these operating leases as of June 30, 2018 were as follows:

Year Ending December 31,	
2018	$ 832
2019	1,917
2020	1,999
2021	2,038
2022	1,005
Thereafter	267
Total	$ 8,058

11. 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 for the three and six months ended June 30:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Net loss	$(17,980)	$(11,311)	$(33,857)	$(25,186)
Net loss attributable to non-controlling interest	—	—	—	(1,060)
Net loss attributable to Solid Biosciences Inc.	$(17,980)	$(11,311)	$(33,857)	$(24,126)
Accretion of preferred units to redemption value	—	—	—	(959)
Redemption of preferred units	—	—	—	15,685
Redemption of redeemable interest from non-controlling interest in Solid GT	—	—	—	(1,925)
Net loss attributable to common stockholders	$(17,980)	$(11,311)	$(33,857)	$(11,325)

The denominator is as follows for the three and six months ended June 30:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Weighted average shares of common stock outstanding, basic and diluted	34,449,758	17,041,311	31,916,295	10,083,502

Net loss per share attributable to common stockholders, basic and diluted is as follows for the three and six months ended June 30:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Net loss per share attributable to common stockholders	$(0.52)	$(0.66)	$(1.06)	$(1.12)

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 three and six months ended June 30:

	2018	2017
Options to purchase shares of common stock	752,773	—
Unvested shares of common stock	951,066	—
Series B common units	—	814,620
Series D common units	—	1,122,390
	1,703,839	**1,937,010**

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, 2017 included in our annual report filed on Form 10-K on March 29, 2018.

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, 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 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 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 DMD, a genetic muscle-wasting disease predominantly affecting boys, with symptoms that usually manifest between three and five years of age. DMD 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. DMD 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 DMD and, for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments. Our lead product candidate, SGT-001, is a gene transfer under development to restore functional dystrophin protein expression in patients' muscles. Based on our preclinical program that included multiple animal species of different phenotypes and genetic variations, we believe the mechanism of action of SGT-001, if our clinical trials prove to be successful, has the potential to slow or even halt the progression of DMD, regardless of the type of genetic mutation or stage of the disease. We filed an IND in September 2017 and initiated the IGNITE DMD clinical trial for SGT-001 in the United States during the fourth quarter of 2017. In November 2017, the FDA notified us that we were not permitted to dose patients in the higher-dose group of IGNITE DMD due to a partial clinical hold. The partial clinical hold related to the number of vials and manufacturing lots utilized per patient, as well as manufacturing processes to support the higher-dose group. We have since submitted a response to the FDA and were notified that the partial clinical hold has been resolved.

In March 2018, we announced that IGNITE DMD was placed on full clinical hold following an unexpected serious adverse event reported in the first patient dosed in the clinical trial. The event was characterized by a decrease in platelet count followed by a reduction in red blood cell count, transient renal impairment and evidence of complement activation. The patient received standard medical care, a modified steroid regimen and a limited course of eculizumab for the observed complement activation. He remained clinically stable and generally asymptomatic throughout the event, which fully resolved.

In its clinical hold letter, the FDA requested additional information, including an assessment of the etiology of the event, the patient's clinical status and laboratory parameters, and any additional measures to address patient safety. We submitted a comprehensive response to the FDA that included the requested information and in June 2018, the FDA lifted the clinical hold.

We made changes to the IGNITE DMD protocol, including the addition of IV glucocorticoids in the initial weeks post administration of SGT-001 and enhanced monitoring measures that include a panel for complement activation. The amended protocol also specifies that eculizumab will be available as a treatment option if complement activation is observed. We plan to enroll and dose several children prior to dosing additional adolescents, and now have the choice to obtain the intermediate muscle biopsy at 45 days post administration of SGT-001 to collect additional information about the time course of microdystrophin expression. We have since resumed screening patients for IGNITE DMD and we now expect to report initial data from a pre-specified interim analysis of IGNITE DMD in the second half of 2019.

Since our inception, we have devoted substantial resources to identifying and developing SGT-001 and our other product candidates, developing our manufacturing processes, organizing and staffing our company and providing general and administrative support for these operations. We do not have any products approved for sale. To date, we have not generated any revenue. 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 and our other product candidates. If successfully developed and approved, we intend to commercialize SGT-001 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 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.

We have never been profitable, and since our inception, we have incurred significant operating losses. Our net losses were $33.9 million and $25.2 million for the six months ended June 30, 2018 and 2017, respectively. As of June 30, 2018, we had an accumulated deficit of $158.1 million. We expect to incur significant expenses and increasing operating losses for the foreseeable future.

As we seek to develop and commercialize SGT-001 and our 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 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.

On January 30, 2018, we completed our initial public offering in which we sold 8,984,375 shares of our common stock, including the underwriters' over-allotment option, at a public offering price of $16.00 per share, in exchange for net proceeds of $129.1 million, after deducting underwriting discounts and commissions and offering expenses.

As of June 30, 2018, we had cash, cash equivalents and available-for-sale securities of $162.8 million. We believe that our existing cash, cash equivalents and available-for-sale securities will enable us to fund our operating expenses and capital expenditure requirements until the end of 2019. 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.

Corporate Conversion

We operated as a Delaware limited liability company under the name Solid Biosciences, LLC until immediately prior to the effectiveness of our registration statement on Form S-1 on January 25, 2018, at which time we converted into a Delaware corporation pursuant to a statutory conversion and changed our name to Solid Biosciences Inc., or the Corporate Conversion. As a result of the Corporate Conversion, the holders of the Series 1 and 2 Senior Preferred, Junior Preferred Units, Series A, B, C and D Common Units of Solid Biosciences, LLC became holders of common stock of Solid Biosciences Inc.

Merger and Recapitalization

We historically owned 100% of the voting units of our wholly owned subsidiary, Solid GT, LLC, or Solid GT, and the results of Solid GT are included in our condensed consolidated financial statements. Solid GT was organized in Delaware in August 2014 and was engaged in the business of developing disease-modifying interventions for DMD through gene therapy. In November 2015, Solid GT issued voting units to new investors, which decreased our voting ownership in Solid GT to 77%. We consolidated the results of Solid GT as we owned a majority voting interest in Solid GT and we directed the activities of Solid GT.

Net loss attributable to non-controlling interests in our condensed consolidated statement of operations and comprehensive loss consists of the portion of the net income or loss of Solid GT that is not allocated to us. Changes in the amount of net loss attributable to non-controlling interests are directly impacted by changes in the net income or loss of Solid GT. On March 29, 2017, we merged the operations of Solid GT into the company and Solid GT ceased to exist as a separate legal entity. As a result, for periods subsequent to March 29, 2017, we no longer report any non-controlling interests related to Solid GT.

Financial Operations Overview

Revenue

We have not generated any revenue as we do not have any approved products and do not expect to generate any revenue from the sale of our products for the next few years. If our development efforts for SGT-001 or our other product candidates are successful and result in marketing approval or if we enter into collaboration or license agreements with third parties, we may generate revenue in the future from a combination of product sales or payments from those collaboration or license agreements.

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 and our other product candidates and include:

- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct research, preclinical and clinical activities on our behalf as well as contract manufacturing organizations, or CMOs, that manufacture SGT-001 and our other product candidates for use in our preclinical 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;
- the costs of laboratory supplies and acquiring, developing and manufacturing preclinical study and clinical trial materials;
- costs incurred in seeking regulatory approval of SGT-001 and our other product candidates;
- expenses incurred under our intellectual property licenses; and
- facility-related research and development expenses, which include direct depreciation and rent costs as well as allocated expenses for rent and maintenance of facilities and other operating costs.

We expense research and development expenses as incurred. We recognize costs for certain development activities, such as preclinical research and development, 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 condensed 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:

	Three Months Ended June 30,		Six Months Ended June 30,	
(In thousands)	**2018**	**2017**	**2018**	**2017**
SGT-001	$ 6,124	$5,466	$13,177	$11,824
Other product candidates	792	493	1,478	954
Unallocated research and development expenses	6,678	2,648	10,868	4,562
Total research and development expenses	$13,594	$8,607	$25,523	$17,340

We cannot determine with certainty the duration, costs and timing of clinical trials of SGT-001 and our other product candidates or if, when or to what extent we will generate revenue from the commercialization and sale of any 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 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 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 continue to increase for the foreseeable future as we proceed with clinical trials for SGT-001, initiate clinical trials for product candidates other than SGT-001 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 potential commercialization of SGT-001 and our other product candidate. We also expect to incur increased expenses associated with being a public company, including costs of accounting, audit, legal, regulatory and tax-related services associated with maintaining compliance with exchange listing and SEC requirements, director and officer insurance costs and investor and public relations costs.

Other income (expense)

Interest income

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

Other income

We have received funding from charitable organizations, which are not considered to be an ongoing major or central part of our business. The amounts received are recorded as other income as services are performed and research expenses are incurred in the condensed consolidated statements of operations.

Income taxes

Since our inception in 2013, we had been organized as a Delaware limited liability company for federal and state income tax purposes and treated as a partnership for U.S. income tax purposes. As such, we were not viewed as a taxpaying entity in any jurisdiction and did not require a provision for income taxes. Each member of our company was responsible for the tax liability, if any, related to its proportionate share of our taxable income.

As a result of the Corporate Conversion on January 25, 2018, we are now treated as a corporation for U.S. income tax purposes and are subject to U.S. federal, state and local income taxes at the prevailing corporate tax rates. We expect to generate net operating losses at the corporate level.

We account for income taxes using an asset and liability approach. Under the asset and liability method, deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in income in the period that includes the enactment date. We record valuation allowances to reduce deferred income tax assets to the amount that is more likely than not to be realized. We determine whether it is more likely than not that a tax position will be sustained upon examination. If it is not more likely than not that a position will be sustained, no amount of benefit attributable to the position is recognized. The tax benefit to be recognized of any tax position that meets the more likely than not recognition threshold is calculated as the largest amount that is more than 50% likely of being realized upon resolution of the contingency.

Critical Accounting Policies and Use of Estimates

Our management's discussion and analysis of financial condition and results of operations is based on our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of our 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 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 under different assumptions or conditions.

During the three and six months ended June 30, 2018, 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, 2017 and the notes to the unaudited condensed consolidated financial statements included in Part I, Item 1, "Financial Statements (unaudited)," of this quarterly report on Form 10-Q. We believe that of our critical accounting policies, the following accounting policies involve the most judgment and complexity:

- Accrued research and development expenses 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 June 30, 2018 and 2017

The following table summarizes our results of operations for the three months ended June 30, 2018 and 2017:

	Three Months Ended June 30,		Increase
(in thousands)	**2018**	**2017**	**(decrease)**
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	13,594	8,607	4,987
General and administrative	4,584	3,280	1,304
Total operating expenses	18,178	11,887	6,291
Loss from operations	(18,178)	(11,887)	(6,291)
Other income (expense):			
Revaluation of preferred unit tranche rights	—	20	(20)
Interest income	80	52	28
Other income	118	504	(386)
Total other income (expense)	198	576	(378)
Net loss	$(17,980)	$(11,311)	$ (6,669)

Research and development expenses

	Three Months Ended June 30,		Increase
(in thousands)	**2018**	**2017**	**(decrease)**
SGT-001	$ 6,124	$5,466	$ 658
Other product candidates	792	493	299
Unallocated research and development expenses	6,678	2,648	4,030
Total research and development expenses	$13,594	$8,607	$ 4,987

Research and development expenses for the three months ended June 30, 2018 were $13.6 million, compared to $8.6 million for the three months ended June 30, 2017. The increase of $5.0 million in research and development costs was due to a $4.0 million increase in unallocated research and development costs driven by increased personnel and facility related expenses, including costs incurred to operate the new lab facility, a net $0.7 million increase in costs related to our lead product candidate SGT-001 driven by a $1.6 million increase in manufacturing activities offset by a $0.9 million reduction in preclinical costs, and a $0.3 million increase in costs related to our other product candidates.

General and administrative expenses

General and administrative expenses were $4.6 million for the three months ended June 30, 2018, compared to $3.3 million for the three months ended June 30, 2017. The increase of $1.3 million was due to an increase of $0.5 million in personnel and facility related expenses and a net increase of $0.8 million of other corporate expenses.

Interest income

Interest income remained consistent at $0.1 million for the three months ended June 30, 2018 and 2017.

Other income

Other income for the three months ended June 30, 2018 was $0.1 million compared to $0.5 million for the three months ended June 30, 2017. The decrease of $0.4 million related to income from charitable organizations. We do not expect these contributions to significantly increase in future periods.

Comparison of the six months ended June 30, 2018 and 2017

The following table summarizes our results of operations for the six months ended June 30, 2018 and 2017:

	Six Months Ended June 30,		Increase
(in thousands)	**2018**	**2017**	**(decrease)**
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	25,523	17,340	8,183
General and administrative	8,628	8,660	(32)
Total operating expenses	34,151	26,000	8,151
Loss from operations	(34,151)	(26,000)	(8,151)
Other income (expense):			
Revaluation of preferred unit tranche rights	—	20	(20)
Interest income	145	114	31
Other income	149	680	(531)
Total other income (expense)	294	814	(520)
Net loss	$(33,857)	$(25,186)	$ (8,671)

Research and development expenses

	Six Months Ended June 30,		Increase
(in thousands)	**2018**	**2017**	**(decrease)**
SGT-001	$13,177	$11,824	$ 1,353
Other product candidates	1,478	954	524
Unallocated research and development expenses	10,868	4,562	6,306
Total research and development expenses	$25,523	$17,340	$ 8,183

Research and development expenses for the six months ended June 30, 2018 were $25.5 million, compared to $17.3 million for the six months ended June 30, 2017. The increase of $8.2 million in research and development costs was due to a $6.3 million increase in unallocated research and development costs driven by increased personnel and facility related expenses, including costs incurred to operate the new lab facility, a net $1.4 million increase in costs related to our lead product candidate SGT-001 driven by a $4.8 million increase in clinical development and manufacturing activities offset by a $3.4 million reduction in preclinical costs, and a $0.5 million increase in costs related to our other product candidates.

General and administrative expenses

General and administrative expenses were $8.6 million for the six months ended June 30, 2018, compared to $8.7 million for the six months ended June 30, 2017. The decrease of $0.1 million was due to an increase of $1.8 million in personnel and facility related expenses and an increase of $0.9 million of other corporate expenses, offset by a decrease in equity-based compensation of $2.8 million. The decrease in equity-based compensation of $2.8 million was primarily due to a charge associated with the exchange of certain of our vested common units in connection with the recapitalization of Solid Biosciences, LLC and our merger with Solid GT on March 29, 2017.

Interest income

Interest income remained consistent at $0.1 million for the six months ended June 30, 2018 and 2017.

Other income

Other income for the six months ended June 30, 2018 was $0.1 million compared to $0.7 million for the six months ended June 30, 2017. The decrease of $0.6 million related to income from charitable organizations. We do not expect these contributions to significantly increase in future periods.

Liquidity and Capital Resources

Sources of liquidity

To date, we have financed our operations primarily through private placements of preferred units and our initial public offering. Through June 30, 2018, 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 $129.1 million of net proceeds from the sale of our common stock after deducting underwriting discounts and commission and offering expenses in our initial public offering.

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, in exchange for net proceeds of $129.1 million.

As of June 30, 2018, we had cash, cash equivalents and available-for-sale securities of $162.8 million and had no debt outstanding.

Cash flows

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

	Six Months Ended June 30,		Increase
(in thousands)	2018	2017	(decrease)
Cash used in operating activities	$ (33,503)	$(19,803)	$ (13,700)
Cash (used in) provided by investing activities	(4,101)	20,573	(24,674)
Cash provided by financing activities	131,520	24,264	107,256
Net increase in cash, cash equivalents and restricted cash	$ 93,916	$ 25,034	$ 68,882

Operating activities

During the six months ended June 30, 2018, operating activities used $33.5 million of cash, primarily resulting from our net loss of $33.9 million and cash used in changes in our operating assets and liabilities of $2.3 million offset by non-cash charges of $2.6 million due primarily to equity-based compensation of $2.1 million. Net cash used in changes in our operating assets and liabilities during the six months ended June 30, 2018 consisted of an increase in prepaid expenses and other assets of $0.4 million due to an increase in director and officers' insurance as we entered the public company market and deposits made for our new facilities and a decrease in accounts payable and accrued expenses of $1.9 million due to the timing of payments.

During the six months ended June 30, 2017, operating activities used $19.8 million of cash, primarily resulting from our net loss of $25.2 million offset by non-cash charges of $4.0 million due primarily to equity-based compensation of $3.7 million, which included $3.2 million associated with the exchange of Series A common units into Series B and D common units, and cash provided by changes in our operating assets and liabilities of $1.4 million. Net cash provided by changes in our operating assets and liabilities

during the six months ended June 30, 2017 consisted primarily of a decrease in prepaid expenses and other currents assets of $1.6 million due to the timing of prepaid research and development expense payments and net increase in accounts payable and accrued expenses of $0.2 million due to the timing of payments.

Investing activities

During the six months ended June 30, 2018, investing activities used $4.1 million of cash, consisting primarily of purchases of available-for-sale securities and property and equipment partially offset by proceeds on the sale and maturity of available-for-sale securities.

During the six months ended June 30, 2017, investing activities provided $20.6 million of cash, consisting primarily from proceeds on the sale and maturity of available-for-sale securities partially offset by purchases of available-for-sale securities and property and equipment.

We expect that purchases of property and equipment will increase over the next several years resulting from our move into a new office and laboratory facility in 2018.

Financing activities

During the six months ended June 30, 2018, net cash provided by financing activities was $131.5 million, primarily due to the proceeds from our initial public offering of $133.7 million partially offset by $2.2 million of payments made in connection with costs incurred for our initial public offering.

During the six months ended June 30, 2017, net cash provided by financing activities was $24.3 million, primarily due to the net proceeds from our sale of Series 1 Senior Preferred Units of $24.5 million offset in part by payments made in connection with our then-proposed initial public offering.

Funding Requirements

We expect our expenses to increase substantially in connection with our ongoing development activities related to SGT-001. 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:

- seek to enroll patients in IGNITE DMD and continue clinical development of SGT-001;
- move other current or future product candidates into clinical trials;
- continue research and preclinical development of our other product candidate;
- 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 and protect 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 ongoing development activity;
- acquire or in-license other drugs and technologies; and
- add operational, financial and management information systems and personnel.

On January 30, 2018, we completed our initial public offering in which we sold 8,984,375 shares of common stock, including shares of common stock issued upon the exercise in full of the underwriters' over-allotment option, at a public offering price of $16.00 per share, resulting in net proceeds of $129.1 million, after deducting underwriting discounts and commissions and offering expenses.

As of June 30, 2018, we had cash, cash equivalents and available-for-sale securities of $162.8 million. We believe that our existing cash, cash equivalents and available-for-sale securities will enable us to fund our operating expenses and capital expenditure requirements until the end of 2019. 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 and 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 and our other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001 and our other product candidates;
- the scope, progress, results and costs of drug discovery, laboratory testing, manufacturing, preclinical development and clinical trials for 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 or 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; and
- the extent to which we acquire or in-license other product candidates, technologies and intellectual property.

We intend to supply our clinical development program for SGT-001 with drug product produced at a current good manufacturing practices, or cGMP, compliant facility located at one of our Contract Development Manufacturing Organizations, or CDMO, partners. We intend to establish the capability and capacity to supply SGT-001 at commercial scale from multiple sources, including potentially building our own GMP facility to ensure redundancy and reliability. We expect that such a facility would require capital expenditures of between $35.0 million to $45.0 million to commence operations. We expect to finalize plans to potentially build our own GMP facility after we have initial data from IGNITE DMD.

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

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

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

Contractual Obligations and Commitments

The following table summarizes our contractual obligations at June 30, 2018 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

	Payments due by period				
(in thousands)	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More Than 5 Years
Operating lease commitments (1)	$8,058	$ 1,763	$3,999	$2,296	$ —

(1) Represents minimum payments due for our leases of office and laboratory space in Cambridge, Massachusetts.

In January 2018, we executed a lease agreement for lab space in Cambridge, Massachusetts. The lease consists of approximately 9,500 square feet with an initial term of five years with the option to extend the term for one additional two-year term. The future minimum rent commitment for the initial five-year term is approximately $3.7 million. In addition to rent, the lease requires us to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

In January 2018, we executed a lease agreement for office space in Cambridge, Massachusetts. The lease will serve as our corporate headquarters and consists of approximately 16,000 square feet. The term of the lease runs through February 2022. The future minimum rent commitment for the lease term is approximately $4.4 million. In addition to rent, the lease requires us to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

Under various agreements with third-party licensors, we have agreed to make milestone payments and pay royalties to third parties based on specific milestones. We have not included any such contingent payment obligations in the table above as the amount, timing and likelihood of such payments are not known.

We enter into contracts in the normal course of business with CROs and CMOs for clinical trials, preclinical research studies and testing, manufacturing and other services and products for operating purposes. These contracts do not contain any minimum purchase commitments and are cancelable by us upon prior notice of 30 days and, as a result, are not included in the table of contractual obligations above.

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, 2017.

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 June 30, 2018, our cash equivalents consisted of money market accounts that have contractual maturities of less than 90 days. As of June 30, 2018, our investments consisted of corporate bond securities and U.S. government agency 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 principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2018. 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 its 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 June 30, 2018, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures were not effective at the reasonable assurance level, due to the material weakness described below.

Material Weakness

The management of the company is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. In connection with the preparation of our consolidated financial statements as of and for the years ended December 31, 2016 and 2015, we identified material weaknesses in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. The material weaknesses we identified were as follows:

- We did not design or maintain an effective control environment commensurate with our financial reporting requirements. We lacked a sufficient number of professionals with an appropriate level of accounting knowledge, training and experience to appropriately analyze, record and disclose accounting matters timely and accurately. Additionally, the limited personnel resulted in our inability to consistently establish appropriate authorities and responsibilities in pursuit of our financial reporting objectives, as demonstrated by, among other things, our insufficient segregation of duties in our finance and accounting functions. The material weakness contributed to the additional material weaknesses detailed below.
- We did not design and maintain formal accounting policies, procedures and controls to achieve complete, accurate and timely financial accounting, reporting and disclosures, including controls over the preparation and review of account reconciliations and journal entries. Additionally, we did not design and maintain controls over the appropriate cut-off, classification and presentation of accounts and disclosures in the financial statements.
- We did not design and maintain formal accounting policies, processes and controls to analyze, account for and disclose complex transactions. Specifically, we did not design and maintain controls to analyze, account for and disclose complex transactions, including variable interest entities, preferred units, the preferred unit tranche right and equity-based compensation.

These material weaknesses also resulted in a restatement of our previously issued 2015 annual consolidated financial statements and adjustments to our 2016 annual consolidated financial statements, which were recorded prior to their issuance.

We are in the process of implementing measures designed to improve our internal control over financial reporting and remediate the control deficiencies that led to the material weaknesses, including hiring additional finance and accounting personnel and initiating design and implementation of our financial control environment, including the establishment of formal accounting policies and procedures, financial reporting controls and controls to account for and disclose complex transactions.

Changes in Internal Control over Financial Reporting

Other than as described above, there were no changes in our internal control over financial reporting identified in connection with the evaluation required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the fiscal quarter ended June 30, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

On March 27, 2018, James Watkins, a purported stockholder of ours, filed a putative class action complaint alleging violations of the federal securities laws, in the United States District Court for the District of Massachusetts (Case No. 18-10587), against us, Ilan Ganot, our Chief Executive Officer, Jennifer Ziolkowski, our Chief Financial Officer, and the underwriters in our initial public offering, J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC, Leerink Partners, LLC, Nomura Securities Co., LLC and Chardan Capital Markets LLC. The plaintiff in this suit claims to represent purchasers of our common stock during the period from January 25, 2018 to March 14, 2018 and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to our lead product candidate. On May 29, 2018, the plaintiff, Mr. Watkins, and Ashish Bhandari, another purported stockholder, filed separate motions each seeking his appointment as lead plaintiff and approval of his selected lead counsel. On June 5, 2018, Mr. Watkins withdrew his motion. The court has not yet issued an order appointing a lead plaintiff or approving lead counsel. Pursuant to a stipulation filed by the parties on April 16, 2018 and allowed by the court on April 30, 2018, the lead plaintiff will file an amended complaint within sixty (60) days after entry of the order appointing the lead plaintiff and approving lead counsel. Defendants will then answer, move, or otherwise respond to the amended complaint no later than sixty (60) days after the filing of that amended complaint. Lead plaintiff will have forty-five (45) days within which to file an opposition to any motion to dismiss filed by defendants, and defendants shall file a reply to lead plaintiff's opposition no later than forty-five (45) days after the filing of the opposition.

On March 28, 2018, Robert Lowinger, a purported stockholder of ours, filed a putative class action complaint alleging violations of the federal securities laws, in the Business Litigation Section of the Superior Court of the Commonwealth of Massachusetts (Civil Action No. 1884-00984), against us, Ilan Ganot, Jennifer Ziolkowski, our directors and certain of the underwriters in our initial public offering. The plaintiff in this suit claims to represent purchasers of our common stock in or traceable to our January 25, 2018 initial public offering and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to our lead product candidate. On April 30, 2018, all defendants including us moved to stay the proceedings in favor of the prior-filed federal court securities class action. The plaintiff filed his opposition to this motion on May 14, 2018, and defendants filed a reply in support of their motion on May 24, 2018. After oral argument on June 13, 2018, the court issued an order on June 22, 2018 allowing the motion to stay and directing the parties to advise the court of the status of the federal court action every six months.

On April 3, 2018, Michael Walsh, a purported stockholder of ours, filed a putative class action complaint alleging violations of the federal securities laws, in the United States District Court for the District of Massachusetts (Case No. 18-10639), against us, Ilan Ganot and Jennifer Ziolkowski. The plaintiff in this suit claims to represent purchasers of our common stock during the period from January 25, 2018 to March 14, 2018 and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to our lead product candidate. On June 6, 2018, the plaintiff voluntarily dismissed the action, without prejudice, as to all defendants.

While we believe that we have meritorious defenses to the allegations made in these complaints, it is not currently possible to assess whether or not the outcome of these suits may have a material adverse effect on our business, financial condition, results of operations or prospects.

In addition, we may be involved in various other legal proceedings arising out of our operations. We are not currently a party to any such other legal proceedings that, in the opinion of our management, are likely to have a material adverse effect on our business, financial condition, results of operations or prospects. Regardless of outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

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.

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 $33.9 million for the six months ended June 30, 2018. In addition, our net losses were $53.2 million, $23.8 million and $6.7 million for the years ended December 31, 2017, 2016 and 2015, respectively. As of June 30, 2018, we had an accumulated deficit of $158.1 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:

- seek to enroll patients in IGNITE DMD and continue clinical development of SGT-001;
- move other current or future product candidates into clinical trials;
- continue research and preclinical development of our other product candidate;
- 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 ongoing development activity;
- acquire or in-license other drugs, technologies and intellectual property; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more 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 seek to resume and complete clinical trials of SGT-001, obtain marketing approval for SGT-001, 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 and our other product candidates. In addition, if we obtain marketing approval for SGT-001 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 June 30, 2018 will enable us to fund our operating expenses and capital expenditure requirements until the end of 2019, we anticipate that we will need additional funding to complete the development of SGT-001 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 and our other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001 and our other product candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for 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 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; and
- the extent to which we acquire or in-license other product candidates, technologies and intellectual property.

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.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies, SGT-001 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 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 and our other product candidate, SB-001, 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 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 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 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 and our other product candidates, if approved;
- obtaining market acceptance, if and when approved, of SGT-001 and our 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 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.

Risks related to the development of our 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 one gene transfer product has been approved in the United States for commercialization and only two such products have been approved in the European Union.

We have concentrated our research and development efforts on SGT-001 for the treatment of DMD and our future success depends on our successful development of that product candidate. Our risk of failure is high. We have experienced, and may experience additional, 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 one *in vivo* gene transfer product, Spark Therapeutics, Inc.'s Luxturna, has received FDA approval and only one *in vivo* gene transfer product, uniQure N.V.'s Glybera, has received marketing authorization from the European Commission. 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.

In March 2018, the FDA placed IGNITE DMD on full clinical hold after we reported an unexpected serious adverse event in the first patient dosed in the clinical trial. Even though the full clinical hold was lifted in June 2018, we cannot guarantee that similar events will not happen in the future.

On February 14, 2018, the first patient in the IGNITE DMD clinical trial, a non-ambulatory adolescent, was dosed with 5E13 vg/kg of SGT-001. Several days after administration the patient was hospitalized due to laboratory findings that included a decrease in platelet count followed by a reduction in red blood cell count and evidence of complement activation. We reported the unexpected serious adverse event to the FDA and, because it was unexpected, classified it as Suspected Unexpected Serious Adverse Reaction, or SUSAR. In March 2018, the FDA placed a full clinical hold on SGT-001 following an unexpected serious adverse event in IGNITE DMD, which is designed to evaluate SGT-001 in ambulatory and non-ambulatory males with DMD aged four to 17 years, and we halted enrollment and dosing in IGNITE DMD. In June 2018, the FDA lifted the full clinical hold and we have since resumed screening patients for IGNITE DMD. In connection with the lifting of the clinical hold, we made changes to the IGNITE DMD protocol, including the addition of IV glucocorticoids in the initial weeks post administration of SGT-001 and enhanced monitoring measures that include a panel for complement activation. The amended protocol also specifies that eculizumab will be available as a treatment option if complement activation is observed. We also plan to enroll and dose several children in IGNITE DMD prior to dosing additional adolescents, and now have the choice to obtain the intermediate muscle biopsy at 45 days post administration of SGT-001 to collect additional information about the time course of microdystrophin expression. These changes to the IGNITE DMD protocol, and any other such changes that may be made in the future, may impact our development timeline and result in increased costs and expenses.

If the FDA or other regulatory agencies continue to express safety concerns even though the full clinical hold has been lifted, 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 and 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.

In addition, we may not be able to obtain institutional review board committee, or IRB, or data safety monitoring board committee approvals for IGNITE DMD as a result of the clinical hold, and any related risks or otherwise, 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 since-resolved full 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, our lead product candidate, 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 or our product candidates.

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, recently we reported an unexpected serious adverse event in IGNITE DMD, which resulted in a full clinical hold which has since been lifted by the FDA. In addition, it is possible that as we test SGT-001 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. 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 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. Recently, James M. Wilson, M.D., Ph.D., resigned from our Scientific Advisory Board citing 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 our 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 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 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 only recently initiated our first clinical trial for SGT-001 and have not commenced preclinical studies for our other product candidates. 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 or our other product candidates. We only recently initiated our first clinical trial for SGT-001, 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 DMD as the FDA has not given clear guidance as to the necessary endpoints for approval of a treatment for DMD. In addition, we have had limited interactions with the FDA and cannot be certain how many clinical trials of SGT-001 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 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 and our other product candidates.

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

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

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 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 resuming IGNITE DMD for SGT-001 following the lifting of the clinical hold by the FDA;
- 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 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; 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 or our other product candidates, we may:

- be delayed or fail in obtaining marketing approval for SGT-001 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 the products 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 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 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 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 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 or our other product candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-001 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 to participate in our gene therapy clinical trials because of negative publicity from adverse events related to our product candidates, including the unexpected serious adverse event we recently reported, other approved gene therapies, the biotechnology or gene therapy fields, 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. These delays could result in increased costs, delays in advancing SGT-001 or our other product candidates, delays in testing the effectiveness of SGT-001 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;
- 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 March 2018, the FDA placed IGNITE DMD on full clinical hold following our report of an unexpected serious adverse event in the clinical trial. In June 2018, the FDA lifted the full clinical hold. We only recently reinitiated screening of patients for the clinical trial and may not be able to enroll patients on the timeline expected.

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 or our other product candidates and the approval may be for a more narrow indication than we seek.

We cannot commercialize SGT-001 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.

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 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.

If we fail to comply with applicable regulatory requirements following approval of SGT-001 or our other product candidates, a regulatory authority may, among other things, suspend or withdraw regulatory approval, narrow the product label, restrict the marketing or manufacturing of the product, suspend any ongoing clinical trials or seize or detain the product or otherwise require the withdrawal of the product from the market.

Even if we obtain and maintain approval for SGT-001 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 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 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 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 RAC; however, the NIH announced that the Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC, will soon 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. 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.

In addition, 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 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 DMD 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 DMD.

Even if we maintain orphan drug exclusivity for SGT-001 or obtain orphan drug exclusivity for our 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.

We may seek a breakthrough therapy designation for SGT-001 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 or our other product candidates; however, we cannot assure our stockholders that SGT-001 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 qualify as breakthrough therapies, 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.

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 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.

The FDA has granted RPDD to SGT-001. RPDD does not guarantee that a BLA for such drug will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. We will need to request a rare pediatric disease priority review voucher in our BLA for SGT-001. The use of a priority review voucher allows for a drug to be reviewed by the FDA within six months. However, the FDA may determine that a BLA for SGT-001 does not meet the eligibility criteria for a priority review voucher upon approval. Moreover, even if SGT-001 does satisfy those criteria, the product will need to be licensed before September 30, 2022 in order to be granted a rare disease priority review voucher.

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

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. If we seek fast track designation for a product candidate, we may not receive it from the FDA. Even if we receive fast track designation, 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 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 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.

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 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 DMD. 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. 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 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 or our other product candidates. 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 and our other product candidates

We may not be successful in finding strategic collaborators for continuing development of SGT-001 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 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 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 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 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 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 and our other product candidates. Any such failure could delay or prevent our commercialization of SGT-001 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 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 other MAA on a timely basis and must adhere to the FDA's and the European Union's cGMP requirements before SGT-001 and our other product candidates can obtain marketing approval. 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 will need to increase the scale of our manufacturing process at our third-party manufacturers, 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, 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 and consistency of yields of our manufacturing process. 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, there could be a significant disruption in supply of SGT-001 or our other product candidates. Further, we may not be able to enter into arrangements with 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, our other product candidates or future product candidates.

Although we may establish our own SGT-001 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 intend to utilize materials manufactured by cGMP-compliant third-party suppliers. Even following our potential establishment of a validated cGMP manufacturing facility, we intend to maintain our current and additional 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 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. 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 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. 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 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, which will expose us to risks including:

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

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 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 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 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 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 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 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 DMD. Our understanding of the patient population with this disease is based on estimates in published literature and by DMD 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 DMD 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 DMD, the exact DMD-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 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 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 adversely affect our ability to conduct our business or obtain regulatory approvals for SGT-001.

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 DMD 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 event that led to the full clinical hold on IGNITE DMD, which has since been lifted by the FDA, 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.

Failure to comply with ongoing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures.

The regulatory authorities may, at any time following approval of a product for sale, audit the manufacturing facilities for such product. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility.

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 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, no gene transfer product has 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 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 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 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, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, the referendum could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our 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 our 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.

If we are unable to manage expected 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, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the EMA and other regulatory authorities, comply with health care fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the health care industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, including insider trading, which could result in regulatory sanctions and cause serious harm to our reputation. 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 government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us and we are not successful in defending ourselves or asserting our rights, those actions could result in 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.

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. 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 the President 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, will become effective in 2019. According to the Congressional Budget Office, the repeal of the individual mandate will cause 13 million fewer Americans to be insured in 2027 and premiums in insurance markets may rise. Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the Health Care Reform Law. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the Health Care Reform Law. The Congress will likely consider other legislation to replace elements of the Health Care Reform Law, during the next Congressional session.

The Trump Administration has also taken executive actions to change or delay implementation of the Health Care Reform Law. In January 2017, President Trump signed an Executive Order 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. In October 2017, the President signed a second Executive Order allowing for the use of association health plans and short-term health insurance, which may provide fewer health benefits than the plans sold through the Health Care Reform Law exchanges. At the same time, the Administration announced that it will discontinue the payment of CSR payments to insurance companies until Congress approves the appropriation of funds for such CSR payments. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the Health Care Reform Law. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain. Further, in July 2018 following a federal district court decision from New Mexico, the Administration announced that it would be freezing payments to insurers under the Health Care Reform Law to cover sicker patients until it or Congress can address the appropriate methodology for calculating and making such payments. It remains to be seen how this action will affect the implementation of the Health Care Reform Law.

We will continue to evaluate the effect that the Health Care Reform Law and its possible repeal and replacement could have on our business. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace Health Care Reform Law provisions is uncertain in many respects, it is also possible that some of the Health Care Reform Law provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with Health Care Reform Law coverage expansion provisions. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop commercialize product candidates.

The costs of prescription pharmaceuticals in the United States has also been the subject of considerable discussion in the United States, and members of Congress and the Administration have stated that they will address such costs through new legislative and administrative measures. To date, there have been several recent U.S. congressional inquiries and proposed state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. The pricing of prescription pharmaceuticals is also subject to governmental control outside the United States. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. 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 candidates to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our ability to generate revenues and become profitable could be impaired. The uncertain status of the Health Care Reform Law ability to may have a negative impact on our business.

In addition, on May 11, 2018, the Administration issued a plan to lower drug prices. Under this blueprint for action, the Administration indicated that the Department of Health and Human Services will: take steps to end the gaming of regulatory and patent processes by drug makers to unfairly protect monopolies; advance biosimilars and generics to boost price competition; evaluate the inclusion of prices in drug makers' ads to enhance price competition; speed access to and lower the cost of new drugs by clarifying policies for sharing information between insurers and drug makers; avoid excessive pricing by relying more on value-based pricing by expanding outcome-based payments in Medicare and Medicaid; work to give Part D plan sponsors more negotiation power with drug makers; examine which Medicare Part B drugs could be negotiated for a lower price by Part D plans, and improving the design of the Part B Competitive Acquisition Program; update Medicare's drug-pricing dashboard to increase transparency; prohibit Part D contracts that include "gag rules" that prevent pharmacists from informing patients when they could pay less out-of-pocket by not using insurance; and require that Part D plan members be provided with an annual statement of plan payments, out-of-pocket spending, and drug price increases.

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.

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.

There have been a number of federal and state legislative changes made over the last few years regarding the pricing of pharmaceutical and biologic products. Concerns about drug pricing have been expressed by members of Congress and the President.

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.

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 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;
- the federal Health Insurance Portability and Accountability Act of 1996, or 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.

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, 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 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 may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize SGT-001.

Our ability to develop and commercialize SGT-001 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 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. 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, could be adversely affected. For more information, see Part I, Item 1, "Business—Strategic partnerships and collaborations/licenses" in our Annual Report on Form 10-K for the year ended December 31, 2017.

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 vector we are developing for use in SGT-001 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 and such AAV vector. Such licenses may not be available on commercially reasonable terms, or at all. 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. 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 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 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, 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 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, we currently do not own any issued patents or pending non-provisional patent applications and we only own three provisional patent applications in the United States. Each of these 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 also currently do not own or license any issued patents or pending patent applications with respect to our product candidate SB-001. While we have an option to negotiate a license for issued patents and pending patent applications relating to such product candidate, we may not exercise our option in a timely manner or at all, or satisfy any conditions upon which our option to such patents and patent applications is contingent. In addition, the third party granting us such option may breach our option agreement and license such patents and patent applications to other third parties, including our competitors, before we exercise our option. In any event, even if we exercise such option, we are still required to negotiate and enter into a definitive agreement pursuant to which we could license rights to the optioned patents and we may be unable to enter into such a definitive agreement within the required timeframe or under terms that are acceptable to us. If we are unable to do so, the party who has granted us our option may offer the patent rights to other parties. If we are unable to secure a license to any issued patents and pending patent applications relating to SB-001, we may need to cease our development of such product candidate.

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 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, SB-001 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 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.

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 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 or our other product candidates;
- lose patent protection for SGT-001 or our other product candidates;
- experience significant delays in the development or commercialization of SGT-001 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 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 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, 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 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 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 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 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 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 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 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 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.

We may not be successful in obtaining necessary rights to SGT-001 or our other product candidates through acquisitions and in-licenses.

We currently have certain rights to intellectual property, through licenses from third parties, to develop SGT-001. Because development and commercialization of our current and future product candidates may require the use of additional proprietary rights held by these or other third parties, the growth of our business may depend, in part, on our ability to acquire, in-license or use these additional proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes or other intellectual property rights from third parties that we identify as necessary for SGT-001 or our other product candidates. 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.

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 required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of SGT-001 or our other product candidates.

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.

Issued patents relating to SGT-001 or our other product candidates could be found invalid or unenforceable if challenged.

If one of our licensing partners or we initiate legal proceedings against a third party to enforce a patent relating to SGT-001 or our other product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, written description, non-enablement or failure to claim patent eligible subject matter. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review, interference proceedings, derivation proceedings and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). 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 or our other product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner, we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on SGT-001 or our other product candidates or technologies.

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 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 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 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 all matters submitted to our stockholders for approval.

Our executive officers, directors and stockholders who own more than 5% of our outstanding common stock, in the aggregate, beneficially own shares representing approximately 66.0% of our common stock outstanding as of August 1, 2018. As a result, if these stockholders were to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they act together, would control 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 could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in management of our company with which our public stockholders disagree.

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. All lock-up agreements entered into in connection with our initial public offering expired on July 24, 2018. 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, as of August 1, 2018, holders of an aggregate of approximately

24.2 million 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 addition, on January 29, 2018, we filed a Registration Statement on Form S-8 to register approximately 5.0 million shares reserved for future issuance under our 2018 Omnibus Incentive Plan will become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. These shares can be freely sold in the public market upon issuance, subject to black-out periods and volume limitations applicable to affiliates.

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

Our stock price has been and may in the future 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 clinical trials of SGT-001 or our other product candidates or those of our competitors;
- the success of competitive products or technologies;
- 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. As described in Part II, Item 1, “Legal Proceedings,” we and certain of our executive officers and board members have been named as defendants in purported class action lawsuits. This litigation, and any additional litigation instituted against us, could cause us to incur substantial costs to defend such claims and divert management’s attention and resources.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. If one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

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

Prior to our initial public offering, which occurred on January 26, 2018, there was no public market for our common stock. 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 our stockholders to sell shares without depressing the market price for the shares, or at all.

We are an "emerging growth company," and the reduced disclosure requirements applicable to emerging growth 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 may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in this quarterly report on Form 10-Q. 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 now 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 will be required to furnish a report by our management on our internal control over financial reporting, including, once we are no longer an EGC, 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.

We have identified material weaknesses in our internal control over financial reporting. If we are unable to remediate these material weaknesses, or if we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect our business and stock price.

We have identified material weaknesses in our internal control over financial reporting. A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. If we are unable to remediate these material weaknesses, or if we identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, we may not be able to accurately or timely report our financial condition or results of operations, which may adversely affect investor confidence in us and, as a result, our stock price.

In connection with the audits of our consolidated financial statements as of and for the years ended December 31, 2015 and December 31, 2016, we identified material weaknesses in our internal control over financial reporting. The material weaknesses we identified were as follows:

- We did not design or maintain an effective control environment commensurate with our financial reporting requirements. We lacked a sufficient number of professionals with an appropriate level of accounting knowledge, training and experience to appropriately analyze, record and disclose accounting matters timely and accurately. Additionally, the limited personnel resulted in our inability to consistently establish appropriate authorities and responsibilities in pursuit of our financial reporting objectives, as demonstrated by, among other things, our insufficient segregation of duties in our finance and accounting functions. This material weakness contributed to the additional material weaknesses detailed below.
- We did not design and maintain formal accounting policies, procedures and controls to achieve complete, accurate and timely financial accounting, reporting and disclosures, including controls over the preparation and review of account reconciliations and journal entries. Additionally, we did not design and maintain controls over the appropriate cut-off, classification and presentation of accounts and disclosures in the financial statements.
- We did not design and maintain formal accounting policies, processes and controls to analyze, account for and disclose complex transactions. Specifically, we did not design and maintain controls to analyze, account for and disclose complex transactions, including variable interest entities, preferred units, the preferred unit tranche right and equity-based compensation.

Each of the control deficiencies could result in a misstatement of our accounts or disclosures that would result in a material misstatement of our annual or interim consolidated financial statements that would not be prevented or detected, and accordingly, we determined that these control deficiencies constitute material weaknesses.

These material weaknesses also resulted in a restatement of our previously issued 2015 annual consolidated financial statements and adjustments to our 2016 annual consolidated financial statements, which were recorded prior to their issuance.

We are in the process of implementing measures designed to improve our internal control over financial reporting and remediate the control deficiencies that led to the material weaknesses, including hiring additional finance and accounting personnel and initiating design and implementation of our financial control environment, including the establishment of formal accounting policies and procedures, financial reporting controls and controls to account for and disclose complex transactions.

We cannot assure our stockholders that the measures we have taken to date, and actions we may take in the future, will be sufficient to remediate the control deficiencies that led to our material weaknesses in our internal control over financial reporting or that they will prevent or avoid potential future material weaknesses. In addition, neither our management nor an independent registered public accounting firm has performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act because no such evaluation has been required. Had we or our independent registered public accounting firm performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act, additional material weaknesses may have been identified. If we are unable to successfully remediate our existing or any future material weaknesses in our internal control over financial reporting, or identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our share price may decline as a result.

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 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. 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.

Use of Proceeds from Initial Public Offering

On January 30, 2018, we closed our initial public offering, in which we issued and sold 8,984,375 shares of common stock, including 1,171,875 shares of our common stock pursuant to the underwriters' over-allotment, at a public offering price of $16.00 per share. The aggregate gross proceeds to us from our initial public offering were approximately $143.8 million. All of the shares of common stock issued and sold in our initial public offering were registered under the Securities Act pursuant to a registration statement on Form S-1 (Registration No. 333-222357), which was declared effective by the SEC on January 25, 2018 and a registration statement on Form S-1 MEF (Registration No. 333-222705) filed pursuant to Rule 462(b) of the Securities Act. J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC were joint book-running managers for the initial public offering. The offering commenced on January 25, 2018 and did not terminate until the sale of all of the shares offered. The aggregate net proceeds to us were approximately $129.1 million, after deducting underwriting discounts and commissions and offering expenses payable by us of approximately $14.7 million.

Except as set forth below, no offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates. JPMC Strategic Investments II Corporation, or JPMC, owned in excess of 10% of our issued and outstanding common stock immediately prior to our initial public offering, and JPMC is an affiliate of J.P. Morgan Securities LLC, which was a book running manager in our initial public offering. In addition, Mr. Robert Huffines, one of our directors, is an employee of J.P. Morgan Securities LLC.

There has been no material change in the planned use of net proceeds from our initial public offering as described in our final prospectus filed with the SEC on January 29, 2018 pursuant to Rule 424(b). We have been using and plan to continue to use the net offering proceeds to fund research, development and clinical trial expenses, and the remainder for general and administrative expenses and other general corporate purposes.

Item 6. Exhibits.

Exhibit Number	Description
10.1	Summary of Non-Employee Director Compensation Program
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	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

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: August 10, 2018

By: /s/ Ilan Ganot

Ilan Ganot
Chief Executive Officer (Principal Executive Officer)

Date: August 10, 2018

By: /s/ Jennifer Ziolkowski

Jennifer Ziolkowski
Chief Financial Officer (Principal Financial and Accounting Officer)

Exhibit 10.1

SOLID BIOSCIENCES INC.

NON-EMPLOYEE DIRECTOR COMPENSATION POLICY

Effective as of July 25, 2018, the non-employee directors of Solid Biosciences Inc. (the "Company") shall receive the following compensation for their service as members of the Board of Directors of the Company (the "Board"). Unless otherwise noted, Andrey Zarur, the Chairman of the Board, is not deemed a non-employee director for purposes of this Policy, and his compensation is separately set forth below.

Director Compensation

Our goal is to provide compensation for our non-employee directors in a manner that enables us to attract and retain outstanding director candidates and reflects the substantial time commitment necessary to oversee the Company's affairs. We also seek to align the interests of our directors and our stockholders and we have chosen to do so by compensating our non-employee directors with a mix of cash and equity-based compensation.

Cash Compensation

The fees that will be paid to our non-employee directors for service on the Board, and for service on each committee of the Board on which the director is then a member, and the fees that will be paid to the chairperson of each committee of the Board will be as follows:

	Member Annual Fee	Chairperson Incremental Annual Fee
Board of Directors	$35,000	–
Audit Committee	$ 7,500	$ 7,500
Compensation Committee	$ 5,000	$ 5,000
Nominating and Corporate Governance Committee	$ 4,000	$ 4,000

The foregoing fees will be payable in arrears in equal semi-annual installments not later than the 15th business day following the end of the second and fourth calendar quarters, provided that the amount of such payment will be prorated for any portion of such semi-annual period that the director is not serving on the Board, on such committee or in such position, and no fee shall be payable in respect of any period prior to the completion of our initial public offering.

In his role as Chairman of the Board, Andrey Zarur will be paid cash compensation for fiscal year 2018 in the amount of $225,000. Unless otherwise noted, Dr. Zarur will not receive any other cash or equity compensation described in this Policy.

Equity Compensation

Initial Grants. Upon initial election to our Board, each non-employee director will be granted, automatically and without the need for any further action by the Board, an initial equity award of an option, with a Black-Scholes value of $100,000, to purchase shares of our common stock. The initial award shall have a term of ten years from the date of the award, and shall vest and become exercisable as to 1/3 of the shares underlying such award on each anniversary of the grant date until the third anniversary of the grant date, subject to the director's continued service as a director through each applicable vesting date. The vesting shall accelerate as to 100% of the shares upon a change in control of the Company. The exercise price shall be the closing price of our common stock on the date of grant.

Annual Grants. Each non-employee director who has served as a member of our Board for at least six months prior to the date of our annual meeting of stockholders for a particular year will be granted, automatically and without the need for any further action by the Board, an equity award on the date of our annual meeting of stockholders for such year of an option, with a Black-Scholes value of $50,000, to purchase shares of our common stock. The annual award shall have a term of ten years from the date of the award, and shall vest and become exercisable in full on the earlier to occur of the one-year anniversary of the grant date and immediately prior to our first annual meeting of stockholders occurring after the grant date, subject, in each case, to the director's continued service as a director through the applicable vesting date. The vesting shall accelerate as to 100% of the shares upon a change in control of the Company. The exercise price shall be the closing price of our common stock on the date of grant.

The foregoing share amounts shall be automatically adjusted in the event of any change in the capital structure or business of the Company by reason of any stock split, reverse stock split, stock dividend, combination or reclassification of shares, recapitalization, merger, consolidation, spin off, split off, reorganization or partial or complete liquidation, issuance of rights or warrants to purchase common stock or securities convertible into common stock, sale or transfer of all or part of the Company's assets or business, or other corporate transaction or event that would be considered an "equity restructuring" within the meaning of FASB ASC Topic 718, in each case, pursuant to the terms of our 2018 Omnibus Incentive Plan, or any successor plan.

The initial awards and the annual awards shall be subject to the terms and conditions of our 2018 Omnibus Incentive Plan, or any successor plan, and the terms of the option agreements entered into with each director in connection with such awards.

Expenses

Upon presentation of documentation of such expenses reasonably satisfactory to the Company, each non-employee director (including, in this case, Dr. Zarur) shall be reimbursed for his or her reasonable out-of-pocket business expenses incurred in connection with the performance of their duties as directors, including travel expenses in connection with their attendance in-person at Board and committee meetings.

Exhibit 31.1

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

I, 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)) 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) 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

    c) 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
Chief Executive Officer
(Principal Executive Officer)
Dated: August 10, 2018

Exhibit 31.2

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

I, Jennifer Ziolkowski, 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)) 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) 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

   c) 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/ Jennifer Ziolkowski
Jennifer Ziolkowski
Chief Financial Officer
(Principal Financial and Accounting Officer)
Dated: August 10, 2018

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of Solid Biosciences Inc. (the "Company") for the quarter ended June 30, 2018 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: August 10, 2018

/s/ Ilan Ganot
Ilan Ganot
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.

Exhibit 32.2

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

In connection with the Quarterly Report on Form 10-Q of Solid Biosciences Inc. (the "Company") for the quarter ended June 30, 2018 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Jennifer Ziolkowski, 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 her 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: August 10, 2018

/s/ Jennifer Ziolkowski
Jennifer Ziolkowski
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.