Filed Pursuant to Rule 424(b)(4)
Registration No. 333-222705

Prospectus

7,812,500 shares

Common Stock

This is an initial public offering of shares of common stock by Solid Biosciences Inc. Solid Biosciences Inc. is selling 7,812,500 shares of our common stock. The initial public offering price is $16.00 per share.

Prior to this offering, there has been no public market for our common stock. We have been approved to list our common stock on the NASDAQ Global Select Market, under the symbol "SLDB."

We are an "emerging growth company" as defined under the federal securities laws and will be subject to reduced public company reporting requirements.

	Per Share	Total
Initial public offering price	$ 16.00	$ 125,000,000
Underwriting discounts and commissions (1)	$ 1.12	$ 8,750,000
Proceeds, before expenses, to us	$ 14.88	$ 116,250,000

(1) We refer you to "Underwriting (conflicts of interest)" for additional disclosure regarding total underwriting compensation.

We have granted the underwriters an option for a period of 30 days to purchase up to an additional 1,171,875 shares of common stock.

Certain of our existing stockholders have agreed to purchase an aggregate of approximately 3,955,000 shares of our common stock in this offering at the initial public offering price. The underwriters will receive the same underwriting discounts and commissions on the shares purchased by these stockholders as they will on the other shares sold to the public in this offering.

Investing in our common stock involves a high degree of risk. Before buying any shares, you should carefully read the discussion of material risks of investing in our common stock in "Risk factors" beginning on page 14 of this prospectus.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to investors on or about January 30, 2018.

J.P. Morgan **Goldman Sachs & Co. LLC** **Leerink Partners**

Nomura **Chardan**

The date of this prospectus is January 25, 2018

Neither we nor the underwriters have authorized anyone to provide you with information other than that contained in this prospectus or any free writing prospectus prepared by or on behalf of us or to which we have referred you. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. We and the underwriters are offering to sell, and seeking offers to buy, common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus or any free writing prospectus is accurate only as of its date, regardless of its time of delivery or of any sale of shares of our common stock. Our business, financial condition, results of operations and prospects may have changed since that date.

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No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

PROSPECTUS SUMMARY

This prospectus summary highlights certain information appearing elsewhere in this prospectus. As this is a summary, it does not contain all of the information that you should consider in making an investment decision. You should read the entire prospectus carefully, including the information under "Risk factors," "Management's discussion and analysis of financial condition and results of operations" and our consolidated financial statements and the related notes thereto included in this prospectus, before investing. This prospectus includes forward-looking statements that involve risks and uncertainties. See "Information regarding forward-looking statements." In this prospectus, unless the context otherwise requires, the terms "Solid Biosciences," "Solid," "the company," "we," "us" and "our" refer, prior to the Corporate Conversion discussed herein, to Solid Biosciences, LLC and its subsidiaries, and after the Corporate Conversion, to Solid Biosciences Inc. and its subsidiaries.

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.

SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, in the United States and Orphan Drug Designations in both the United States and European Union. The safety and efficacy of SGT-001 are currently being evaluated in a Phase I/II clinical trial.

Our founders, who are personally touched by the disease, created a biotechnology company purpose-built to accelerate the discovery and development of meaningful therapies for all patients affected by DMD. Through this disease-focused business model, our research team, led by experts in DMD biology and drug development, along with key opinion leaders in DMD, continuously evaluate emerging science to identify high-potential product candidates. Our selection process includes extensive diligence and initial pharmacology research with highly specific, predefined criteria, which provide us with confidence in our development program decisions. Through this data-driven selection process, we have evaluated a number of programs and identified gene therapy as a potentially beneficial approach for DMD, and thus initiated development of our lead product candidate SGT-001. We will continue to apply this rigorous approach and reject the majority of the candidates we evaluate in our effort to develop only programs that we believe have the greatest likelihood of becoming therapies for DMD patients.

SGT-001 is a gene transfer candidate designed to address the underlying genetic cause of DMD by delivering a synthetic transgene that produces dystrophin-like protein that is only expressed in muscles of the body, including cardiac and respiratory muscles. The transgene is delivered via an adeno-associated virus, or AAV, vector, which also contains a muscle-specific promoter. Our vector is a modified version of an AAV, a naturally occurring, non-pathogenic virus selected for its ability to efficiently enter skeletal, diaphragm and cardiac muscle tissues. The vector will carry a synthetic dystrophin transgene construct, called microdystrophin,

that retains the most critical components of the full-size dystrophin gene yet is small enough to fit within AAV packaging constraints. SGT-001 is designed to drive microdystrophin protein expression in affected muscles throughout the body. We have studied the efficacy, safety and durability of SGT-001 in multiple preclinical models and its functional benefits in DMD animal studies. In contrast to other therapeutic approaches that are designed to target specific mutations in the dystrophin gene, we believe SGT-001 is a mutation agnostic approach.

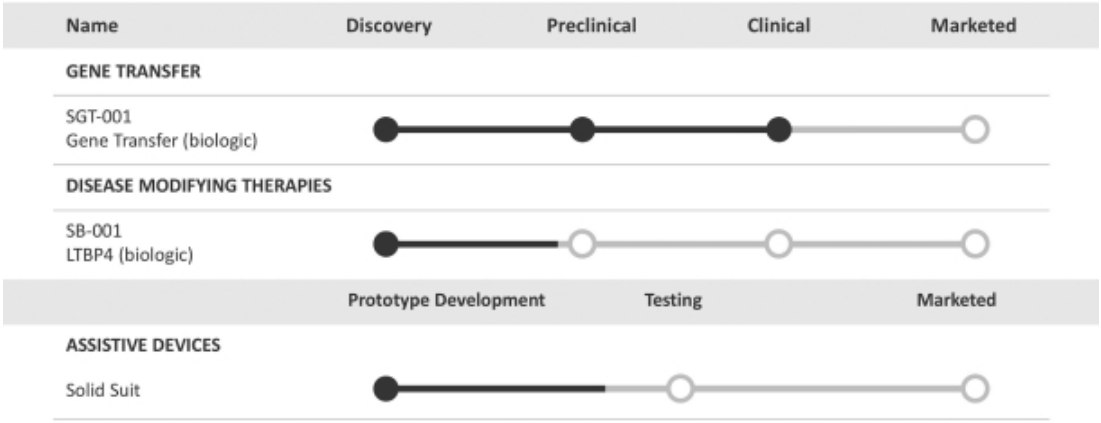
In the fourth quarter of 2017, we announced the initiation of a randomized, controlled, open-label, single-ascending dose Phase I/II clinical study, called IGNITE DMD, designed to evaluate SGT-001 in ambulatory and non-ambulatory males with DMD aged four to 17 years. The primary objectives of the study are to assess the safety and tolerability of SGT-001, as well as efficacy as defined by microdystrophin protein expression. The study will also assess muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with microdystrophin production, patient reported outcomes and quality of life measures, among other endpoints. The study will enroll approximately 16 to 32 patients with DMD, who will be randomly assigned to either an active treatment group or a delayed-treatment control group. Initially, adolescents aged 12 to 17 years will receive treatment, and at a later stage of the study, children aged four to 11 years will be dosed. Our Investigational New Drug application, or IND, permits us to proceed with administering our proposed low dose to patients. Prior to dosing patients in our higher-dose group, we will be required to resolve the partial clinical hold on SGT-001 outlined in a November 2017 letter to us from the U.S. Food and Drug Administration, or the FDA. In order to do so we will need to decrease the number of vials and utilize no more than a single production lot per patient and demonstrate that we have the appropriate manufacturing processes in place to support the higher-dose group. In addition, the FDA had additional comments and requests for information that were characterized as not clinical hold comments. We expect that we will be able to address the specific deficiencies identified by the FDA by submitting additional information demonstrating manufacturing capacity and product attributes that will support the high-dose group. The Company intends to submit a response to the FDA addressing the specific deficiencies in the near future, after which the FDA will have 30 days to respond. The Company does not expect that the overall timing for clinical development of SGT-001 will be affected by the partial clinical hold. Further, the partial clinical hold does not impact the Company's ability to conduct its clinical development activities of SGT-001 at low-dose levels. If the partial clinical hold is not lifted on our Phase I/II clinical trial, we will not be able to evaluate the safety, tolerability and efficacy of SGT-001 at the high-dose level, which could negatively impact the development of SGT-001. Efficacy will be assessed by comparing microdystrophin protein expression in muscle biopsy before treatment and 12 months after treatment for each patient. Participants in the control group who continue to meet inclusion criteria and not meet exclusion criteria will receive active treatment after 12 months. Based on results from this study, we will evaluate the need for future clinical trials that may include other patient populations, as well as the need for larger confirmatory clinical trials. If approved, we intend to commercialize SGT-001 in the United States and European Union, and we may enter into licensing agreements or strategic collaborations to commercialize the product candidate in other markets.

Taking into account the prevalence and incidence of DMD and the anticipated dosing requirements for gene transfer, we anticipate that there will be a need for a substantial supply of SGT-001 for clinical trials and, if approved, for commercial markets. Through significant targeted investments to address this challenge, we believe we have generated sufficient drug product supply to initiate our first clinical trial. We continue to develop our manufacturing process to meet future clinical and commercial production needs for SGT-001.

While we believe DMD disease progression can be slowed or halted by gene transfer, many patients will still suffer from the manifestations of the disease, such as tissue damage to their muscles, inflammation, cardiac dysfunction and fibrosis. As part of our disease-focused business model, we are also building a portfolio of complementary disease-modifying therapies to address these manifestations. Our portfolio currently includes a preclinical biologic candidate, SB-001, a monoclonal antibody designed to reduce fibrosis and inflammation, as well as a number of emerging and complementary programs. We intend to commence preclinical studies for SB-001 in 2018.

In addition to developing our pipeline of product candidates, we believe it is critical to invest time and resources in tools and technologies designed to help us more effectively understand DMD, accurately monitor disease progression and assist patients in daily life. As part of this goal, we are developing biomarkers and sensors that may allow us to identify treatment targets faster, measure the therapeutic impact of potential product candidates better and reach decision points earlier. In addition, through our Solid Suit program, we are developing a line of soft, wearable assistive devices with the goal of providing functional and therapeutic benefits to DMD patients.

Our pipeline



We seek to protect our proprietary and intellectual property position through a combination of patents, trade secret laws, proprietary know-how, continuing technological innovation, and entering into non-disclosure, confidentiality and invention assignment agreements. We have exclusively licensed three issued U.S. patents, one pending U.S. non-provisional patent application, and seven issued patents and eleven pending patent applications in foreign jurisdictions. We have filed two pending U.S. provisional patent applications. We intend to continue building out our intellectual property protection to further strengthen our position in the DMD field.

Who we are

Solid Biosciences was founded in 2013 by our Chief Executive Officer, Ilan Ganot, our Chairman of the Board, Andrey Zarur, and our President, Gilad Hayeem, with the goal of developing meaningful therapies for patients with DMD. Solid is the English translation of Eytani, the Hebrew name of Ilan and Annie Ganot's son, who was diagnosed with the disease in 2012. Our founders, unsatisfied with the existing therapeutic landscape, proceeded to raise funds to execute on our disease-focused business model. We assembled a passionate management team and scientific advisory board composed of individuals with extensive experience in DMD, gene therapy, product discovery, research and development, manufacturing, business strategy and finance.

In 2015, we began exclusively licensing the elements of the construct for SGT-001 and other elements of our gene transfer program from the University of Michigan, the University of Missouri and the University of Washington. Since then, we have continued to use our extensive network across the academic, business and patient communities to identify, vet and pursue high-potential complementary product candidates to address the needs of DMD patients.

Since our inception, we have raised private capital from a group of investors, including entities affiliated with Bain Capital Life Sciences, Biogen, JPMC Strategic Investments II Corporation, Perceptive Advisors and RA Capital, along with several additional corporate and private investors. In addition, three leading U.K.-based DMD charities provided initial seed funding for our gene transfer program in return for equity in our company. We continue to work closely with the patient advocacy community and have accepted additional contributions from several DMD charities to fund our early-stage research programs.

Mission

Our mission, which guides every aspect of our operations, is to cure DMD. Underscoring this mission, our disease-focused business model is founded on the following fundamental values:

- identify and develop meaningful therapies for all patients with DMD;
- bring together the leading experts in DMD science, technology, disease management and care; and
- be guided by the needs of DMD patients.

Our strengths

Guided by our mission, we set out to create a company that understands DMD and develops therapies that are intended to provide meaningful benefits to DMD patients. We believe we are well positioned to execute on our mission based on the following competitive strengths:

- ***Singular focus on DMD.*** We are singularly focused on meeting the diverse needs of all DMD patients, regardless of their genetic mutation or disease stage.
- ***Deep understanding of the impact of the disease.*** We are founded by people personally touched by DMD, and we have established meaningful partnerships within the DMD community, which provide us with unique insights into the disease.
- ***Rigorous product candidate selection process.*** We subject each potential product candidate to a highly focused, data-driven selection process that lies at the core of our business model.
- ***Highly experienced management team focused on DMD.*** Our management team has extensive expertise in DMD, gene therapy, product discovery, research and development, manufacturing, business strategy and finance.
- ***Network of world-renowned experts advising our development efforts.*** We have assembled a scientific advisory board and a broad network of the world's leading experts in DMD, gene therapy, biologics manufacturing, immunology and clinical development.
- ***Foundational work in scalable manufacturing processes.*** We are working to develop a scalable manufacturing process to meet future clinical and commercial production needs for SGT-001.

Our strategic priorities

Our disease-focused business model is purpose-built to identify and accelerate the discovery and development of multiple product candidates. Key elements of our strategy include the following:

- Rapidly advance SGT-001 through clinical trials and deliver it to patients;
- Continue to advance SB-001 and emerging and complementary programs through preclinical development;
- Continue to build our product pipeline with high-potential product candidates for DMD;

- Continue to scale our manufacturing process to meet clinical and commercial needs;
- Develop tools to accelerate the discovery and development of therapies for DMD; and
- Partner with the DMD community to inform our programs.

Recent developments

In connection with the unit purchase agreement, originally entered into on March 29, 2017 and as amended, or the Senior Preferred Unit Purchase Agreement, pursuant to which we previously sold $25.0 million of Series 1 Senior Preferred Units to certain investors, we achieved certain preclinical milestones that required the holders of our Series 1 Senior Preferred Units to purchase Series 2 Senior Preferred Units. On October 26, 2017, we closed this second round of financing under the Senior Preferred Unit Purchase Agreement raising net proceeds of $55.0 million. We refer to this sale of the Series 2 Senior Preferred Units as our Series 2 Senior Preferred Financing in this prospectus. See "Certain Relationship and Related-Person Transactions —Equity Financings."

We estimate that as of December 31, 2017, our cash, cash equivalents and available-for-sale securities was approximately $69.0 million. Our independent registered public accountants have not audited, reviewed or performed any procedures with respect to this financial data and accordingly do not express an opinion or any other form of assurance with respect thereto. This financial data reflects the best information available to management as of the date of this prospectus and could change as a result of our financial close process and subsequent review and audit by our independent registered public accountants.

Risks associated with our business

Investing in our common stock involves a high degree of risk. You should carefully consider the risks described in "Risk factors" before making a decision to invest in our common stock. If any of these risks actually occurs, our business, financial condition, results of operations and prospects would likely be materially adversely affected. In that event, the trading price of our common stock could decline, and you could lose part or all of your investment. Below is a summary of some of the principal risks we face:

- 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.
- Even if this offering is successful, we will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.
- We have never generated revenue from product sales and do not expect to do so for the next several years, if ever.
- 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.
- The FDA placed the SGT-001 Phase I/II clinical trial on partial clinical hold requiring us to submit additional CMC information that demonstrates that manufacturing capacity and product attributes can support the high dose group.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- Success in preclinical studies may not be indicative of results obtained in later trials.
- We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

- Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize SGT-001 or our other product candidates and the approval may be for a more narrow indication than we seek.
- 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.
- We face significant competition.
- We have limited gene transfer manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-001 or our other product candidates.
- Although we intend to 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.
- 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.
- The commercial success of SGT-001 and our other product candidates, if approved, will depend upon market acceptance by physicians, patients, third-party payors and others in the medical community.
- 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.
- We rely heavily on our license agreements for rights to intellectual property granted to us by others to develop and commercialize SGT-001.
- If we are unable to obtain and maintain patent protection for our product candidates or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected.
- If we are unable to conduct our business without infringing or otherwise violating any intellectual property rights of any third party, our ability to successfully commercialize our product candidates may be adversely affected.
- 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.

Implications of being an emerging growth company

We qualify as an “emerging growth company” as defined in the Jumpstart our Business Startups Act of 2012, or the JOBS Act. An emerging growth company may take advantage of specified reduced reporting and other burdens that are otherwise applicable generally to public companies. These provisions include:

- inclusion of only two years, as compared to three 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;

- an exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting pursuant to the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act;
- an exemption from compliance with any new requirements adopted by the Public Company Accounting Oversight Board requiring mandatory audit firm rotation;
- reduced disclosure about executive compensation arrangements; and
- an exemption from the requirement to seek non-binding advisory votes on executive compensation or golden parachute arrangements.

We may take advantage of these provisions until we are no longer an emerging growth company. We will remain an emerging growth company until the earliest of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least $1.07 billion or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, and (2) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period.

We have taken advantage of the reduced reporting requirements in this prospectus. Accordingly, the information contained herein may be different from the information you receive from other public companies that are not emerging growth companies.

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.

Our corporate information

We were originally formed as SOLID Ventures Management, LLC in March 2013 as a Delaware limited liability company. We changed our name in June 2015 to Solid Biosciences, LLC. Prior to the effectiveness of the registration statement of which this prospectus forms a part, we will convert into a Delaware corporation pursuant to a statutory conversion and be renamed Solid Biosciences Inc. In addition, entities affiliated with certain of our unitholders will be merged with and into us. See “Corporate conversion.”

Our principal executive offices are located at 161 First Street, Third Floor, Cambridge, MA 02142. Our main telephone number is (617) 337-4680. Our internet website is www.solidbio.com. The information contained in, or that can be accessed through, our website is not incorporated by reference and is not a part of this prospectus.

Trademark notice

We have registered trademarks with the U.S. Patent and Trademark Office, or USPTO, for the marks “SOLID BIOSCIENCES”, “SOLID GT” and “SOLID”. All other trademarks, service marks and trade names in this prospectus are the property of their respective owners. We have omitted the ® and ™ designations, as applicable, for the trademarks used in this prospectus.

THE OFFERING	
Common stock offered by us	7,812,500 shares
Common stock to be outstanding after this offering	34,151,022 shares
Option to purchase additional shares	We have granted the underwriters a 30-day option to purchase up to 1,171,875 additional shares of our common stock.
Use of proceeds	We expect to receive net proceeds from this offering of approximately $112.5 million, or approximately $129.9 million if the underwriters exercise their option to purchase additional shares of our common stock in full, at the initial public offering price of $16.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering (including any additional proceeds that we may receive if the underwriters exercise their option to purchase additional shares of our common stock), together with our existing cash and cash equivalents, as follows: approximately $150.0 million to fund research and development expenses, including to advance SGT-001 through preliminary results from Phase I/II clinical trial activities, which we initiated in the fourth quarter of 2017; and the remainder for general and administrative expenses and other general corporate purposes. See “Use of proceeds.”
NASDAQ symbol	We have been approved to list our common stock on the NASDAQ Global Select Market under the symbol “SLDB.”
Risk factors	Investing in our common stock involves a high degree of risk. See “Risk factors” beginning on page 14 of this prospectus for a discussion of factors you should carefully consider before deciding to invest in our common stock.
Conflicts of interest	Because an affiliate of J.P. Morgan Securities LLC, an underwriter in this offering, owns in excess of 10% of our issued and outstanding equity interests, J.P. Morgan Securities LLC is deemed to have a “conflict of interest” within the meaning of Rule 5121 of the Financial Industry Regulatory Authority, or FINRA. Accordingly, this offering is being made in compliance with the requirements of FINRA Rule 5121. In accordance with this rule, Goldman Sachs & Co. LLC has assumed the responsibilities of acting as a qualified independent underwriter and has participated in due diligence and the preparation of this prospectus and the registration statement of which this prospectus is a part. For more information, please see “Underwriting (conflicts of interest)—Conflicts of interest.”

Certain of our existing stockholders have agreed to purchase approximately 3,955,000 shares of our common stock in this offering at the initial public offering price. The underwriters will receive the same underwriting discounts and commissions on the shares purchased by these stockholders as they will on the other shares sold to the public in this offering.

The number of shares outstanding after this offering is based on 26,338,522 shares of our common stock outstanding as of September 30, 2017, after giving effect to the Series 2 Preferred Financing and the Corporate Conversion, and excludes:

- 171,433 shares of our common stock that will be converted, after giving effect to the Corporate Conversion, from 202,049 units granted to certain of our employees, including certain of our executive officers, after September 30, 2017; and
- 5,001,000 shares of our common stock reserved for issuance under our 2018 Omnibus Incentive Plan, or the 2018 Plan, which we expect to adopt in connection with this offering.

In addition, the number of shares outstanding after this offering includes:

- 11,396 shares of our common stock, which correspond to 13,432 units of Solid Biosciences, LLC that were forfeited after September 30, 2017.

Unless otherwise indicated, all information in this prospectus assumes:

- the completion of the Corporate Conversion, as a result of which all outstanding units of Solid Biosciences, LLC will be converted into 26,498,559 shares of common stock of Solid Biosciences Inc., on a one-for-0.8485 basis; and
- no exercise by the underwriters of their option to purchase 1,171,875 additional shares of our common stock.

SUMMARY CONSOLIDATED FINANCIAL DATA

You should read the following summary consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Cash and capitalization," "Selected consolidated financial data" and "Management's discussion and analysis of financial condition and results of operations" sections of this prospectus. We have derived the consolidated statements of operations data for the years ended December 31, 2015 and 2016 from our audited consolidated financial statements appearing at the end of this prospectus. The consolidated statement of operations data for the nine months ended September 30, 2016 and 2017 and the consolidated balance sheet data as of September 30, 2017 have been derived from our unaudited consolidated financial statements appearing at the end of this prospectus, which have been prepared on the same basis as the audited consolidated financial statements. In the opinion of management, the unaudited consolidated financial statements reflect all adjustments, consisting only of normal recurring adjustments, necessary for a fair statement of the financial information in those consolidated statements. Our historical results are not necessarily indicative of results that should be expected in any future period, and our results for any interim period are not necessarily indicative of results that should be expected for any full year.

	Year ended December 31,		Nine months ended September 30,	
(in thousands, except units and per unit data)	2015	2016	2016	2017
Consolidated statements of operations data:				
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	4,192	20,116	13,048	27,959
General and administrative	2,372	5,460	3,807	11,737
Total operating expenses	6,564	25,576	16,855	39,696
Loss from operations	(6,564)	(25,576)	(16,855)	(39,696)
Other income (expense):				
Revaluation of preferred unit tranche rights	(103)	1,163	1,163	(68)
Interest and other income	3	640	438	1,073
Total other income (expense), net	(100)	1,803	1,601	1,005
Net loss	$ (6,664)	$ (23,773)	$ (15,254)	$ (38,691)
Net loss per unit attributable to common unitholders, basic and diluted (1)	$ (7.61)	$ (10.14)	$ (7.50)	(1.99)
Weighted average common units outstanding, basic and diluted (1)	846,569	1,698,904	1,677,909	12,446,769

	Year ended December 31, 2016	Nine months ended September 30, 2017
Pro forma net loss per share (2):		
Pro forma net loss per share attributable to common stockholders, basic and diluted	$ (1.62)	$ (2.05)
Pro forma weighted average common shares outstanding, basic and diluted	14,052,917	19,233,147

(in thousands)	As of September 30, 2017		
	Actual	Pro forma (2)	Pro forma as adjusted (2)(3)
Consolidated balance sheet data:			
Cash, cash equivalents and available-for-sale securities	$ 29,570	$ 84,570	$ 197,020
Working capital (4)	18,966	74,493	186,943
Total assets	35,445	90,445	202,895
Redeemable preferred units	69,177	—	—
Accumulated deficit	(109,771)	(109,244)	(109,244)
Total members'/stockholders' equity (deficit)	(45,583)	79,121	191,571

(1) See Note 15 to our financial statements appearing at the end of this prospectus for details on the calculation of basic and diluted net loss per unit attributable to common unitholders.

(2) Pro forma gives effect to the Corporate Conversion as well as the Series 2 Senior Preferred Financing. Pro forma information is illustrative only.

(3) Pro forma as adjusted gives effect to (i) the Corporate Conversion as well as the Series 2 Senior Preferred Financing and (ii) the sale of shares of our common stock in this offering at the initial public offering price of $16.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. This pro forma as adjusted information is illustrative only and will depend on the actual initial public offering price and other terms of this offering determined at pricing.

(4) We define working capital as current assets less current liabilities.

INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This prospectus includes forward-looking statements, which involve risks and uncertainties. These forward-looking statements can be identified by the use of forward-looking terminology, including the terms “believe,” “estimate,” “project,” “anticipate,” “expect,” “seek,” “predict,” “continue,” “possible,” “intend,” “may,” “might,” “will,” “could,” would” or “should” or, in each case, their negative, or other variations or comparable terminology. These forward-looking statements include all matters that are not historical facts. They appear in a number of places throughout this prospectus and include statements regarding our intentions, beliefs or current expectations concerning, among other things, our product candidates, research and development and clinical trial plans, commercialization objectives, prospects, strategies, the industry in which we operate and potential collaborations. We derive many of our forward-looking statements from our operating budgets and forecasts, which are based upon many detailed assumptions. While we believe that our assumptions are reasonable, we caution that it is very difficult to predict the impact of known factors, and, of course, it is impossible for us to anticipate all factors that could affect our actual results. All forward-looking statements are based upon information available to us on the date of this prospectus. Important factors that could cause our results to vary from expectations include, but are not limited to:

- the timing, progress and results of preclinical studies and clinical trials for SGT-001 and our other product candidates;
- our ability to obtain and maintain U.S. regulatory approval of SGT-001, and the timing and scope thereof;
- the potential for substantial delays in our clinical trials or our failure to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities;
- our ability to obtain and maintain foreign regulatory approvals, and the timing and the scope thereof;
- undesirable side effects or other properties relating to our product candidates that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval;
- the size of the patient populations for SGT-001 and our other product candidates, if approved for commercial use;
- our manufacturing capabilities and strategy, including the scalability and commercial viability of our manufacturing methods and processes;
- our ability to successfully commercialize SGT-001 and our other product candidates, if approved;
- the pricing and reimbursement of SGT-001 and any other product candidates we may develop, if approved;
- the establishment of sales, marketing and distribution capabilities and entry into agreements with third parties to market and sell SGT-001 or our other product candidates, if approved;
- the rate and degree of market acceptance and clinical utility of SGT-001 and any other product candidates we may develop and for which we may receive approval;
- our expenses, ongoing losses, future revenue, capital requirements and need for and ability to obtain additional financing;
- our ability to identify, recruit and retain key personnel;
- our and our licensors’ ability to prosecute, maintain, protect and enforce our intellectual property rights for SGT-001 and our other product candidates, and the scope of such protection;
- our ability to avoid and defend against intellectual property infringement, misappropriation and other claims;
- our competition and market development; and
- the impact of laws and regulations on our operations.

By their nature, forward-looking statements involve risks and uncertainties because they relate to events and depend on circumstances that may or may not occur in the future. We caution you that forward-looking statements

are not guarantees of future performance and that our actual results of operations, financial condition, business and prospects may differ materially from those made in or suggested by the forward-looking statements contained in this prospectus. In addition, even if our results of operations, financial condition, business and prospects are consistent with the forward-looking statements contained in this prospectus, those results may not be indicative of results in subsequent periods.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including our consolidated financial statements and related notes included elsewhere in this prospectus, before making an investment decision. If any of the following risks are realized, our business, financial condition, results of operations and prospects would likely be materially and adversely affected. In that event, the trading price of our common stock could decline and you could lose part or all of your investment.

Risks related to our financial position and need for additional capital

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

Since inception, we have incurred significant net losses. Our net losses were $6.7 million and $23.8 million for the years ended December 31, 2015 and 2016, respectively, and $38.7 million for the nine months ended September 30, 2017. As of September 30, 2017, we had an accumulated deficit of $109.8 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:

- conduct our clinical trials of SGT-001;
- continue research and preclinical development of our other product candidates;
- seek to identify additional product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- arrange for manufacture of larger quantities of our product candidates for clinical development and potential commercialization;
- maintain, expand, protect and enforce our intellectual property portfolio;
- hire and retain additional clinical, quality control and scientific personnel;
- build out new facilities or expand existing facilities to support our 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 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.

Even if this offering is successful, 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, continue clinical trials for, 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. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. While we believe that the net proceeds from this offering and our existing cash, cash equivalents and available-for-sale securities will be sufficient to fund our current operating plans through at least the next 12 months, 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 our current 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;
- the costs associated with constructing and validating our own manufacturing facility;
- 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 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, your ownership interest will be diluted and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. 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 current good manufacturing practices, or 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 market 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 you 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 you make about our prospects may not be as accurate as they could be if we had a longer operating history.

Our auditors have expressed substantial doubt about our ability to continue as a going concern, which may hinder our ability to obtain further financing.

Our recurring losses from operations raise substantial doubt about our ability to continue as a going concern. As a result, our independent registered public accounting firm included an explanatory paragraph in its report on our consolidated financial statements for the year ended December 31, 2016 with respect to this uncertainty. Our ability to continue as a going concern will require us to obtain additional funding. We believe that the net proceeds from this offering and our existing cash, cash equivalents and available-for-sale securities will be sufficient to fund our current operating plans through at least the next 12 months. We have based these estimates, however, on assumptions that may prove to be wrong, and we could spend our available financial resources much faster than we currently expect and need to raise additional funds sooner than we anticipate. If we are unable to raise capital when needed or on acceptable terms, we would be forced to delay, reduce or eliminate our research and development programs and commercialization efforts.

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 may experience problems or delays in developing SGT-001. Any such problems or delays would cause unanticipated costs, and any development problems may not be solved. For example, we or another party may uncover a previously unknown risk associated with SGT-001, the 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.

The FDA placed the SGT-001 Phase I/II clinical trial on partial clinical hold requiring us to submit additional CMC information that demonstrates that manufacturing capacity and product attributes can support the high-dose group.

Our IND permits us to proceed with administering our proposed low dose to patients. Prior to dosing patients in our higher-dose group, we will be required to resolve the partial clinical hold on SGT-001 outlined in

a November 2017 letter to us from the FDA. In order to do so we will need to decrease the number of vials and utilize no more than a single production lot per patient and demonstrate that we have the appropriate manufacturing processes in place to support the higher-dose group. In addition, the FDA had additional comments and requests for information that were characterized as not clinical hold comments. We expect that we will be able to address the specific deficiencies identified by the FDA by submitting additional information demonstrating manufacturing capacity and product attributes that will support the high-dose group. The Company intends to submit a response to the FDA addressing the specific deficiencies in the near future, after which the FDA will have 30 days to respond. The Company does not expect that the overall timing for clinical development of SGT-001 will be affected by the partial clinical hold. Further, the partial clinical hold does not impact the Company's ability to conduct its clinical development activities of SGT-001 at low-dose levels. If the partial clinical hold is not lifted on our Phase I/II clinical trial, we will not be able to evaluate the safety, tolerability and efficacy of SGT-001 at the high-dose level, which could negatively impact the development of SGT-001. If the FDA does not permit us to administer SGT-001 at our proposed higher dose in our Phase I/II clinical trial, we may be unable to continue or complete our clinical trial of SGT-001. Any inability to continue or complete our clinical trial of SGT-001, as a result of the partial 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. 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 studies. 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 deoxyribonucleic acid, or 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 in the future we are unable to demonstrate that any such adverse events 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 have 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 have 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. The FDA placed SGT-001 on a partial clinical hold that prohibits us from administering SGT-001 at our proposed high dose. If the partial clinical hold is not lifted on our Phase I/II clinical trial, we will not be able to evaluate the safety, tolerability and efficacy of SGT-001 in the high-dose group, which could negatively impact the development of SGT-001. We cannot be sure that submission of an IND, will result in the FDA allowing clinical studies to begin or that, once begun, issues will not arise that suspend or terminate such studies. 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 Phase I/II trial, may not be indicative of results obtained in later trials.

Results from preclinical studies or early clinical trials, including our recently initiated Phase I/II 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 SGT-001 has not been tested in humans. 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 successfully addressing the specific deficiencies raised by the FDA with respect to the partial clinical hold placed on our Phase I/II clinical trial for SGT-001;
- delays in reaching agreement on acceptable terms with prospective contract research organizations, or CROs, and clinical trial sites;
- delays in opening clinical trial sites or obtaining required institutional review board, or 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 GCP, 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. Significant preclinical or clinical study or 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. 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 candidate 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 studies because of negative publicity from adverse events related to 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 studies 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;
- 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.

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 and cell therapy products have changed frequently and may continue to change in the future.

The FDA has established the Office of Tissues and Advanced Therapies, or OTAT, within its Center for Biologics Evaluation and Research, or 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 United States National Institutes of Health, or the NIH, also are potentially subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC; however, the NIH recently announced that 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 SGT-001 and our other product candidates, 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.

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 you 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.

The FDA has granted 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.

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.

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 intend 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 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 will require substantial additional investment, will 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 intend to establish our own manufacturing facility to support a commercial launch, if we are unable 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, and perform extensive audits of contract laboratories, manufacturers and suppliers.

We currently rely on a third-party manufacturer for our SGT-001 supply, and our agreement with that manufacturer expires at the end of 2018. We do not currently have a backup manufacturer for SGT-001 supply for clinical trials, and have not selected a manufacturer or backup manufacturer for SGT-001 supply for commercial sale. In order to produce sufficient quantities of SGT-001 for future clinical trials and initial U.S. commercial demand, we will need to increase the scale of our manufacturing process at our third-party manufacturers, as well as through our own planned commercial-scale manufacturing facility. We may not be able to enter into arrangements with additional third-party manufacturers on favorable terms or at all. 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. If supply from a manufacturing facility is interrupted, there could be a significant disruption in commercial supply of SGT-001 or our other 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 intend to 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 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 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 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 preclinical studies required to support future IND submissions and 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, 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 our 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 contract manufacturing organizations, or CMOs, to produce our product candidates in the necessary quantities, or in compliance with current cGMP or in compliance with pertinent regulatory requirements and within our planned time frame and cost parameters;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers and 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, 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 treatment outcomes for these patients.

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 treatment outcomes of these patients. While we are working to better understand 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 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 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 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 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, 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 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; 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.

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

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

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

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;
- the diversion of our management’s attention from our existing product candidates and initiatives in pursuing such acquisition or strategic collaboration;

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

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

Risks related to our business operations

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

We are highly dependent on members of our executive team, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with certain of our executive officers, any of them could leave our employment at any time. We currently do not have “key person” insurance on any of our employees. The loss of the services of one or more of our current 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, 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 in the United States of the Patient Protection and Affordable Care Act and the companion Health Care and Education Reconciliation Act in 2010, 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.

The President and the majorities of both houses of Congress have stated their intention to repeal and replace the Health Care Reform Law although recent efforts to do so have failed. The uncertain status of the Health Care Reform Law ability to may have a negative impact on our business.

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 Physician Payments 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 ACA 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. While we are not aware of any such material system failure, accident 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 "Business—Strategic partnerships and collaborations/licenses."

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 two 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 is 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 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.

The USPTO issued a guidance memorandum to patent examiners entitled 2014 Procedure For Subject Matter Eligibility Analysis Of Claims Reciting Or Involving Laws Of Nature/Natural Principles, Natural Phenomena, And/Or Natural Products. These guidelines instruct USPTO examiners on the ramifications of the Prometheus and Myriad rulings and apply the Myriad ruling 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 2014 USPTO guidance could impact our ability to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you 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 this offering and ownership of our common stock

After this offering, our executive officers, directors and principal stockholders will maintain the ability to control all matters submitted to our stockholders for approval.

After giving effect to the sale by us of shares of common stock in this offering, our executive officers, directors and stockholders who owned more than 5% of our outstanding common stock before this offering will, in the aggregate, beneficially own shares representing approximately 68.1% of our capital stock upon completion of this offering. 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 portion of our total outstanding shares are restricted from immediate resale but 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, subject to certain restrictions described below. 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. After this offering, we will have outstanding 34,151,022 shares of common stock based on the number of shares outstanding as of September 30, 2017, after giving effect to the Series 2 Preferred Financing and the Corporate Conversion. This includes the shares that we are selling in this offering, which may be resold in the public market immediately without restriction, unless purchased by our affiliates. The remaining 26,338,522 shares are currently restricted as a result of securities laws or lock-up agreements but will be able to be sold after the offering as described in the "Shares eligible for future sale" and "Underwriting" sections of this prospectus. Moreover, after this offering, holders of an aggregate of approximately 24.2 million shares of our common stock will have rights, subject to some 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, approximately 5.0 million shares reserved for future issuance under our 2018 Plan will become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. We intend to register all shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates and the lock-up agreements described in the "Underwriting" section of this prospectus.

In addition, J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC may, in their sole discretion, release all or some portion of the shares subject to lock-up agreements at any time and for any reason. Sales of a substantial number of such shares upon expiration of the lock-up agreements, the perception that such sales may occur, or early release of these agreements, could cause our market price to fall or make it more difficult for you to sell your common stock at a time and price that you deem appropriate.

If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.

The initial public offering price of our common stock will be substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. Based on the initial public offering price of $16.00 per share, you will experience immediate dilution of $10.39 per share, representing the difference between our pro forma net tangible book value per share after giving effect to this offering at the initial public offering price. In addition, purchasers of common stock in this offering will have contributed approximately 46% of the aggregate price paid by all purchasers of our stock but will own only approximately 23% of our common stock outstanding after this offering. See "Dilution."

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for purchasers of our common stock in this offering.

Our stock price is likely to be volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, you may not be able to sell your common stock at or above the initial public offering price. 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. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

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. Such litigation, if 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 will rely, in part, on the research and reports that industry or financial analysts publish about us or our business. We do not currently have, and may never obtain, research coverage by industry or financial analysts. If no, or few, analysts commence coverage of us, the trading price of our stock would likely decrease. Even if we do obtain analyst coverage, 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 develop.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock will be determined through negotiations with the underwriters. Although we have been approved to have our common stock listed on the NASDAQ Global Select Market, an active trading market for our shares may never develop or be sustained following this offering. If an active market for our common stock does not develop, it may be difficult for you to sell shares you purchase in this offering 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 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) the last day of the fiscal year following the fifth anniversary of the date of the completion of this offering; (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 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 prospectus. In particular, we have not included all of the executive compensation information that would be required if we were not an EGC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

As a public company, and particularly after we are no longer an EGC, we will 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 will need to 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 you 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 corporate charter 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 corporate charter and our bylaws that will become effective prior to the effectiveness of the registration statement of which this prospectus forms a part may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your 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 charter 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, will be your sole source of gain.

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 your sole source of gain for the foreseeable future.

Our charter will provide 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 charter that we expect it to be in effect prior to the effectiveness of the registration statement of which this prospectus forms a part will provide 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 charter 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 charter to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

USE OF PROCEEDS

We expect to receive net proceeds from this offering of approximately $112.5 million, or approximately $129.9 million if the underwriters exercise their option to purchase additional shares in full, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

We estimate that as of December 31, 2017, our cash, cash equivalents and available-for-sale securities was approximately $69.0 million. We intend to use the net proceeds from this offering, together with our existing cash, cash equivalents and available-for-securities, as follows:

- approximately $150.0 million to fund research and development expenses, including to advance SGT-001 through preliminary results from Phase I/II clinical trial activities, which we initiated in the fourth quarter of 2017; and
- the remainder for general and administrative expenses and other general corporate purposes.

Our independent registered public accountants have not audited, reviewed or performed any procedures with respect to our cash, cash equivalents and available-for-sale securities as of December 31, 2017 and accordingly do not express an opinion or any other form of assurance with respect thereto. This financial data reflects the best information available to management as of the date of this prospectus and could change as a result of our financial close process and subsequent review and audit by our independent registered public accountants.

Based on our current operational plans and assumptions, we expect that the net proceeds from this offering, combined with our current cash, cash equivalents and available-for-sale securities, will be sufficient to fund operations through the fourth quarter of 2019 and enable us to advance SGT-001 through preliminary results from our planned Phase I/II clinical trial activities, which we initiated in the fourth quarter of 2017. We will need to raise additional capital in order to complete the Phase I/II clinical trials and any potential future trials that may be required by regulatory authorities.

Our expected use of net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with complete certainty all of the particular uses for the net proceeds to be received upon the completion of this offering or the actual amounts that we will spend on the uses set forth above. We believe opportunities may exist from time to time to expand our current business through the acquisition or in-license of complementary product candidates. While we have no current agreements for any specific acquisitions or in-licenses at this time, we may use a portion of the net proceeds for these purposes.

The amounts and timing of our actual expenditures will depend on numerous factors, including the progress of our clinical trials and other development and commercialization efforts for SGT-001, as well as the amount of cash used in our operations. We therefore cannot estimate with certainty the amount of net proceeds to be used for the purposes described above. We may find it necessary or advisable to use the net proceeds for other purposes, and we will have broad discretion in the application of the net proceeds. Pending the uses described above, we plan to invest the net proceeds from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

DIVIDEND POLICY

We have not declared or paid any cash dividends on our capital stock since our inception. We intend to retain future earnings, if any, to finance the operation and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future.

CORPORATE CONVERSION

We currently operate as a Delaware limited liability company under the name Solid Biosciences, LLC. Prior to the effectiveness of the registration statement of which this prospectus forms a part, Solid Biosciences, LLC will convert into a Delaware corporation pursuant to a statutory conversion and change its name to Solid Biosciences Inc. In addition, special purpose entities formed solely for the purpose of holding membership interests in our limited liability company will be merged with and into us. In this prospectus, we refer to all of the transactions related to our conversion to a corporation and the mergers described above as the Corporate Conversion.

In conjunction with the Corporate Conversion, all of our outstanding units will be converted into an aggregate of 26,498,559 shares of our common stock (which includes 1,132,425 shares of restricted stock). The number of shares of common stock and the number of shares of restricted stock issuable in connection with the Corporate Conversion will be determined pursuant to the applicable provisions of the plan of conversion.

In connection with the Corporate Conversion, Solid Biosciences Inc. will continue to hold all property and assets of Solid Biosciences, LLC and will assume all of the debts and obligations of Solid Biosciences, LLC. Solid Biosciences Inc. will be governed by a certificate of incorporation filed with the Delaware Secretary of State and bylaws, the material portions of which are described under the heading “Description of capital stock.” On the effective date of the Corporate Conversion, the members of the board of managers of Solid Biosciences, LLC will become the members of Solid Biosciences Inc.’s board of directors and the officers of Solid Biosciences, LLC will become the officers of Solid Biosciences Inc.

The purpose of the Corporate Conversion is to reorganize our corporate structure so that the top-tier entity in our corporate structure—the entity that is offering common stock to the public in this offering—is a corporation rather than a limited liability company and so that our existing investors will own our common stock rather than membership units in a limited liability company.

Except as otherwise noted herein, the consolidated financial statements included elsewhere in this prospectus are those of Solid Biosciences, LLC and its combined operations. We do not expect that the Corporate Conversion will have a material effect on the results of our core operations.

CASH AND CAPITALIZATION

The following table describes our cash, cash equivalents and available-for-sale securities and capitalization as of September 30, 2017:

- on an actual basis;
- on a pro forma basis to give effect to the Corporate Conversion and the Series 2 Preferred Financing; and
- on a pro forma as adjusted basis to additionally give effect to the sale of 7,812,500 shares of our common stock in this offering, at the initial public offering price of $16.00 per share, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

You should read the following information together with the information contained under the headings “Selected consolidated financial data” and “Management’s discussion and analysis of financial condition and results of operations” and our consolidated financial statements and the related notes appearing at the end of this prospectus.

	As of September 30, 2017		
(in thousands, except share and per share data)	**Actual**	**Pro forma (1)(2)**	**Pro forma as adjusted (1)**
Cash, cash equivalents and available-for-sale securities	$ 29,570	$ 84,570	$ 197,020
Redeemable preferred units	69,177	—	—
Members’ deficit:			
Series A, B, C and D common units	64,191	—	—
Accumulated other comprehensive loss	(3)	—	—
Accumulated members’ deficit	(109,771)	—	—
Total members’ deficit	(45,583)	—	—
Stockholders’ equity:			
Preferred stock, $0.001 par value; no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized and no shares issued or outstanding, pro forma and pro forma as adjusted	—	—	—
Common stock, $0.001 par value; no shares authorized, issued or outstanding, actual; 300,000,000 shares authorized, 26,338,522 shares issued and outstanding, pro forma; 300,000,000 shares authorized, 34,151,022 shares issued and outstanding, pro forma as adjusted	—	26	34
Additional paid-in capital	—	188,342	300,784
Accumulated deficit	—	(109,244)	(109,244)
Accumulated other comprehensive loss	—	(3)	(3)
Total stockholders’ equity	—	79,121	191,571
Total capitalization	$ 23,594	$ 79,121	$ 191,571

(1) In connection with the Corporate Conversion, preferred units, Series A, B, C and D common units and members’ accumulated deficit will be reduced to zero to reflect the elimination of all outstanding units and other interests in Solid Biosciences, LLC and corresponding adjustments will be reflected as common stock, additional paid-in capital, stockholders’ accumulated deficit, stockholders’ accumulated other comprehensive loss and total stockholders’ equity of Solid Biosciences Inc. The pro forma and pro forma as adjusted information is illustrative only.

(2) The following table sets forth the number of shares of common stock and restricted common stock that will be issued in connection with the Corporate Conversion and the consummation of this offering to holders of our Series A, B, C, and D common units:

Shares of common stock to be issued for:	
Series A common units	10,368,069
Series B vested common units	2,073,615
Series C common units	1,388,064
Series D vested common units	719,816
Shares of restricted common stock to be issued for:	
Series B unvested common units	691,204
Series D unvested common units	1,085,173
Total	16,325,941

In addition to the common stock and restricted common stock that will be issued in connection with the Corporate Conversion as indicated above, we will also issue 10,012,581 shares of common stock to holders of our preferred units.

DILUTION

If you invest in our common stock, your ownership interest will be diluted to the extent of the difference between the initial public offering price in this offering per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock upon consummation of this offering. Net tangible book value per share represents the book value of our total tangible assets less the book value of our total liabilities divided by the number of shares of common stock then issued and outstanding.

After giving effect to the Series 2 Preferred Financing and the Corporate Conversion, pro forma net tangible book value as of September 30, 2017 was $76.9 million, or $2.92 per share based on 26,338,522 shares of our common stock outstanding. After giving effect to our sale of 7,812,500 shares of common stock in this offering, at the initial public offering price of $16.00 per share, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of September 30, 2017 would have been $191.6 million, or $5.61 per share (assuming no exercise of the underwriters' option to purchase additional shares of our common stock). This represents an immediate and substantial dilution of $10.39 per share to new investors purchasing common stock in this offering. The following table illustrates this dilution per share:

Assumed initial public offering price per share		$16.00
Pro forma net tangible book value per share as of September 30, 2017	$2.92	
Increase in pro forma net tangible book value per share attributable to this offering	$2.69	
Pro forma as adjusted net tangible book value per share after giving effect to this offering		$ 5.61
Dilution per share to new investors in this offering		$10.39

If the underwriters fully exercise their option to purchase 1,171,875 additional shares of common stock in this offering, our pro forma as adjusted net tangible book value per share after this offering would be $5.92 and the dilution in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering would be $10.08, assuming no change in the initial public offering price per share and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us.

The following table summarizes, on a pro forma as adjusted basis as of September 30, 2017, the differences between the number of shares of common stock purchased from us, the total consideration paid and the average price per share paid by existing stockholders and to be paid by the new investors purchasing shares of common stock in this offering, at the initial public offering price of $16.00 per share, before deducting the underwriting discounts and commissions and estimated offering expenses payable by us in connection with this offering.

	Shares purchased		Total consideration		Average price per share
	Number	Percent	Amount	Percent	
Existing investors	26,338,522	77%	$144,600,000	54%	$ 5.49
New investors in this offering	7,812,500	23%	$125,000,000	46%	$16.00
Total	34,151,022	100%	$269,600,000	100%	

The table above assumes no exercise of the underwriters' option to purchase additional shares in this offering. If the underwriters' option to purchase additional shares is fully exercised, the number of shares of our common stock held by existing stockholders would be reduced to 74.6% of the total number of shares of our common stock outstanding after this offering, and the number of shares of common stock held by new investors purchasing common stock in this offering would be increased to 25.4% of the total number of shares of our common stock outstanding after this offering.

The table above is based on no shares of common stock outstanding as of September 30, 2017 and gives effect to the Corporate Conversion and the Senior 2 Preferred Financing.

We expect to require additional capital to fund our current and future operating plans. To the extent additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities could result in further dilution to our stockholders. See “Risk factors—Risks related to this offering and ownership of our common stock—If you purchase shares of common stock in this offering, you will suffer immediate dilution of your investment.”

SELECTED CONSOLIDATED FINANCIAL DATA

You should read the following selected consolidated financial data together with our consolidated financial statements and the related notes appearing at the end of this prospectus and the "Cash and capitalization" and "Management's discussion and analysis of financial condition and results of operations" sections of this prospectus. We have derived the consolidated statements of operations data for the years ended December 31, 2015 and 2016 and the consolidated balance sheet data as of December 31, 2015 and 2016 from our audited consolidated financial statements appearing at the end of this prospectus. The consolidated statements of operations data for the nine months ended September 30, 2016 and 2017 and the consolidated balance sheet data as of September 30, 2017 have been derived from our unaudited consolidated financial statements appearing at the end of this prospectus and have been prepared on the same basis as the audited financial statements. In the opinion of management, the unaudited consolidated financial statements reflect all adjustments, consisting only of normal recurring adjustments, necessary for a fair statement of the financial information in those consolidated statements. Our historical results are not necessarily indicative of results that should be expected in any future period, and our results for any interim period are not necessarily indicative of results that should be expected for any full year.

	Year ended December 31,		Nine months ended September 30,	
(in thousands, except units and per unit data)	2015	2016	2016	2017
Consolidated statements of operations data:				
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	4,192	20,116	13,048	27,959
General and administrative	2,372	5,460	3,807	11,737
Total operating expenses	6,564	25,576	16,855	39,696
Loss from operations	(6,564)	(25,576)	(16,855)	(39,696)
Other income (expense):				
Revaluation of preferred unit tranche rights	(103)	1,163	1,163	(68)
Interest and other income	3	640	438	1,073
Total other income (expense), net	(100)	1,803	1,601	1,005
Net loss	$ (6,664)	$ (23,773)	$ (15,254)	$ (38,691)
Net loss attributable to Solid Biosciences, LLC	$ (6,377)	$ (21,539)	$ (13,783)	$ (37,631)
Net loss attributable to common unitholders	$ (6,445)	$ (17,230)	$ (12,585)	$ (24,830)
Net loss per unit attributable to common unitholders, basic and diluted (1)	$ (7.61)	$ (10.14)	$ (7.50)	$ (1.99)
Weighted average common units outstanding, basic and diluted (1)	846,569	1,698,904	1,677,909	12,446,769

	As of December 31,		As of September 30,
(in thousands)	2015	2016	2017
Consolidated balance sheet data:			
Cash, cash equivalents and available-for-sale securities	$ 55,387	$ 37,658	$ 29,570
Working capital	41,772	33,099	18,966
Total assets	55,696	40,636	35,445
Redeemable preferred units	61,697	71,649	69,177
Accumulated members' deficit	(67,711)	(84,941)	(109,771)
Total deficit	(19,925)	(37,886)	(45,583)

(1) See Note 15 to our financial statements appearing at the end of this prospectus for details on the calculation of basic and diluted net loss per unit attributable to common unitholders.

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and consolidated results of operations together with the "Selected consolidated financial data" section of this prospectus and our consolidated financial statements and the related notes included at the end of this prospectus. This discussion and other parts of this prospectus contain forward-looking statements that involve risks and uncertainties, such as statements of our plans, objectives, expectations and intentions. As a result of many factors, including those factors set forth in the "Risk factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

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.

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 have incurred significant losses every year since our inception. We do not have any products approved for sale. To date, we have not generated any revenue. 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. 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.

Due to our significant research and development expenditures, licensing and patenting investment, and general and administrative costs associated with our operations, we have generated substantial operating losses in each period since inception. Our net losses were $6.7 million and $23.8 million for the years ended December 31, 2015 and 2016, respectively, and were $15.3 million and $38.7 million for the nine months ended September 30, 2016 and 2017, respectively. As of September 30, 2017, we had an accumulated deficit of $109.8 million.

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.

In its report on our consolidated financial statements for the year ended December 31, 2016, our independent registered public accounting firm included an explanatory paragraph stating that our recurring losses from operations since inception and required additional funding to finance our operations raise substantial doubt about our ability to continue as a going concern.

On October 26, 2017, we completed the sale of 4,886,000 Series 2 Senior Preferred Units at a price of $11.26 per unit in exchange for net proceeds of $55.0 million.

As of September 30, 2017, we had cash, cash equivalents and available-for-sale securities of $29.6 million. We believe that the anticipated net proceeds from this offering, together with our existing cash, cash equivalents and available-for-sale securities as well as the proceeds from the sale of the Series 2 Senior Preferred Units in October 2017, will enable us to fund our operating expenses and capital expenditure requirements through at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See “—Liquidity and capital resources.”

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 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 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.

Corporate conversion

We currently operate as a Delaware limited liability company, under the name Solid Biosciences, LLC. Prior to the effectiveness of the registration statement of which this prospectus forms a part, Solid Biosciences, LLC will convert into a Delaware corporation pursuant to a statutory conversion and change its name to Solid Biosciences Inc. In addition, entities formed solely for the purpose of holding membership interests in our limited liability company will be merged with and into us. 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 will become holders of common stock of Solid Biosciences Inc.

The purpose of the Corporate Conversion is to reorganize our structure so that the entity that is offering our common stock to the public in this offering is a corporation rather than a limited liability company and so that

our existing investors will own our common stock rather than equity interests in a limited liability company. For further information regarding the Corporate Conversion, see “Corporate conversion.” References in this prospectus to our capitalization and other matters pertaining to our equity and shares prior to the Corporate Conversion relate to the capitalization and equity and shares of Solid Biosciences, LLC, and after the Corporate Conversion, to Solid Biosciences Inc.

The consolidated financial statements included elsewhere in this prospectus are those of Solid Biosciences, LLC and its subsidiaries. We do not expect that the Corporate Conversion will have a material effect on the results of our core operations.

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 CROs, that conduct research and preclinical activities on our behalf as well as 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 costs and 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 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:

	Year ended December 31,		Nine months ended September 30,	
	2015	2016	2016	2017
SGT-001	$1,940	$13,891	$ 8,281	$17,508
Other product candidates	233	1,021	490	1,167
Unallocated research and development expenses	2,019	5,204	4,277	9,284
Total research and development expenses	$4,192	$20,116	$13,048	$27,959

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;
- 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)

Revaluation of preferred unit tranche rights

Included in the terms of the Redeemable Preferred Unit Purchase Agreement was a right, which we refer to as the Redeemable Preferred Tranche Right, granted to the holders of the Redeemable Preferred Units issued in December 2013. The Redeemable Preferred Tranche Right obligates the holders to purchase, and provides the holders with the right to purchase, additional redeemable preferred units under certain circumstances. The Redeemable Preferred Tranche Right was transferrable by the investors.

The terms of the Series 1 Senior Preferred Unit Purchase Agreement, as amended on September 1, 2017, also contained a right, which we refer to as the Series 1 Tranche Right. The Series 1 Tranche Right obligates the holders of the Series 1 Senior Preferred Units to purchase 1,973,430 Series 2 Senior Preferred Units at a purchase price of $12.67 per unit in the event the Company achieves certain preclinical milestones. In addition, the holders of a majority of the Series 1 Senior Preferred Units have the right to require the holders of the Series 1 Senior Preferred Units to purchase the Series 2 Senior Preferred Units at any time prior to December 1, 2017. The Series 1 Tranche Right is subject to certain transfer rights.

We concluded that the Redeemable Preferred Tranche Right and the Series 1 Tranche Right, together the Tranche Rights, met the definition of a freestanding financial instrument as the Tranche Rights were legally detachable and separately exercisable from the Redeemable Preferred Units and the Series 1 Senior Preferred Units. Therefore, we allocated the net proceeds between the Tranche Rights and the Redeemable Preferred Units or the Series 1 Senior Preferred Units. The Tranche Rights were initially recorded at fair value and are re-measured at fair value each reporting period. Changes in the fair market value are recognized as a component of other income (expense), net, in the consolidated statements of operations.

In October 2016, the Redeemable Preferred Tranche Right was settled with the closing of the Redeemable Preferred Unit financing. In October 2017, the Series 1 Tranche Right was settled in connection with the closing of the Series 2 Senior Preferred Financing.

Interest income

Interest income consists of interest income earned on our cash, cash equivalents and available-for-sale securities. Our interest income has not been significant due to low investment balances and low interest earned on those balances.

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 consolidated statements of operations.

Income taxes

Since our inception in 2013, we have 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 are not viewed as a taxpaying entity in any jurisdiction and do not require a provision for income taxes. Each member of our company is responsible for the tax liability, if any, related to its proportionate share of our taxable income.

After consummation of this offering, we will be treated as a corporation for U.S. income tax purposes and thus will become subject to U.S. federal, state and local income taxes and will be taxed at the prevailing corporate tax rates. Among other things, we may begin to generate net operating losses at the corporate level. We will account for income taxes using an asset and liability approach, which requires recognition of deferred tax assets and liabilities for the expected future tax consequences of events that have been recognized in the consolidated financial statements, but have not been reflected in taxable income. A valuation allowance is established to reduce deferred tax assets to their estimated realizable value.

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

Critical accounting policies and use of estimates

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

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing at the end of this prospectus, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Accrued research and development expenses

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

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

We base our expenses related to preclinical studies on our estimates of the services received and efforts expended pursuant to quotes and contracts with multiple CROs that conduct and manage preclinical studies and clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our vendors will exceed the level of services provided and result in a prepayment of the expense. In accruing fees, we estimate the time period over which services will be performed, and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or amount of prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, our understanding of the status and timing of services performed relative to the actual status and timing of services performed may vary and may result in us reporting amounts that are too high or too low in any particular period. To date, we have not made any material adjustments to our prior estimates of accrued research and development expenses.

Tranche Rights

We measure the fair value of the Tranche Rights based on the fair value of the tranche rights at inception and remeasure their fair value at each reporting date until settled. Changes in the fair market value are recognized as a component of other income (expense), net in the consolidated statement of operations. As there has been no public market for our preferred units, the estimated fair value of our preferred units has been determined from our most recently available third-party valuations of preferred units. These third-party valuations were performed in accordance with the guidance outlined in the American Institute of Certified Public Accountants' Accounting and Valuation Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, also known as the Practice Aid. Our preferred unit valuations were prepared using a market approach based on the most recent round of equity financing and an option-pricing method, or OPM, with the exception of the December 6, 2016 valuation, which was performed using the hybrid method and the expected probability of closing a financing round. The hybrid method was used in anticipation of an equity financing transaction, which had not closed as of the valuation date. The OPM treats preferred units and common units as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. The hybrid method is a probability-weighed expected return method, or PWERM, where the equity value in one or more scenarios is calculated using OPM. The PWERM is a scenario-based methodology that estimates the fair value of preferred units based upon an analysis of future values for the company, assuming various outcomes. The preferred unit value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of units. The values of the preferred units under each outcome is probability weighted to arrive at an indication of value for the common units. The OPM and hybrid methods were selected to properly account for the limited liability company structure.

Equity-based compensation

Certain of our employees and consultants have received grants of common units in our company. These awards are accounted for in accordance with guidance prescribed for accounting for equity-based compensation. Based on this guidance and the terms of the awards, the awards are equity classified. The common units receive distributions only if a threshold, that is equivalent to the overall value of our company on the grant date, is exceeded. The threshold impacts the fair value of our common units because as the overall value of our company increases, common units with a lower threshold have a higher per unit fair value than common units subject to higher thresholds because proceeds are distributed in an order of priority in accordance with our limited liability company agreement.

Under the terms of our limited liability company agreement, upon conversion to a corporation, holders of our preferred units would be contractually entitled to receive the number of shares of common stock in the converted corporation that equals the value of the units that such holders held in our company immediately prior to the conversion. Therefore, if the equity value of our company has not reached a specific threshold that would

allow the holders of preferred units to receive their full value, such holders, pursuant to the terms of our limited liability company agreement, would be entitled to receive more shares of common stock upon a corporate conversion in order to them "whole." This contractual protection for the benefit of holders of our preferred units would result in the holders of our Series D Common Units receiving less value for their Series D Common Units in an initial public offering. For example, until such time as the equity value of our company has increased to reach the specified threshold that results in the Series D Common Unit holders having caught up to the value of the holders of our preferred units, Series D Common Unit holders will receive fewer shares of common stock in the converted corporation than originally issued, and certain Series D Common Unit holders with a higher specified threshold (due to receiving their units at a later grant date) may not receive any shares in an initial public offering.

We are a private company with no active public market for our common equity. Therefore, we have periodically determined the overall value of our company and the estimated per share fair value of our common equity at their various dates using contemporaneous valuations performed in accordance with the guidance outlined in the Practice Aid. Once a public trading market for our common stock has been established in connection with the completion of this offering, it will no longer be necessary for us to estimate the fair value of our common stock in connection with our accounting for equity awards we may grant, as the fair value of our common stock will be its public market trading price.

For financial reporting purposes, we performed common unit valuations with the assistance of a third-party specialist, for the years ended December 31, 2014, 2015 and 2016 and for each quarter in the period from January 1, 2016 through September 30, 2017.

Our common unit valuations were prepared using a market approach based on the most recent round of equity financing and an OPM, with the exception of the December 6, 2016 valuation, which was performed using the hybrid method and the expected probability of closing a financing round. The hybrid method was used in anticipation of an anticipated equity financing transaction, which had not closed as of the valuation date. The OPM treats common units and preferred units as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common unit has value only if the funds available for distribution to stockholders exceeded the value of the preferred stock liquidation preference at the time of the liquidity event, such as a strategic sale, merger or public offering. The hybrid method is a PWERM where the equity value in one or more scenarios is calculated using an OPM. The PWERM is a scenario-based methodology that estimates the fair value of common units based upon an analysis of future values for the company, assuming various outcomes. The common unit value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of units. The values of the common unit under each outcome is probability weighted to arrive at an indication of value for the common unit. The OPM and hybrid methods were selected to properly account for the limited liability company structure.

In connection with the preparation of valuations of our common units, our management and valuation specialists collectively used various objective and subjective factors to determine the fair value of our common unit as of each grant date, including:

- the prices at which we sold preferred units and the superior rights and preferences of the preferred units relative to our common units at the time of each grant;
- the progress of our research and development programs, including the status and results of preclinical studies for our product candidates;
- our stage of development and commercialization and our business strategy;
- external market conditions affecting the biopharmaceutical industry and trends within the biopharmaceutical industry;
- our financial position, including cash on hand, and our historical and forecasted performance and operating results;

- the lack of an active public market for our common units and preferred units;
- the likelihood of achieving a liquidity event, such as an initial public offering or sale of our company in light of prevailing market conditions; and
- the analysis of initial public offerings and the market performance of similar companies in the biopharmaceutical industry.

The assumptions underlying these valuations represented management's best estimate, which involved inherent uncertainties and the application of management's judgment. As a result, if we had used significantly different assumptions or estimates, the fair value of our common units and our equity-based compensation expense could have been materially different.

Results of operations

Comparison of the nine months ended September 30, 2016 and 2017

The following table summarizes our results of operations for the nine months ended September 30, 2016 and 2017:

	Nine months ended September 30,		Increase (decrease)
(in thousands)	**2016**	**2017**	
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	13,048	27,959	14,911
General and administrative	3,807	11,737	7,930
Total operating expenses	16,855	39,696	22,841
Loss from operations	(16,855)	(39,696)	(22,841)
Other income (expense):			
Revaluation of preferred unit tranche rights	1,163	(68)	(1,231)
Interest income	270	165	(105)
Other income	168	908	740
Total other income (expense)	1,601	1,005	(596)
Net loss	$(15,254)	$(38,691)	$(23,437)

Research and development expenses

	Nine months ended September 30,		Increase (decrease)
(in thousands)	**2016**	**2017**	
SGT-001	$ 8,281	$17,508	$ 9,227
Other product candidates	490	1,167	677
Unallocated research and development expenses	4,277	9,284	5,007
Total research and development expenses	$13,048	$27,959	$ 14,911

Research and development expenses for the nine months ended September 30, 2016 were $13.0 million, compared to $27.9 million for the nine months ended September 30, 2017. The increase of $14.9 million in

research and development costs was due to a $9.2 million increase in preclinical research and manufacturing costs related to our lead product candidate SGT-001, $0.7 million increase in costs related to our other product candidates and $5.0 million increase in unallocated research and development costs due primarily to increased compensation and headcount.

General and administrative expenses

General and administrative expenses were $3.8 million for the nine months ended September 30, 2016, compared to $11.7 million for the nine months ended September 30, 2017. The increase of $7.9 million was primarily due to in an increase in equity-based compensation of $3.6 million, an increase of $2.4 million in professional fees related to preparation for this offering, an increase of $1.5 million in personnel-related expenses and an increase of $0.4 million of other corporate expenses. The increase in equity-based compensation of $3.6 million during the nine months ended September 30, 2017 was primarily due to a charge associated with the exchange of certain of our vested common units in connection with the recapitalization of our company and our merger with Solid GT on March 29, 2017.

Revaluation of preferred unit tranche rights

The revaluation of the Redeemable Preferred Tranche Right resulted in a gain of $1.2 million for the nine months ended September 30, 2016 due to a decrease in the fair value of the preferred units. The Redeemable Preferred Tranche Right expired in October 2016. We issued the Series 1 Tranche Right on March 29, 2017. The revaluation of the Series 1 Tranche Right resulted in a loss of $0.1 million due to an increase in the fair value of the Series 1 units.

Interest income

Interest remained consistent at $0.3 million and $0.2 million for the nine months ended September 30, 2016 and for the nine months ended September 30, 2017.

Other income

Other income for the nine months ended September 30, 2016 was $0.2 million compared to $0.9 million for the nine months ended September 30, 2017. The increase of $0.7 million was due to income from charitable organizations. We do not expect these contributions to significantly increase in future periods.

Comparison of the years ended December 31, 2015 and 2016

The following table summarizes our results of operations for the years ended December 31, 2015 and 2016:

(in thousands)	Year ended December 31, 2015	2016	Increase (decrease)
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	4,192	20,116	15,924
General and administrative	2,372	5,460	3,088
Total operating expenses	6,564	25,576	19,012
Loss from operations	(6,564)	(25,576)	(19,012)
Other income (expense):			
Revaluation of preferred unit tranche rights	(103)	1,163	1,266
Interest income	3	369	366
Other income	—	271	271
Total other income (expense)	(100)	1,803	1,903
Net loss	$(6,664)	$(23,773)	$(17,109)

Research and development expenses

(in thousands)	Year ended December 31, 2015	2016	Increase (decrease)
SGT-001	$1,940	$13,891	$ 11,951
Other product candidates	233	1,021	788
Unallocated research and development expenses	2,019	5,204	3,185
Total research and development expenses	$4,192	$20,116	$ 15,924

Research and development expenses for the year ended December 31, 2015 were $4.2 million, compared to $20.1 million for the year ended December 31, 2016. The increase of $15.9 million in research and development costs was due to a $12.0 million increase in preclinical research and manufacturing costs related to our lead product candidate, SGT-001, $0.8 million increase in costs related to our other product candidates due to increased discovery costs, and $3.2 million increase in unallocated research and development costs due primarily to increased compensation and headcount, the full year impact of employees hired in 2015 and an increase of $0.6 million in equity-based compensation.

General and administrative expenses

General and administrative expenses were $2.4 million for the year ended December 31, 2015, compared to $5.5 million for the year ended December 31, 2016. The increase of $3.1 million was due to an increase of $1.8 million in compensation and related costs due to increased headcount and new hires, $0.7 million in legal and accounting fees, $0.2 million in facilities costs due to new corporate and research space, and $0.4 million of other corporate-related costs. The increase in professional fees was due to increases in the use of accounting consultants and in legal fees.

Revaluation of preferred unit tranche rights

The revaluation of the Redeemable Preferred Tranche Right resulted in a loss of $0.1 million for the year ended December 31, 2015 compared to a gain of $1.2 million for the year ended December 31, 2016. The increase of $1.3 million was due to a decrease in the underlying preferred units during the year ended December 31, 2016. The Redeemable Preferred Tranche Right expired in October 2016.

Interest income

Interest income was less than $0.1 million for the year ended December 31, 2015, compared to $0.4 million for the year ended December 31, 2016. The increase of $0.3 million was due to increased cash, cash equivalents and available-for-sale securities for the year ended December 31, 2016 compared to the year ended December 31, 2015.

Other income

There was no other income for the year ended December 31, 2015 compared to $0.3 million for the year ended December 31, 2016. The increase of $0.3 million was due to income from charitable organizations. We do not expect these contributions to significantly increase.

Liquidity and capital resources

Sources of liquidity

To date, we have financed our operations primarily through private placements of preferred units. Through September 30, 2017, we raised an aggregate of $89.6 million of gross proceeds from our sales of preferred units, which includes $25.0 million from our sale of our Series 1 Senior Preferred Units on March 29, 2017. On October 26, 2017, we raised an additional $55.0 million from our sale of our Series 2 Senior Preferred Units in the Series 2 Senior Preferred Financing.

As of September 30, 2017, we had cash, cash equivalents and available-for-sale securities of $29.6 million and had no debt outstanding.

Cash flows

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

	Year ended December 31,		Nine months ended September 30,	
(in thousands)	2015	2016	2016	2017
Cash used in operating activities	$ (4,204)	$(20,120)	$(14,546)	$(29,242)
Cash provided by (used in) investing activities	(26,806)	(4,217)	(9,866)	13,260
Cash provided by financing activities	51,592	3,420	—	23,321
Net increase (decrease) in cash and cash equivalents	$ 20,582	$(20,917)	$(24,412)	$ 7,339

Operating activities. During the nine months ended September 30, 2017, operating activities used $29.2 million of cash, primarily resulting from our net loss of $38.7 million offset by non-cash charges of $5.0 million due primarily to equity-based compensation of $4.5 million, which included $3.4 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 $4.5 million. Net cash provided by changes in our operating assets and liabilities during the nine months ended September 30, 2017 consisted of a decrease in prepaid expenses and other currents assets of $1.1 million due to the timing of prepaid research and development expense payments and net increase in accounts payable and accrued expenses of $3.3 million due to the timing of payments and the increase in the overall activity of the company.

During the nine months ended September 30, 2016, operating activities used $14.5 million of cash, primarily resulting from our net loss of $15.3 million offset by net non-cash adjustments of $0.4 million, due primarily to equity-based compensation of $1.1 million and amortization of premiums on the company's available-for-sale securities of $0.4 million, partially offset by a $1.2 million gain on revaluation of our Redeemable Preferred Tranche Right, and net cash provided by changes in our operating assets and liabilities of $0.3 million during the nine months ended September 30, 2016. Net cash provided by changes in operating assets and liabilities consisted primarily of a $2.6 million increase in accounts payable, accrued expenses and other current liabilities, partially offset by a $2.2 million increase in prepaid expenses and other current assets. The increase in accounts payable, accrued expenses and other current liabilities was largely due to increased preclinical activities. The increase in prepaid expenses and other current assets was largely due to the payment of preclinical activities in advance of the related research and development.

During the year ended December 31, 2016, operating activities used $20.1 million of cash, primarily resulting from our net loss of $23.8 million offset by non-cash charges of $0.9 million and cash provided by changes in our operating assets and liabilities of $2.8 million. Non-cash charges of $0.9 million represented equity-based compensation expense of $1.5 million and amortization of premiums on available-for-sale securities of $0.5 million, offset by $1.1 million of gains on the revaluation of our Redeemable Preferred Tranche Right due to a decrease in the fair value of the underlying preferred units for the year ended December 31, 2016. Net cash provided by changes in our operating assets and liabilities during the year ended December 31, 2016 consisted of an increase of $4.8 million in accounts payable, accrued expenses and other current liabilities, partially offset by a $2.0 million increase in prepaid expenses and other current assets. The increase in accounts payable, accrued expenses and other current liabilities was largely due to an increase of preclinical trial-related expenses. The increase in prepaid expenses and other current assets was primarily due to the payment of preclinical activities in advance of the related research and development.

During the year ended December 31, 2015, operating activities used $4.2 million of cash, primarily resulting from our net loss of $6.7 million, partially offset by non-cash charges of $0.9 million due primarily to

$0.7 million of equity-based compensation expense, and cash provided by changes in our operating assets and liabilities of $1.6 million. Net cash provided by changes in our operating assets and liabilities during the year ended December 31, 2015 consisted of a $1.9 million increase in accounts payable, accrued expenses and other current liabilities, partially offset by a $0.3 million increase in prepaid expenses and other current assets. The increase in accounts payable, accrued expenses and other current liabilities was largely due to an increase of preclinical trial-related expenses. The increase in prepaid expenses and other current assets was primarily due to the payment of preclinical activities in advance of the related research and development.

Investing activities

During the nine months ended September 30, 2017, investing activities provided $13.3 million of cash, consisting primarily from the net proceeds on the sale and maturity of available-for-sale securities partially offset by purchases of property and equipment.

During the nine months ended September 30, 2016, investing activities used $9.9 million of cash, consisting primarily of net purchases of available-for-sale securities and to a lesser extent the acquisition of property and equipment.

During the year ended December 31, 2016, investing activities used $4.2 million of cash, consisting primarily of net purchases of investments and to a lesser extent the acquisition of property and equipment.

During the year ended December 31, 2015, investing activities used $26.8 million of cash, consisting primarily of net purchases of investments.

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

Financing activities

During the nine months ended September 30, 2017, net cash provided by financing activities was $23.3 million, primarily due to the proceeds from our sale of Series 1 Senior Preferred Units of $25.0 million partially offset by payments made in connection with our proposed initial public offering.

During the nine months ended September 30, 2016, there was no cash provided by or used in financing activities.

During the year ended December 31, 2016, net cash provided by financing activities was $3.4 million, due to the proceeds from our sale of Redeemable Preferred Units.

During the year ended December 31, 2015, net cash provided by financing activities was $51.6 million, due to the proceeds from our sales of Redeemable Preferred Units of $6.8 million and net proceeds of $44.8 million from the issuance of non-controlling interests in our consolidated subsidiary Solid GT.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing development activities related to SGT-001. In addition, commencing upon the closing of this offering, 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:

- conduct our additional preclinical research of SGT-001 and 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 October 26, 2017, we completed the sale of 4,886,000 Series 2 Senior Preferred Units at a price of $11.26 per unit in exchange for net proceeds of $55.0 million.

As of September 30, 2017, we had cash, cash equivalents and available-for-sale securities of $29.6 million. We believe that the anticipated net proceeds from this offering, together with our existing cash, cash equivalents and available-for-sale securities, as well as the proceeds from the sales of the Series 2 Senior Preferred Units in October 2017 will enable us to fund our operating expenses and capital expenditure requirements through at least the next 12 months. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect.

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 our planned 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;
- the costs associated with constructing and validating our own manufacturing facility;
- 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 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 cGMP compliant facility located at one of our CDMO partners. We intend to establish the capability and capacity to supply SGT-001 at commercial scale from multiple sources, including eventually building our own GMP facility to ensure redundancy and reliability. We expect that such a facility would require capital expenditures of between $35.0 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 our Phase I/II clinical trials for SGT-001.

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 drugs 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, your 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 December 31, 2016 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	4 - 5 Years	More Than 5 Years
Operating lease commitments (1)	$313	$ 288	$ 25	$—	$ —

(1) Represents minimum payments due for our lease of office and laboratory space in Cambridge, Massachusetts under an operating lease agreement that, as amended, expires in January 2018. Amounts exclude office and laboratory space in Cambridge, Massachusetts, for which we entered into a lease in May 2017 and amended during the third quarter of 2017, which extends through April 2018, at a monthly amount of $136,000. Amounts also exclude laboratory space in Cambridge, Massachusetts, for which we entered into a lease in January 2018. The initial lease term is for five years and the minimum rent commitment due over the initial term is approximately $3.8 million.

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. See "Business—Strategic partnerships and collaborations/licenses."

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. Payments due upon cancelation consist only of payments for services provided and expenses incurred up to the date of cancelation.

Internal control over financial reporting

During the audit of our consolidated financial statements as of and for the years ended December 31, 2015 and 2016, we identified material weaknesses in our internal control over financial reporting. A company's internal control over financial reporting is a process designed by, or under the supervision of, a company's principal executive and principal financial officers, or persons performing similar functions, and effected by a company's board of directors, management and other personnel to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements in accordance with generally accepted accounting principles. Under standards established by the Public Company Accounting Oversight Board, a material weakness is a deficiency or 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 and corrected on a timely basis. The material weaknesses that 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.

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, and our independent registered public accounting firm, were not required to perform an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act. Accordingly, we cannot assure you that we have identified all, or that we will not in the future have additional, material weaknesses. Material weaknesses may still exist when we report on the effectiveness of our internal control over financial reporting as required by reporting requirements under Section 404 after the completion of this offering. See "Risk factors—We have identified material weaknesses in our internal control over financial reporting."

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.

Recently issued accounting pronouncements

We have reviewed all recently issued standards and have determined that, other than as disclosed in Note 2 to our consolidated financial statements appearing at the end of this prospectus, such standards will not have a material impact on our consolidated financial statements or do not otherwise apply to our operations.

Emerging growth company status

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.

Quantitative and qualitative disclosures about market risk

We are exposed to market risk related to changes in interest rates. As of September 30, 2017, our available-for-sale securities consisted of corporate bond securities and U.S. government agency securities that have contractual maturities of one year or less. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

BUSINESS

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.

SGT-001 has been granted RPDD, in the United States and Orphan Drug Designations in both the United States and European Union. The safety and efficacy of SGT-001 are currently being evaluated in a Phase I/II clinical trial.

For patients suffering from DMD, symptoms usually begin to manifest between three and five years of age, when they fail to reach developmental milestones or experience motor function challenges, such as difficulty walking or climbing stairs. As the disease progresses, patients with DMD experience frequent falls; can no longer run, play sports or perform most daily functions; and are further weakened by physical activity. By their early teens, DMD patients typically lose their ability to walk and ultimately become dependent on a wheelchair for mobility. By their 20s, patients essentially become paralyzed from the neck down and require a ventilator to breathe. Though disease severity and life expectancy vary, a DMD patient's quality of life dramatically decreases over time, with death typically occurring by early adulthood from either cardiac or respiratory complications.

Our founders, who are personally touched by the disease, created a biotechnology company purpose-built to accelerate the discovery and development of meaningful therapies for all patients affected by DMD. Through this disease-focused business model, our research team, led by experts in DMD biology and drug development, along with key opinion leaders in DMD, continuously evaluate emerging science to identify high-potential product candidates. Our selection process includes extensive diligence and initial pharmacology research with highly specific, predefined criteria, which provide us with confidence in our development program decisions. Through this data-driven selection process, we have evaluated a number of programs and identified gene therapy as a potentially beneficial approach for DMD, and thus initiated development of our lead product candidate SGT-001. We will continue to apply this rigorous approach and reject the majority of the candidates we evaluate in our effort to develop only programs that we believe have the greatest likelihood of becoming therapies for DMD patients.

Our product candidates

SGT-001 is our lead gene transfer candidate. Gene transfer, a type of gene therapy, is designed to address diseases caused by mutated genes through the delivery of functional versions of those genes, called transgenes. The transgenes are then utilized by the body to produce proteins that are absent or not functional prior to treatment, potentially offering long-lasting beneficial clinical effects. SGT-001 is designed to address the underlying genetic cause of DMD by delivering a synthetic transgene that produces dystrophin-like protein that is only expressed in muscles of the body, including cardiac and respiratory muscles. The transgene is delivered

via an AAV vector, which also contains a muscle-specific promoter. Our vector is a modified version of an AAV, a naturally occurring, non-pathogenic virus selected for its ability to efficiently enter skeletal, diaphragm and cardiac muscle tissues. The vector will carry a synthetic dystrophin transgene construct, called microdystrophin, that retains the most critical components of the full-size dystrophin gene yet is small enough to fit within AAV packaging constraints. SGT-001 is designed to drive microdystrophin protein expression in affected muscles throughout the body. We have studied the efficacy, safety and durability of SGT-001 in multiple preclinical models and its functional benefits in DMD animal studies. In contrast to other therapeutic approaches that are designed to target specific mutations in the dystrophin gene, we believe SGT-001 is a mutation agnostic approach.

In the fourth quarter of 2017, we announced the initiation of a randomized, controlled, open-label, single-ascending dose Phase I/II clinical study, called IGNITE DMD, which is designed to evaluate SGT-001 in ambulatory and non-ambulatory males with DMD aged four to 17 years. The primary objectives of the study are to assess the safety and tolerability of SGT-001, as well as efficacy as defined by microdystrophin protein expression. The study will also assess muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with microdystrophin production, patient reported outcomes and quality of life measures, among other endpoints. The study will enroll approximately 16 to 32 patients with DMD, who will be randomly assigned to either an active treatment group or a delayed treatment group. Initially, adolescents aged 12 to 17 years will receive treatment and, at a later stage of the study, children aged four to 11 years will be dosed. Our IND permits us to proceed with administering our proposed low dose to patients. Prior to dosing patients in our higher-dose group, we will be required to resolve the partial clinical hold on SGT-001 outlined in a November 2017 letter to us from the FDA. In order to do so we will need to decrease the number of vials and utilize no more than a single production lot per patient and demonstrate that we have the appropriate manufacturing processes in place to support the higher-dose group. In addition, the FDA had additional comments and requests for information that were characterized as not clinical hold comments. We expect that we will be able to address the specific deficiencies identified by the FDA by submitting additional information demonstrating manufacturing capacity and product attributes that will support the high-dose group. The Company intends to submit a response to the FDA addressing the specific deficiencies in the near future, after which the FDA will have 30 days to respond. The Company does not expect that the overall timing for clinical development of SGT-001 will be affected by the partial clinical hold. Further, the partial clinical hold does not impact the Company's ability to conduct its clinical development activities of SGT-001 at low-dose levels. If the partial clinical hold is not lifted on our Phase I/II clinical trial, we will not be able to evaluate the safety, tolerability and efficacy of SGT-001 at the high dose level, which could negatively impact the development of SGT-001. Efficacy will be assessed by comparing microdystrophin protein expression in muscle biopsy before treatment and 12 months after treatment for each patient. Participants in the control group who continue to meet inclusion criteria and not meet exclusion criteria will receive active treatment after 12 months. Based on results from this study, we will evaluate the need for future clinical trials that may include other patient populations, as well as the need for larger confirmatory clinical trials. If approved, we intend to commercialize SGT-001 in the United States and European Union, and we may enter into licensing agreements or strategic collaborations to commercialize the product candidate in other markets.

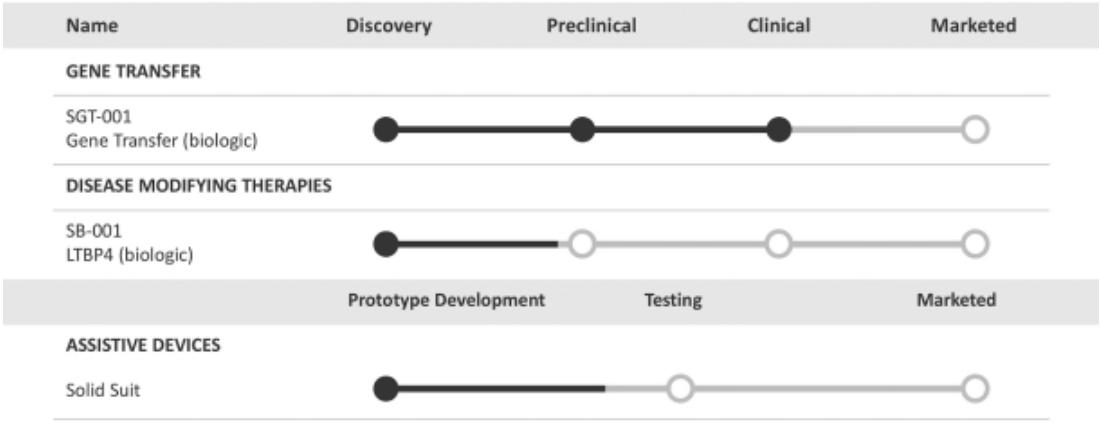
Taking into account the prevalence and incidence of DMD and the anticipated dosing requirements for gene transfer, we anticipate that there will be a need for a substantial supply of SGT-001 for clinical trials and, if approved, for commercial markets. Through significant targeted investments to address this challenge, we believe we have generated sufficient drug product supply to initiate our first clinical trial. We continue to develop our manufacturing process to meet future clinical and commercial production needs for SGT-001.

While we believe DMD disease progression can be slowed or halted by gene transfer, many patients will still suffer from the manifestations of the disease, such as tissue damage to their muscles, inflammation, cardiac dysfunction and fibrosis. As part of our disease-focused business model, we are also building a portfolio of complementary disease modifying therapies to address these manifestations. Our portfolio currently includes a preclinical biologic candidate, SB-001, a monoclonal antibody designed to reduce fibrosis and inflammation, as

well as a number of emerging and complementary programs. We intend to commence preclinical studies for SB-001 in 2018.

In addition to developing our pipeline of product candidates, we believe it is critical to invest time and resources in tools and technologies designed to help us more effectively understand DMD, accurately monitor disease progression and assist patients in daily life. As part of this goal, we are developing biomarkers and sensors that may allow us to identify treatment targets faster, measure the therapeutic impact of potential product candidates better and reach decision points earlier. In addition, through our Solid Suit program, we are developing a line of soft, wearable assistive devices with the goal of providing functional and therapeutic benefits to DMD patients.

Our pipeline



We seek to protect our proprietary and intellectual property position through a combination of patents, trade secret laws, proprietary know-how, continuing technological innovation, and entering into non-disclosure, confidentiality and invention assignment agreements. We have exclusively licensed three issued U.S. patents, one pending U.S. non-provisional patent application, and seven issued patents and eleven pending patent applications in foreign jurisdictions. We have filed two pending U.S. provisional patent applications. We intend to continue building out our intellectual property protection to further strengthen our position in the DMD field.

Who we are

Solid Biosciences was founded in 2013 by our Chief Executive Officer, Ilan Ganot, our Chairman of the Board, Andrey Zarur, and our President, Gilad Hayeem, with the goal of developing meaningful therapies for patients with DMD. Solid is the English translation of Eytani, the Hebrew name of Ilan and Annie Ganot's son, who was diagnosed with the disease in 2012. Our founders, unsatisfied with the existing therapeutic landscape, proceeded to raise funds to execute on our disease-focused business model. We assembled a passionate management team and scientific advisory board composed of individuals with extensive experience in DMD, gene therapy, product discovery, research and development, manufacturing, business strategy and finance.

In 2015, we began exclusively licensing the elements of the construct for SGT-001 and other elements of our gene transfer program from the University of Michigan, the University of Missouri and the University of Washington. Since then, we have continued to use our extensive network across the academic, business and patient communities to identify, vet and pursue high-potential complementary product candidates to address the needs of DMD patients.

Since our inception, we have raised private capital from a group of investors, including entities affiliated with Bain Capital Life Sciences, Biogen, JPMC Strategic Investments II Corporation, Perceptive Advisors and RA Capital, along with several additional corporate and private investors. In addition, three leading U.K.-based DMD charities provided initial seed funding for our gene transfer program in return for equity in our company. We continue to work closely with the patient advocacy community and have accepted additional contributions from several DMD charities to fund our early-stage research programs.

Mission

Our mission, which guides every aspect of our operations, is to cure DMD. Underscoring this mission, our disease-focused business model is founded on the following fundamental values:

- identify and develop meaningful therapies for all patients with DMD;
- bring together the leading experts in DMD, science, technology, disease management and care; and
- be guided by the needs of DMD patients.

Our strengths

Guided by our mission, we set out to create a company that understands DMD and develops therapies that are intended to provide meaningful benefits to DMD patients. We believe we are well positioned to execute on our mission based on the following competitive strengths:

- ***Singular focus on DMD.*** We are singularly focused on meeting the diverse needs of all DMD patients, regardless of their genetic mutation or disease stage. Our product candidates target the underlying cause of DMD, as well as address the multiple manifestations of the disease.
- ***Deep understanding of the impact of the disease.*** We are founded by people personally touched by DMD, and we have established meaningful partnerships within the DMD community. We believe our frequent interactions with patients and caregivers and our understanding of the day-to-day impact of the disease give us a deep sense of urgency, as well as knowledge of our stakeholders and their needs.
- ***Rigorous product candidate selection process.*** We subject each potential product candidate to a highly focused, data-driven selection process that lies at the core of our business model. Our selection process includes extensive diligence and initial pharmacology research with highly-specific, predefined criteria that led us to initiate development of our lead gene transfer candidate, SGT-001, our preclinical disease modifying candidate, SB-001, and emerging and complementary programs from among a significant number of potential therapies that we evaluated. We are technology-agnostic and seek only to advance and invest in product candidates that we believe have the greatest potential for success.
- ***Highly experienced management team focused on DMD.*** Our management team has extensive expertise in DMD, gene therapy, product discovery, research and development, manufacturing, business strategy and finance, with proven track records at organizations including Johnson & Johnson, Pfizer, Philips Healthcare, Roche, Harvard University and the NIH.
- ***Network of world-renowned experts advising our development efforts.*** We have assembled a scientific advisory board and a broad network of the world's leading experts in DMD, gene therapy, biologics manufacturing, immunology and clinical development. We believe this center of excellence provides us with unparalleled access to the latest, most transformative ideas and therapeutic approaches to address the needs of DMD patients.
- ***Foundational work in scalable manufacturing processes.*** We are working to develop a scalable manufacturing process for SGT-001. We believe our early investment in our manufacturing process will enable us to scale production at the quantities needed to carry out clinical trials and to supply commercial markets, with a reduced risk of delay and unexpected costs.

Our strategic priorities

Our disease-focused business model is purpose-built to identify and accelerate the discovery and development of multiple product candidates. Key elements of our strategy include the following:

- ***Rapidly advance SGT-001 through clinical trials and deliver it to patients.*** We initiated a Phase I/II clinical trial to assess the safety and efficacy of SGT-001 in the fourth quarter of 2017. The FDA has granted SGT-001 RPDD, and both the FDA and EMA have granted the candidate Orphan Drug Designation for the treatment of DMD. If approved, we intend to commercialize SGT-001 in the United States and European Union, and we may enter into licensing agreements or strategic collaborations to commercialize the product in other markets.
- ***Continue to advance SB-001 through preclinical development.*** We intend to advance our initial disease-modifying therapy candidate, SB-001, aimed at addressing fibrosis and inflammation. We currently intend to commence preclinical studies for SB-001 in 2018.
- ***Continue to build our product pipeline with high-potential product candidates for DMD.*** Leveraging our network of world-renowned DMD experts and rigorous product candidate selection process, we intend to identify and develop additional high-potential product candidates. These include the next generation of gene therapies, such as novel promoters, vectors and transgenes, as well as additional complementary disease-modifying therapies. We will continue to seek to protect and control the intellectual property, development and commercialization of our product candidates.
- ***Continue to scale our manufacturing process to meet clinical and commercial needs.*** We intend to supply our clinical development program for SGT-001 with drug product produced at a cGMP compliant facility located at one of our Contract Development Manufacturing Organization, or CDMO, partners. Our in-house scientists will continue to work to increase the productivity and efficiency of our manufacturing process. We intend to establish the capability and capacity to supply SGT-001 at commercial scale from multiple sources, including eventually building our own GMP facility to ensure redundancy and reliability.
- ***Develop tools to accelerate the discovery and development of therapies for DMD.*** We believe it is critical to invest time and resources into developing tools that are designed to help us more effectively measure disease progression and the therapeutic impact of our product candidates. We are focused on developing biomarkers and sensors that will allow us to identify treatment targets faster, measure the therapeutic impact of potential product candidates better and reach decision points earlier.
- ***Partner with the DMD community to inform our programs***. We will continue to work with and listen closely to key stakeholders in the DMD community, including scientists, academic experts and patients and their families. This will allow us to remain guided by the needs of patients and inform future development programs and strategies to bring approved therapies to the community.

About Duchenne muscular dystrophy

DMD is an X-chromosome-linked, muscle-wasting disease, predominantly affecting boys. Progressive, irreversible and ultimately fatal, DMD occurs in 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. In DMD, mutations in the dystrophin gene result in the body's inability to produce functioning dystrophin protein, which works to strengthen muscle fibers and protect them from daily wear and tear. Dystrophin protein also serves as the cornerstone of the dystrophin glycoprotein complex, or DGC, a group of proteins that links the inner and outer components of muscle cells to ensure proper muscle function.

Without dystrophin and the DGC, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of scar and fat tissue. More than 1,000 dystrophin gene mutations, which can be inherited or can occur spontaneously, have been identified in people with DMD.

For patients suffering from DMD, symptoms usually begin to manifest between three and five years of age, when they fail to reach developmental milestones or experience motor function challenges, such as difficulty walking or climbing stairs. Muscle wasting initially presents in the legs and pelvic area, then in the muscles of the shoulders, neck and arms. As the disease progresses, patients with DMD experience frequent falls, can no longer run, play sports or perform most daily functions, and are further weakened by physical activity. In addition to physical challenges, DMD also commonly involves cognitive difficulties and behavioral challenges.

By their early teens, DMD patients typically lose their ability to walk and become dependent on a wheelchair for mobility. By their 20s, patients essentially become paralyzed from the neck down and require a ventilator to breathe. Though disease severity and life expectancy vary, a patient's quality of life dramatically decreases over time, with death typically occurring by early adulthood from either cardiac or respiratory complications.

Need for effective therapies

There is no cure for DMD and, for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments.

Glucocorticoid treatment, the current standard-of-care, has been shown to temporarily improve muscle strength, prolong the period of ambulation and slow the progression of DMD. However, glucocorticoid use is associated with well-known adverse events, such as severe weight gain, stunted growth, weakening of bone structure and metabolic dysfunctions, among others. The most commonly used glucocorticoids include prednisone and deflazacort (EMFLAZA). Deflazacort has been commercially available in several countries outside of the United States and was recently approved in the United States for the treatment of DMD.

In recent years, certain regulators have conditionally approved two new therapies, eteplirsen (EXONDYS 51) and ataluren (Translarna), which target specific mutations in the dystrophin gene. These therapies are indicated for only a small portion of the DMD patient population, and their respective efficacy profiles still need to be fully understood.

Eteplirsen is an antisense oligonucleotide indicated for DMD patients who have a confirmed mutation of the dystrophin gene amenable to exon 51 skipping, which affects approximately 13% of DMD patients. Eteplirsen is administered as a weekly intravenous infusion. In 2016, Eteplirsen was granted accelerated approval from the FDA based on an increase in dystrophin in skeletal muscle observed in some patients who received the therapy. However, the FDA concluded that a clinical benefit, including improved motor function, has not been established. Eteplirsen is still under review by regulatory authorities outside of the United States.

Ataluren is a small molecule indicated for the treatment of patients who have DMD resulting from nonsense mutations in the dystrophin gene, which also affect approximately 13% of DMD patients. In 2014, ataluren received conditional marketing authorization from the European Commission, and has since been approved in several other countries outside of the United States. Ataluren's indication is currently limited to ambulatory patients five years of age and older. In October 2017, the FDA issued a complete response letter (CRL) for the NDA for Ataluren.

Current best practices for treating DMD patients also dictate a multidisciplinary approach to disease management, which includes physical and occupational therapy to preserve strength, function and flexibility, orthopedic management to reduce the risk of scoliosis and other bone and joint problems, pulmonary, cardiac and gastrointestinal management, and psychosocial management to support behavior and learning.

Burden of disease

Despite recent therapeutic advances, DMD represents a significant societal and economic burden. The economic burden, estimated at $1.2 billion annually in the United States (excluding costly mortality and

end-of-life care expenses), includes costs associated with hospital admissions, medication, frequent doctor visits and investment in assistive devices, as well as indirect costs related to productivity losses for the caregivers and costs due to pain, anxiety and social handicap. Of this amount, approximately 45% is represented by indirect costs. Only a small proportion of DMD patients are employed and many caregivers reduce their hours or stop working altogether to care for their children, who progressively require more help with everyday tasks, such as eating, dressing and using the bathroom. In some cases, patients also experience serious mental health issues that require additional support and treatment.

Solid's 360-degree solution

We aim to address the full spectrum of DMD disease manifestation, from its underlying genetic cause to other disorders that result from disease progression. We are advancing corrective therapies, disease-modifying therapies and assistive devices, as well as tools to accelerate drug development.

Gene transfer—A corrective therapy

Gene therapy is a therapeutic approach that aims to address diseases caused by gene mutations. A gene is a portion of DNA that provides the instructions for the body to construct proteins that perform functions needed for life. Genes are prone to mutations, which can either be inherited or occur spontaneously. While many mutations are harmless, some lead to the absence of crucial proteins, resulting in serious genetic diseases like DMD.

Gene transfer, a type of gene therapy, is designed to address diseases caused by mutated genes through the delivery of functional versions of those genes, called transgenes. The transgenes are then utilized by the body to produce proteins that are absent or not functional prior to treatment, potentially offering long-lasting beneficial effects.

A gene transfer candidate typically includes three essential components:

- a vector—a vehicle that delivers a transgene to cells in the body;
- a transgene—a functional gene intended to produce a functional protein; and
- a promoter—a specialized DNA sequence that directs cells to produce the protein in specific tissues.

We have focused our initial efforts on gene transfer because we believe it has the greatest potential to address the root cause of DMD: the absence or near-absence of dystrophin protein. If successful, we believe gene transfer can slow or stop the progression of DMD in a majority of patients, irrespective of their genetic mutation, by producing long-term, muscle-specific expression of a functional dystrophin-like protein.

SGT-001

SGT-001, our lead gene transfer candidate, is under investigation for its ability to preserve muscle function in DMD patients after a single administration. The SGT-001 construct is comprised of a functional transgene that is delivered via an adeno-associated viral vector containing a muscle-specific promoter.

Vector: The vector is a modified version of a naturally occurring, non-pathogenic virus called AAV. Vectors derived from AAVs are modified to no longer self-replicate, yet retain their ability to effectively introduce new genetic material directly into patients' cells. AAV vectors have been extensively studied in human clinical trials in multiple disease indications, including in clinical trials of high-dose, systemically delivered AAV gene therapies being conducted by third parties. There are several subtypes of AAV vectors that differ based on the proteins that make up their outer shells, or capsids. These capsids have affinities for different sites in the body. We selected the AAV9 serotype capsid for clinical development based on our preclinical data, which demonstrated the capsid's ability to enter skeletal, diaphragm and cardiac muscle tissues.

Transgene: Dystrophin, the largest gene in the body, exceeds the carrying capacity of AAV vectors. To overcome this challenge, we advanced development of the SGT-001 transgene, a synthetic, dystrophin-like gene that fits into AAV and has the ability to drive functional protein expression in skeletal, diaphragm and cardiac muscle tissue.

The concept of a modified therapeutic dystrophin gene originated from research on Becker muscular dystrophy, or BMD, where researchers discovered that certain BMD patients had mutations in the dystrophin gene that drove expression of a functional form of dystrophin protein, allowing patients to live relatively normal lives. This discovery led scientists to engineer a number of synthetic, dystrophin transgene constructs, called microdystrophins, that retained only the most critical components of the full-size dystrophin gene yet were small enough to fit within AAV packaging constraints. There are several types of microdystrophins that differ based on the configuration of their components. Microdystrophins were subsequently demonstrated to functionally protect muscle in mouse models of DMD.

The SGT-001 microdystrophin construct, which is our lead clinical candidate for DMD, is based on three decades of development and optimization work at the University of Michigan, University of Missouri and University of Washington. In preclinical studies, Jeffrey Chamberlain, Ph.D., from the University of Washington, and Dongsheng Duan, Ph.D., from the University of Missouri, identified a proprietary configuration of genetic components that, when administered systemically, produces functional microdystrophin protein expression that not only stabilizes muscle membranes and protects muscle against injury, but also simultaneously restores the localization of DGC to the muscle membrane, notably increasing neuronal nitric oxide synthase, or nNOS, concentration. In subsequent published studies, Dr. Duan demonstrated in animal models that, in comparison to earlier configurations, nNOS-restoring microdystrophins were more effective in improving muscle function and blood circulation.

Promoter: The expression of the SGT-001 microdystrophin transgene is regulated by a modified, synthetic muscle-specific promoter cassette called CK8, which is derived from the naturally occurring muscle creatine kinase promoter. Regulatory cassettes, such as CK8, are used to prompt gene expression specifically in muscle tissues. In comparison to other regulatory cassettes, we chose CK8 due to its small size and its ability to drive microdystrophin transgene expression in skeletal, diaphragm and cardiac muscle tissues. In our preclinical studies in small and large animal models, CK8 restricted microdystrophin transgene expression to these muscles.

SGT-001 preclinical program

Our comprehensive preclinical program for SGT-001 is comprised of studies that inform efficacy, durability and safety, as well as dose response and the kinetics of transgene expression. Our program includes three different animal species: mice, dogs and NHPs. Our preclinical studies were performed by third-party collaborators over the last three years.

Well established mouse and dog disease models for DMD offered us the opportunity to better evaluate the potential translatability of SGT-001 to humans. While studies in dystrophic mice, such as the mdx mouse, provide important efficacy rationale, we chose to perform additional functional studies in dystrophic dogs because they exhibit a more severe dystrophic phenotype and progress similarly to human patients at earlier stages of the disease. Dog models enabled us to assess various endpoints, including biodistribution, expression, durability and function in a large animal species.

SGT-001 PRECLINICAL PROGRAM OVERVIEW	
Wild-type and Dystrophic Mice	• Expression • Function • Dose Response • GLP Toxicology • Kinetics • Manufacturing Comparability
Mixed Breed Dystrophic Dogs (cDMD)	• Expression • Durability • Function • Dose Response
Golden Retriever Muscular Dystrophy (GRMD)	
Non-human Primates	• GLP Toxicology

Because DMD is a disease defined by a lack of dystrophin protein, it is important to reliably detect microdystrophin expression in muscle after SGT-001 treatment. As part of our core preclinical program, we developed well characterized and well recognized analytic approaches to confirm transgene expression and localization, using the following assays:

- Immunofluorescence: A qualitative method to determine if a transgene is expressed and localized to muscle membrane.
- Western blot: A recognized method to quantify dystrophin expression, which is a validated biomarker.
- Mass spectrometry: A highly sensitive analytical method to quantify transgene expression.

We also employed immunofluorescence to confirm if our microdystrophin construct restored the DGC, including key proteins such as sarcoglycan and nNOS.

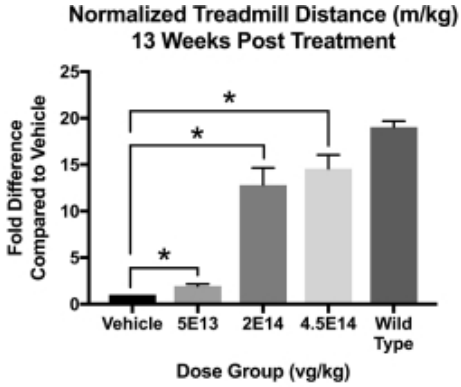
Efficacy in dystrophic mice

Multiple studies in both dystrophic, or mdx, and healthy, or wild-type, mice have demonstrated that a single intravenous administration of SGT-001 induces measurable levels of microdystrophin protein expression. In all

studies, microdystrophin protein expression was measured using immunofluorescence, Western blot and mass spectrometry.

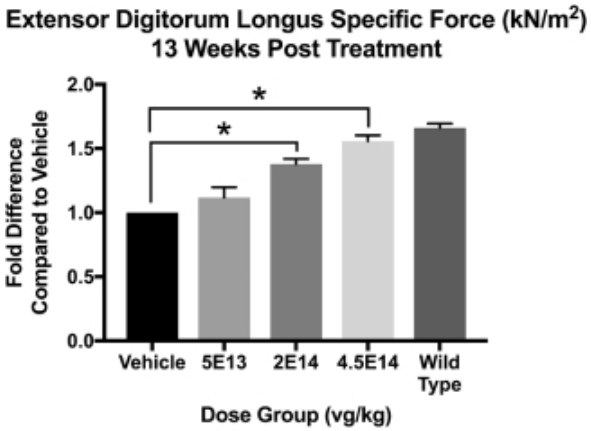
In an mdx dose-response study, a clear dose-dependent pattern of transgene expression was observed at day 28 by all three assays. As an example, at a dose of 1E14 vg/kg, transgene expression as quantified by positive immunoflouresence staining in the quadriceps and heart muscle tissues was 50% and 80% of the full-length dystrophin levels quantified in healthy wild-type control muscles. Similar levels of microdystrophin expression were found in all mdx studies completed to date. Efficacy studies performed in dystrophic mice treated with SGT-001 demonstrated significant, dose-responsive improvements in both muscle morphology and multiple physiological parameters. In a blinded efficacy study performed in mdx mice dosed at approximately six weeks of age, SGT-001 treatment showed a statistically significant improvement in grip strength, which assesses arm and leg strength, at multiple doses.

In addition, using a treadmill exhaustion assay, the total distance run by the SGT-001-treated mdx mice was approximately two- to fifteen-fold longer compared to the untreated mice at all time points five-weeks post-dose.



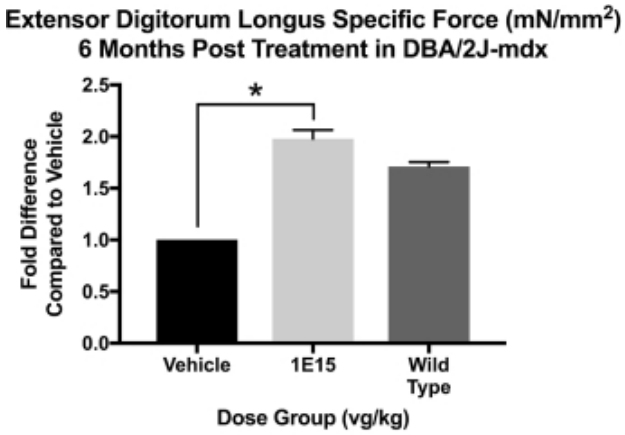
Solid bars represent mean group values. Brackets above each bar represent standard error of the mean. Statistically significant results ($p<0.05$) are indicated by the * symbol. Animal numbers per group are as follows: Vehicle n=12, 5E13 n=11, 2E14 n=12, 4.5E14 n=12, Wild Type n=12.

At study termination, muscle force was measured *ex vivo* in the extensor digitorum longus muscle in all animals. SGT-001-treated mdx mice, dosed at either 2E14 or 4.5E14 vg/kg, exhibited a 1.3-fold increase in specific muscle force over untreated controls when compared to the untreated mdx mice.



Solid bars represent mean group values. Brackets above each bar represent standard error of the mean. Statistically significant results ($p<0.05$) are indicated by the * symbol. Animal numbers per group are as follows: Vehicle n=12, 5E13 n=10, 2E14 n=11, 4.5E14 n=10, Wild Type n=12.

In a second efficacy study employing a more severe dystrophic mouse model, or DBA/2J-mdx, a version of SGT-001 was administered at a dose of 1E15 vg/kg. Treated mice exhibited functional results that were similar to untreated wild-type animals. In the SGT-001-treated DBA/2J-mdx mice, the specific muscle force was similar to wild-type mice. Further, the treated animals were protected against muscle damage associated with eccentric contractions, a type of contraction related to muscle lengthening under load that is known to be highly damaging to dystrophic muscles. In contrast, untreated DBA/2J-mdx mice showed significantly reduced specific force and no protection against eccentric contraction induced muscle damage.



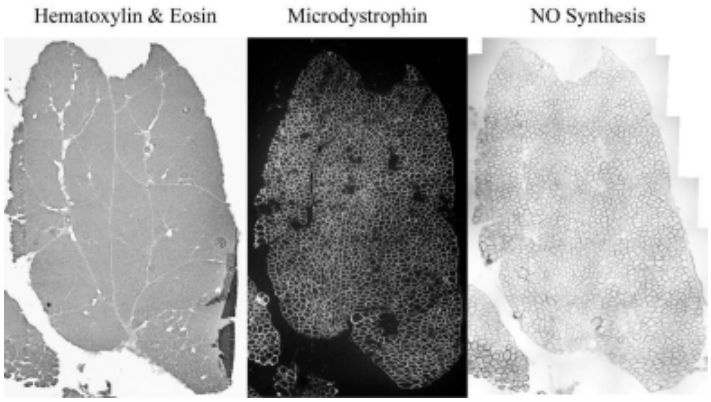
Solid bars represent mean group values. Brackets above each bar represent standard error of the mean. Statistically significant results ($p<0.05$) are indicated by the * symbol. Animal numbers per group are as follows: Vehicle n=11, 1E15 n=5, Wild Type n=9.

Efficacy in dystrophic dogs

Two independent studies in dystrophic dogs assessed durability of microdystrophin expression and efficacy, respectively. These studies were performed in two distinct dystrophic dog models (mixed breed dystrophic dogs, or cDMD, and Golden Retriever Muscular Dystrophy, or GRMD), collectively encompassing a number of genetic mutations that lead to the absence of dystrophin protein. This enabled us to assess SGT-001 across multiple mutations, which is more reflective of the composition of the DMD patient population. Both studies used a canine-optimized version of the microdystrophin gene.

In a long-term dose-ranging study, five three-month-old, juvenile cDMD dogs received an intravenous dose of either 5E13 vg/kg (n=1), 1E14 vg/kg (n=2), 3E14 vg/kg (n=1) or 5E14 vg/kg (n=1). In this study, muscle biopsies were collected from the skeletal muscles at one, three, six, 12, 16, 20, 24 and 30 months after injection. Robust transgene expression was detected by immunofluorescence at all time points and at all the dose levels. In animals dosed with 1E14 vg/kg, approximately 70-90% of the muscle fibers were positive for microdystrophin. In treated muscle samples, transgene expression was associated with stabilization of the DGC, including nNOS. All doses were well tolerated and there was no observed immune response to the transgene. This study is currently ongoing.

Biopsy of the Semitendinosus Muscle 16 Months Post Administration of Canine SGT-001 in the cDMD Dog

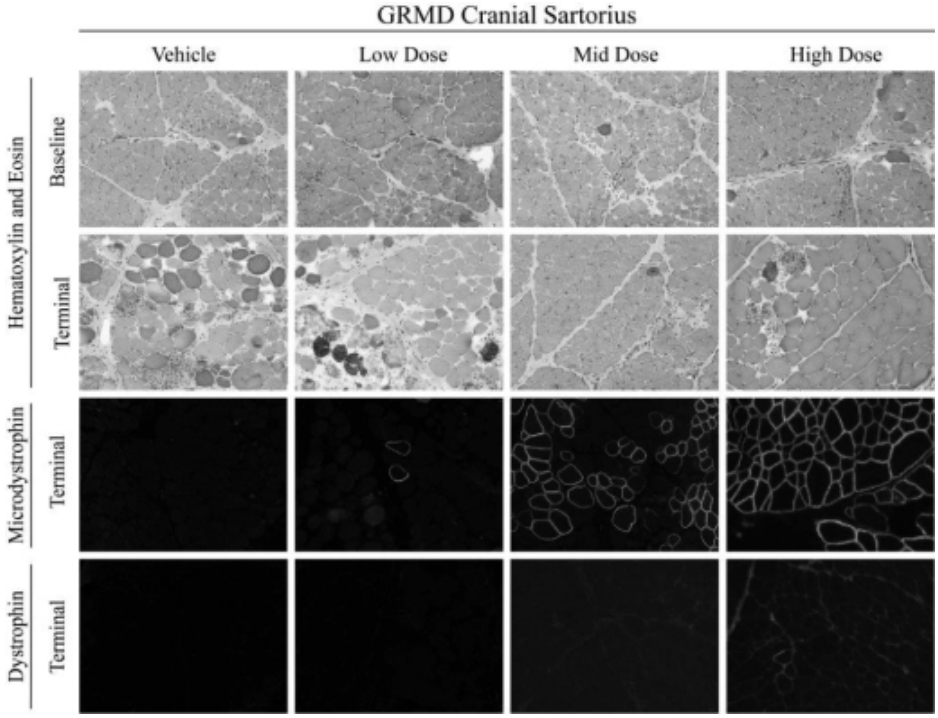


A blinded dose-ranging study in the GRMD model assessed the general safety and efficacy of the canine construct of SGT-001. The three dose levels (1E13, 1E14 and 2E14 vg/kg) were administered at three months of age and animals were followed for three months following administration. All doses were well tolerated and there was no observed immune response to the transgene.

Dose-dependent transgene expression was detected in interim biopsies of skeletal muscles at day 28 and 45 and at the end of the study at day 91 in skeletal, diaphragm and cardiac muscles. A blinded histological evaluation of the muscle tissue revealed a reduction of dystrophic pathology at the higher dose levels. In the mid- and high-dose groups, all muscles biopsied at the end of the study exhibited improved pathology compared to low dose and untreated controls. Biodistribution studies demonstrated dose dependent transgene expression that was only detectable in the muscle tissues.

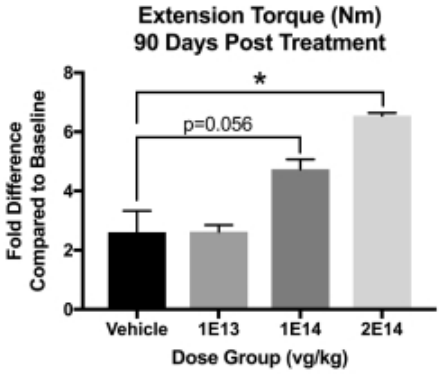
The observed dose response was detectable by both immunofluorescence and Western blot. Quantification by Western blot averaged less than 10% of wild-type in the low-dose (1E13 vg/kg) animals. In the mid-dose animals, the level of expression among the skeletal muscles ranged from an average of approximately 20% to approximately 50% of wild-type control muscles. At 2E14 vg/kg, the level of expression ranged from 30% to 70% of wild-type dystrophin. This data also correlates to quantification of microdystrophin via mass spectrometry.

SGT-001 transgene expression dose response and correlation to improved histopathology in the GRMD model (n = 3/dose level)

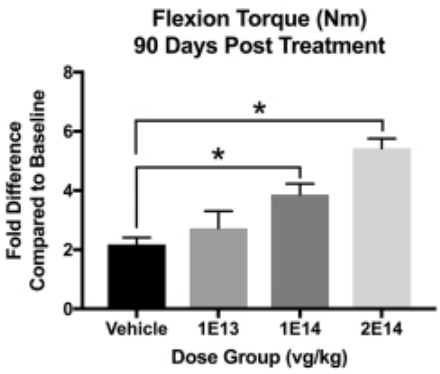


Dose-dependent, sustained expression of microdystrophin not only correlated with histological improvements in muscle, but also provided statistically significant improvements in measures of muscle function. At day 90, muscle force generation was improved in both the 1E14 vg/kg and 2E14 vg/kg cohorts, indicating that the microdystrophin produced by SGT-001 is highly protective in a large animal dystrophic species.

Effect of SGT-001 on muscle strength in the GRMD model 90 days post treatment



Solid bars represent mean group values. Brackets above each bar represent standard error of the mean. Statistically significant results ($p<0.05$) are indicated by the * symbol. Animal numbers per group are as follows: Vehicle n=3, 1E13 n=3, 1E14 n=3, 2E14 n=3.



Solid bars represent mean group values. Brackets above each bar represent standard error of the mean. Statistically significant results ($p<0.05$) are indicated by the * symbol. Animal numbers per group are as follows: Vehicle n=3, 1E13 n=3, 1E14 n=3, 2E14 n=3.

The efficacy data collectively described above in both dystrophic mouse and dog models was incorporated into an overall nonclinical model to inform dose selection for our clinical program.

Manufacturing comparability

As part of our manufacturing process development, we have run comparability studies at each stage of our process scale-up. These comparability studies were carried out using *in vivo* mouse models to ensure that our drug product produced at different scales is comparable to each other.

Safety

As part of our preclinical program, we performed necessary GLP toxicology studies to establish the overall safety profile of SGT-001 in wild-type mice and 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 studies. 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.

Clinical Development of SGT-001

We are developing SGT-001 for the treatment of DMD through a single intravenous administration. In the fourth quarter of 2017, we announced the initiation of IGNITE DMD, a randomized, controlled, open-label, single-ascending dose Phase I/II clinical study designed to evaluate SGT-001 in ambulatory and non-ambulatory males with DMD aged four to 17 years. The study is currently enrolling patients at our first clinical site in the United States. We intend to initiate the clinical trial at additional sites in the United States and abroad.

The primary objectives of IGNITE DMD are to assess the safety and tolerability of SGT-001, as well as efficacy as defined by microdystrophin protein expression. The study will also assess muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with microdystrophin production, patient reported outcomes and quality of life measures, among other endpoints. The study will enroll 16 to 32 patients with DMD. Key inclusion criteria include: established clinical diagnosis of DMD and documented dystrophin gene mutation predictive of DMD phenotype; anti-AAV9 antibodies below pre-specified thresholds; stable cardiac and pulmonary function; and a stable daily dose of oral corticosteroids ^{3} 24 weeks. There is no restriction based on a patient's underlying dystrophin gene mutation.

Study participants will be randomly assigned to either an active treatment group or a delayed treatment control group. The selection of our starting dose was based on safety and efficacy data observed in our preclinical studies. Dose escalation between cohorts and decisions regarding study progression will occur after review by the Data Safety Monitoring Board, or DSMB. Adolescents aged 12 to 17 years will be treated initially, followed by children aged four to 11 years. Efficacy will be assessed by comparing microdystrophin protein expression in muscle biopsy before and 12 months after treatment for each patient. Other endpoints will be compared against the control group. The delayed treatment control group will be rolled into an active treatment phase after 12 months, as long as participants continue to meet the study's exclusion and inclusion criteria and not meet its exclusion criteria. Long-term follow up will continue per regulatory guidelines.

Our IND permits us to proceed with administering our proposed low dose to patients. Prior to dosing patients in our higher-dose group, we will be required to resolve the partial clinical hold on SGT-001 outlined in a November 2017 letter to us from the FDA. In order to do so we will need to decrease the number of vials and utilize no more than a single production lot per patient and demonstrate that we have the appropriate manufacturing processes in place to support the higher-dose group. In addition, the FDA had additional comments and requests for information that were characterized as not clinical hold comments. We expect that we will be able to address the specific deficiencies identified by the FDA by submitting additional information demonstrating manufacturing capacity and product attributes that will support the high-dose group. The Company intends to submit a response to the FDA addressing the specific deficiencies in the near future, after which the FDA will have 30 days to respond. The Company does not expect that the overall timing for clinical development of SGT-001 will be affected by the partial clinical hold. Further, the partial clinical hold does not impact the Company's ability to conduct its clinical development activities of SGT-001 at low-dose levels. If the partial clinical hold is not lifted on our Phase I/II clinical trial, we will not be able to evaluate the safety, tolerability and efficacy of SGT-001 at the high dose level, which could negatively impact the development of SGT-001.

Based on results from this initial study, we will evaluate the need for future clinical trials that may include other patient populations, as well as the need for larger confirmatory trials.

Manufacturing SGT-001

The prevalence and incidence of DMD, combined with average patient weight and anticipated dosing requirements for SGT-001, result in a substantial supply need for clinical trials and, if approved, for commercial markets. To address this challenge, we developed a manufacturing process that we believe will be scalable to meet clinical and commercial production needs for SGT-001.

Our suspension-based process is founded on seminal work by scientists at the University of Florida and has been optimized for manufacturability by our internal process development scientists with the support of CDMO partners. The process consists of three steps. First, we produce two replication-incompetent Herpes Simplex Virus, or HSV, stocks, one containing our microdystropin construct and the other containing the critical elements of the AAV9. We then use these two HSV stocks to coinfect suspension-adapted human embryonic kidney cells (HEK-293), which are then purified and concentrated in our downstream process to produce our gene transfer candidate. Our team has developed the analytical testing methods needed to support consistency and strict

standards of quality and potency. We believe that this approach will increase our speed of development, ensure consistent quality and regulatory compliance, and reduce the risk of delay or unexpected production costs.

Current status and plans for clinical and commercial scale-up

We believe that our investment in our scalable manufacturing process over the last three years will allow us to minimize the need for changes throughout clinical development and upon commercialization, while ensuring supply at the high volume required at all stages. We intend to supply our clinical development program for SGT-001 with drug product produced at a cGMP-compliant facility located at a partner CDMO, which is currently operating at 250-liter scale. Our in-house scientists will continue to work to increase the productivity and efficiency of our manufacturing process. To support success, we intend to establish the capability and capacity to supply SGT-001 at commercial scale from multiple sources, including eventually building our own GMP facility to ensure redundancy and reliability.

Complementary disease-modifying therapies

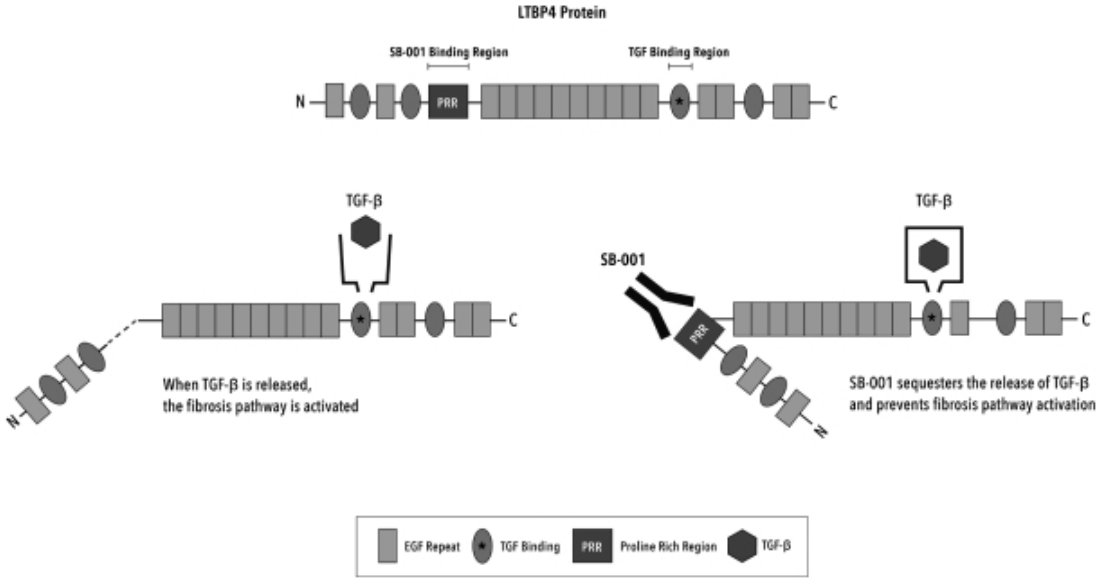
While we believe DMD disease progression can be slowed or halted by gene transfer, many patients will still suffer from the manifestations of the disease, such as tissue damage to their muscles, impaired muscle strength, inflammation, cardiac dysfunction and fibrosis. We are building a portfolio of complementary disease-modifying therapies designed to address these manifestations.

Our portfolio currently includes SB-001, a preclinical biologic product candidate that is aimed at addressing fibrosis, as well as several emerging and complementary programs. We have chosen to focus our efforts on these programs following rigorous preclinical testing and our assessment of clinical potential given natural human modifiers. If initial preclinical studies are successful, we envision initiating additional studies for our disease-modifying programs in combination with SGT-001. We continue to assess additional emerging therapeutic approaches from academia and industry through our highly focused product candidate selection process to further build our portfolio.

SB-001 (LTBP4)

SB-001 is a monoclonal antibody intended to reduce fibrosis and inflammation. It is designed to target and stabilize the LTBP4 protein. LTBP4 is highly expressed in muscle and, when stable, prevents fibrosis and inflammation by inhibiting the activation of the TGF-beta pathway.

SB-001 Mechanism



The rationale for targeting LTBP4 originated from observations in DMD natural history studies. Researchers found that subsets of patients with genetic variants in the LTBP4 gene maintained their ability to walk longer compared to patients in the study who did not. Researchers discovered that these genetic variants lead to reduced TGF-beta signaling. Elizabeth McNally, M.D., Ph.D., Director of the Center for Genetic Medicine at Northwestern University, hypothesized that stabilization of the LTBP4 protein in DMD patients could mimic the effect.

In order to assess the efficacy of potential human antibody clinical candidates in preclinical models, mice expressing the human version of LTBP4 were crossed with mdx mice to generate a DMD model that expressed human LTBP4 (hLTBP4:mdx). Preliminary studies showed that the hLTBP4:mdx animals treated with an anti-LTBP4 antibody showed significantly lower levels of fibrosis and inflammation due to the stabilization of the LTBP4 protein.

In partnership with Dr. McNally and Adimab LLC, SB-001 development efforts are underway to optimize lead candidate human immunoglobulin G, or IgG, antibodies directed against LTBP4. Additional selection and characterization are being employed to obtain high affinity antibodies. We plan to conduct preclinical *in vivo* efficacy, biodistribution and safety studies utilizing these human antibodies in hLTBP4:mdx mice beginning in 2018, following final *in vitro* antibody characterization and scale-up of manufacturing efforts.

Tools to accelerate discovery and development

We believe it is critical to invest time and research into tools designed to help us more effectively measure disease progression and the therapeutic impact of our product candidates. We are focused on developing biomarkers and sensors that will allow us to identify treatment targets faster, better measure the therapeutic impact of potential product candidates and reach therapeutic decision points earlier.

Blood-based and imaging biomarkers

We are working to identify non-invasive blood-based and imaging biomarkers that could potentially reduce or eliminate the need for muscle biopsies in clinical trials, reducing stress on patients and allowing better evaluation of potential product candidates. We are developing a platform technology that may enable the non-invasive measurement of changes associated with increased dystrophin and dystrophin-like protein expression in DMD patients by using established imaging techniques. We are also currently using leading, robust platforms to perform extensive analysis on blood-based samples to establish molecular signatures based on various stages of DMD disease progression.

Sensor-less mobility tracking

We are working to develop naturalistic motor function measurement at home with an ambient measurement system, which is based on sensors such as Microsoft Kinect. This system uses infrared technology to detect body movement and is designed to collect mobility data for DMD patients without requiring wearable sensors. If successful, this new non-invasive technology would enable us to understand in greater detail the therapeutic impact of potential product candidates as they relate to everyday activities, and could provide information to establish and measure clinical endpoints in future clinical trials.

Assistive devices

Solid Suit

We are currently developing a line of soft, wearable assistive devices that may have both functional and therapeutic benefits, with the goal of helping patients perform day-to-day activities with greater ease and preserving their muscle function. We refer to these devices as the Solid Suit. This work is being done in collaboration with technology innovators and engineering and disease experts, and is informed by input from the patient community. The Solid Suit utilizes cutting-edge technologies to power soft, light-weight comfortable exoskeletons with the potential to offset muscle fatigue and augment muscle strength. We are developing the Solid Suit in three separate components, two of which are currently in prototype development.

Intellectual property

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection for our product candidates, including SGT-001, and other know-how, to operate without infringing, misappropriating or otherwise violating the intellectual property rights of others, and to prevent others from infringing, misappropriating or otherwise violating our intellectual property rights. We also rely on patents, trade secrets, know-how and continuing technological innovation to develop and maintain our proprietary and intellectual property position.

As of December 15, 2017, we owned two pending U.S. provisional patent applications and have exclusively licensed three issued U.S. patents, one pending U.S. non-provisional patent application, and seven granted patents and eleven pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire between 2021 and 2028, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent application and U.S. provisional patent applications (assuming U.S. non-provisional patent applications are timely filed with respect to such provisional patent applications and all other applicable requirements are satisfied) would be projected to expire between 2036 and 2038, excluding any patent term adjustments and any patent term extensions.

With respect to our gene transfer programs, we exclusively licensed patent families that relate to microdystrophin genes. With respect to SGT-001, we exclusively licensed one issued U.S. patent and one pending U.S. non-provisional patent application, which generally claim the structural elements of SGT-001 and the promoter sequence used in SGT-001. This issued U.S. patent is projected to expire in 2028, excluding any

patent term adjustments and any patent term extensions. We also own one pending U.S. provisional patent application relating to SGT-001. Any U.S. patents that may issue from the pending U.S. non-provisional patent application and our pending U.S. provisional patent application (assuming a U.S. non-provisional patent application is timely filed with respect to such provisional patent application and all other applicable requirements are satisfied) would be projected to expire between 2036 and 2038, excluding any patent term adjustments and any patent term extensions. Substantive prosecution of our provisional patent application has not yet commenced at the USPTO. Our provisional patent application 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 our provisional patent application. If we do not timely file the non-provisional patent application, we may lose our priority date with respect to our provisional patent application and any patent protection on the inventions disclosed in our provisional patent application. While we intend to file a non-provisional patent application, we cannot predict whether such future patent application will result in the issuance of a patent that effectively protects SGT-001, or if such issued patent or any of our licensor's issued patents will effectively prevent others from commercializing competitive products. In any event, patent prosecution is a lengthy process, during which the scope of the claims initially submitted for examination by the USPTO offices are often significantly narrowed by the time they issue, if they issue at all.

With respect to SB-001, we do not currently own or in-license any issued patents or patent applications relating to such product candidate. We have an option to negotiate for licenses of certain patents and patent applications relating to SB-001 from Ikaika Therapeutics, LLC. If we exercise such option, Ikaika Therapeutics, LLC is only required to negotiate the terms of a potential license agreement with us for certain specified periods of time and we may be unable to enter into such a definitive license agreement within the required timeframe or under terms that are acceptable to us. If we are unable to enter into such a definitive license agreement, we will not have any license to such patents and patent applications.

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

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

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

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

We also seek trademark protection in the United States and internationally where available and when appropriate. We currently own U.S. federal registrations for the marks SOLID, SOLID GT and SOLID BIOSCIENCES and a European Union registration for the mark SOLID GT.

Strategic partnerships and collaborations/licenses:

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

University of Washington License Agreement

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

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We are required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones. There were no milestones achieved as of December 31, 2015 and 2016 and September 30, 2017. We must also pay royalties of a low single digit percentage of future sales by us and our sublicensees of products developed under the licensed patent rights. In addition, we must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to us and our sublicensees.

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

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

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

The University of Missouri License Agreement

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

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We are required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones for each product developed based on the licensed patents. There were no milestones achieved as of December 31, 2015 and 2016 and September 30, 2017. We must pay a royalty of a low single digit percentage of future sales by us or our sublicensees of products developed using the licensed patents. In addition, we must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

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

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

The University of Missouri controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents, the University of Missouri may prosecute and maintain such licensed patents at its own cost. We have the first right to enforce such licensed patents at our expense. However, any settlement, consent judgment or other voluntary disposition of litigation that materially limits the scope, validity or enforceability of the licensed patent or admits fault or wrongdoing on the part of the University of Missouri must be pre-approved in writing by the University of Missouri.

The license agreement remains in effect until the expiration of the last-to-expire patent or the abandonment of the last to be abandoned patent application licensed under the agreement. The University of Missouri may terminate the agreement, or render the license granted thereunder non-exclusive, in individual countries if we and our sublicensees fail to achieve certain milestones. We may terminate the license agreement at any time upon providing six months' written notice to the University of Missouri and paying a termination fee. Each of the University of Missouri and we may also terminate the agreement for an uncured default or breach of the agreement by the other party. Our ability to cure such breach only applies to the first two notices of such breach provided by the University of Missouri, and thereafter, the University of Missouri may terminate the agreement for our default or breach of the agreement upon thirty days' written notice without an opportunity to cure such default or breach.

The University of Michigan License Agreement

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

In consideration for the rights granted by the agreement, we paid a one-time license fee and a separate fee to cover past patent prosecution costs. We recorded the upfront license fee as a research and development expense in 2016. We are required to reimburse the University of Michigan for costs incurred in applying for, prosecuting and maintaining patents, and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones. There were no milestones achieved as of December 31, 2016 and September 30, 2017. We must also pay royalties of a low single-digit percentage of future sales by us or our sublicensees of products developed using the licensed rights, with a minimum annual royalty after certain milestones are achieved. In addition, we must pay an annual maintenance fee in any year in which the minimum annual royalty is not reached.

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

We are obligated to use commercially reasonable efforts to bring one or more products based on the licensed patents to market through a diligence program for utilizing the licensed patents, to continue diligent marketing efforts throughout the term of the agreement, and to make reasonable amounts of such products commercially available, in each case consistent with prudent business practices and judgment.

The University of Michigan controls the prosecution and maintenance of the licensed patents in consultation with us and at our expense. In countries in which we have not requested prosecution or maintenance of licensed patents, the University of Michigan may prosecute and maintain such licensed patents at its own cost. We have the first right to enforce such licensed patents at our expense. However, we may only enter into a settlement with the advice and consent of the University of Michigan.

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. The University of Michigan may terminate the agreement upon our uncured material breach of the agreement, including failure to make required payments under the agreement or to achieve certain milestones, or if we become insolvent or bankrupt. We may terminate the license agreement at any time upon providing sixty days' written notice to the University of Michigan.

Harvard College License Agreements

In 2016 and 2017, we entered into license agreements with the President and Fellows of Harvard College, or Harvard College, under which we obtained non-exclusive, royalty-bearing, sublicensable, worldwide licenses to use certain intellectual property owned by Harvard College to develop, manufacture, and commercialize products for use in the treatment of DMD.

In consideration for the rights granted by each agreement, we paid one-time, non-refundable license fees, which were recorded as a research and development expense in 2016 and 2017. We are required to pay an annual license maintenance fee until certain milestones are achieved, after which time the annual maintenance fee will increase annually. Such annual maintenance fees will further increase if we grant certain rights to a sublicensee or strategic partner with whom we collaborate on the development and commercialization of licensed products. The annual maintenance fees are creditable against royalty payments. We also must pay milestone payments

within thirty days after achieving certain milestones. There were no milestones achieved as of December 31, 2016 and September 30, 2017 under either agreement. We must pay a royalty on future sales by us or our sublicensees of products developed using the licensed technology.

The license agreements each remain in effect for an initial term of fifteen years, with automatic three-year renewal periods thereafter unless one of the parties provides notice of non-renewal. We may terminate the license agreements at any time upon providing sixty days' written notice to Harvard College. Harvard College may terminate the agreements in the event we become bankrupt or insolvent. Both Harvard College and we may also terminate the agreements for an uncured material breach of the agreements by the other party.

Other License Agreements

In 2016, we entered into a license agreement with Life Technologies Corporation, or Life Technologies. In consideration for obtaining a non-exclusive, royalty-free, worldwide license to use certain technologies and associated know-how to develop our product candidates, we paid a one-time, non-refundable license fee. This fee was recorded as a research and development expense in 2016. The license agreement will remain effective in perpetuity unless earlier terminated. Life Technologies has the right to terminate the agreement upon our material, uncured breach of the agreement or in the event that it determines that continued performance of the agreement may violate any laws. We are obligated to diligently pursue regulatory approval necessary for the development, manufacture and sale of the licensed products. We have the right to terminate the agreement at any time upon providing thirty days' written notice to Life Technologies.

Competition

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

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

We are aware of several companies and research institutions focused on developing systemic gene transfers for DMD, including Pfizer Inc. and Sarepta Therapeutics, Inc. Any advances in gene transfer technology made by a competitor may be used to develop therapies that could compete with our lead product candidate.

For our gene transfer product candidate, the main competitors are:

- We are aware that Pfizer Inc. is developing PF-06939926, an AAV-mediated microdystrophin gene transfer.
- We are aware that Sarepta Therapeutics, Inc. has entered into a research and option agreement with Nationwide Children's Hospital for AAVrh74.MHCK7.micro-Dystrophin, its AAV-mediated

microdystrophin gene transfer program. In January 2018, Sarepta announced that Nationwide Children's Hospital had begun dosing patients in a Phase I/II clinical trial designed to assess the safety and tolerability of AAVrh74.MHCK7.micro-Dystrophin in individuals with DMD.

- We are aware that Sarepta Therapeutics, Inc. and Genethon have entered into a research collaboration to develop an AAV-mediated microdystrophin gene transfer.

In addition to the investigational gene transfer programs discussed above, there are two therapies, which are intended to be disease modifying, that are currently approved for DMD by certain regulators. These products are eteplirsen (EXONDYS 51) and ataluren (Translarna), each of which is indicated for approximately 13% of DMD patients.

Government regulation and product approval

U.S. government regulation and product approval

In the United States, biologic products including gene therapy products, such as our lead product candidate, are regulated by the FDA under the Federal Food, Drug, and Cosmetic Act, or FD&C Act, and the Public Health Service Act, or PHS Act, as well as by other federal, state and local statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biologic products. FDA approval must be obtained before conducting human clinical testing of biologic products. Additionally, each clinical trial protocol for a gene therapy product candidate is reviewed by the FDA and, in limited instances, the NIH through its RAC. FDA approval also must be obtained before marketing of biologic products.

Within the FDA, the CBER regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the OTAT and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. CBER works closely with the NIH and the RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. Although the FDA has not yet approved any human gene therapy product for sale, it has provided guidance for the development of gene therapy products. This guidance includes a growing body of guidance documents on chemistry, manufacturing and control, or CMC, clinical investigations and other areas of gene therapy development, all of which are intended to facilitate the industry's development of gene therapy products.

U.S. biologic products development process

The process required by the FDA before a biologic product may be marketed in the United States generally involves the following:

- completion of preclinical laboratory tests and *in vivo* studies according to the FDA's GLPs and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an application for an IND, which allows human clinical trials to begin unless the FDA objects within 30 days;
- approval by an IRB reviewing each clinical site before each clinical trial may be initiated;
- approval by an IBC assessing the safety of the clinical research and identifying any potential risk to public health or the environment;
- performance of adequate and well controlled human clinical trials according to the FDA's regulations commonly referred to as GCPs and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biologic product for its intended use;

- preparation and submission to the FDA of a BLA, for marketing approval that includes substantive evidence of safety, purity and potency from results of preclinical testing and clinical trials, and detailed information about the CMC for the product, reports of the outcomes and full data sets of the clinical trials and proposed labeling and packaging for the product;
- review of the product candidate by an FDA advisory committee, if applicable;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biologic product candidate is produced to assess compliance with cGMP requirements and to assure that the facilities, methods and controls are adequate to preserve the biologic product candidate's identity, safety, strength, quality and purity;
- potential FDA audit of the nonclinical and clinical trial sites that generated the data in support of the BLA;
- payment of user fees; and
- FDA review and approval, or licensure of, the BLA.

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as *in vivo* studies to assess the potential safety and activity of the product candidate and to establish a rationale for therapeutic use. The conduct of certain nonclinical studies must comply with federal regulations and requirements, including GLPs.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documents must be submitted to, and the study registered with, the NIH Office of Biotechnology Activities, or OBA, pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules, or NIH Guidelines. Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA. However, many companies and other institutions, not otherwise subject to the NIH Guidelines, voluntarily follow them. NIH is responsible for convening the RAC that discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA website and may be accessed by the public.

The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical tests may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical trial on a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. With gene therapy protocols, if the FDA allows the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that the sponsor delays initiation of the protocol until after completion of the RAC review process. The FDA also may impose clinical holds on a biologic product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA.

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

clinical hold. The FDA placed SGT-001 on a partial clinical hold that permits us to proceed with administering our proposed low dose to patients, but that prohibits us from administering SGT-001 at our proposed higher dose. See "Risk Factors—Risks related to the development of our product candidates—The FDA placed the SGT-001 Phase I/II clinical trial on a partial clinical hold requiring us to submit additional CMC information that demonstrates that manufacturing capacity and product attributes can support the high-dose group."

Human clinical trials under an IND

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

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative, reviews and approves the study protocol and must monitor the clinical trial until completed. Clinical trials involving recombinant DNA also must be reviewed by an IBC a local institutional committee that reviews and oversees basic and clinical research and utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

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

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

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

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data and clinical trial investigators. Annual progress reports detailing the results of the clinical trials must be submitted to the FDA.

Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other trials, *in vivo* laboratory tests or *in vitro* testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information.

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

Additional regulation for gene therapy clinical trials

In addition to the regulations discussed above, there are a number of additional standards that apply to clinical trials involving the use of gene therapy. The FDA has issued various guidance documents regarding gene therapies, which outline additional factors that the FDA will consider at each of the above stages of development, which relate to, among other things: the proper preclinical assessment of gene therapies; the CMC information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by ten years of annual queries, either in person or by questionnaire.

The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System, which includes information on gene therapy trials and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these trials.

Compliance with cGMP requirements

Manufacturers of biologics must comply with applicable cGMP regulations, including quality control and quality assurance and maintenance of records and documentation. Manufacturers and others involved in the manufacture and distribution of such products also must register their establishments with the FDA and certain state agencies. Both domestic and foreign manufacturing establishments must register and provide additional information to the FDA upon their initial participation in the manufacturing process. Establishments may be subject to periodic, unannounced inspections by government authorities to ensure compliance with cGMP requirements and other laws. Discovery of problems may result in a government entity placing restrictions on a product, manufacturer or holder of an approved BLA, and may extend to requiring withdrawal of the product from the market. The FDA will not approve a BLA unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specification.

Concurrent with clinical trials, companies usually complete additional preclinical studies and must also develop additional information about the physical characteristics of the biologic product candidate as well as finalize a process for manufacturing the product candidate in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents or of causing other adverse events with the use of biologic products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other requirements, the sponsor must

develop methods for testing the identity, strength, quality, potency and purity of the final biologic product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biologic product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. review and approval processes

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

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. According to the FDA's fee schedule, effective from October 1, 2017 through September 30, 2018, the user fee for an application requiring clinical data, such as a new drug application, is $2,421,495. PDUFA also imposes an annual product fee for biologics and an annual establishment fee on facilities used to manufacture prescription biologics. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for product candidates designated as orphan drugs, unless the product candidate also includes a non-orphan indication.

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

The FDA reviews the BLA to determine, among other things, whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biologic products or biologic products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biologic product approval process, the FDA also will determine whether a REMS, is necessary to assure the safe use of the biologic product. REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events and whether the product is a new molecular entity. A REMS could include medication guides, physician communication plans and elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product candidate is manufactured. The FDA will not approve the product candidate unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product

within required specifications. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND study requirements and GCP requirements. cGMP, GLP and GCP compliance requires significant expenditure of time, money and effort in the areas of training, recordkeeping, production and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than how we would interpret the same data. On the basis of the BLA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the biologic product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the BLA, the FDA will issue an approval letter. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes; or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

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

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

Pediatric exclusivity

Under the Biologics Price Competition and Innovation Act, or BPCIA, which was part of the Health Care Reform Law, biologics, such as our product candidates, may be eligible for pediatric exclusivity, an incentive intended to encourage medical product research for children. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods applicable to biological products under the BPCIA—namely, the four-year period during which the FDA will not consider an application for a biosimilar product, and the 12-year period during which the FDA will not approve a biosimilar application. This six-month exclusivity, which runs from the end of these exclusivity protection periods, may be granted based on the voluntary completion of a pediatric trial in accordance with an FDA-issued "written request" for such a trial.

Orphan drug designation

Under the Orphan Drug Act, the FDA may designate a biologic product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a biologic product available in the United States for treatment of the disease or condition will be recovered from sales of the product). Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity or if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. Competitors, however, may receive approval of different products for the same indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan medicinal product status in the European Union has similar, but not identical, benefits.

Expedited development and review programs

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

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

- *Breakthrough therapy designation.* To qualify for the breakthrough therapy program, product candidates must be intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence must indicate that such product candidates may demonstrate substantial improvement on one or more clinically significant endpoints over existing therapies. The FDA will seek to ensure the sponsor of a breakthrough therapy product candidate receives intensive guidance on an efficient drug development program, intensive involvement of senior managers and experienced staff on a proactive, collaborative and cross-disciplinary review and rolling review.
- *Priority review*. A product candidate is eligible for priority review if it treats a serious condition and, if approved, it would be a significant improvement in the safety or effectiveness of the treatment, diagnosis or prevention compared to marketed products. FDA aims to complete its review of priority review applications within six months as opposed to 10 months for standard review.
- *Accelerated approval.* Drug or biologic products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval. Accelerated approval means that a product candidate may be approved on the basis of adequate and well controlled clinical trials establishing that the product candidate has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or

on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity and prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well controlled post-marketing clinical trials. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials.

Fast Track designation, breakthrough therapy designation and accelerated approval do not change the standards for approval but may expedite the development or approval process.

Post-approval requirements

After regulatory approval of a product is obtained, there may be a number of post-approval requirements. For example, as a condition of approval of a BLA, the FDA may require post-marketing testing and surveillance to monitor the product's safety or efficacy. In addition, holders of an approved BLA are required to keep extensive records, to report certain adverse reactions and production problems to the FDA, to provide updated safety and efficacy information and to comply with requirements concerning advertising and promotional labeling for their products. Also, quality control and manufacturing procedures must continue to conform to cGMP regulations and practices, as well as the manufacturing conditions of approval set forth in the BLA. The FDA periodically inspects manufacturing facilities to assess compliance with cGMP, which impose certain procedural, substantive and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

U.S. patent term restoration and marketing exclusivity

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

Government regulation outside of the U.S.

In addition to regulations in the United States, a variety of regulations in other jurisdictions govern, among other things, clinical trials and any commercial sales and distribution of biologic products. Because biologically sourced materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not a sponsor obtains FDA approval for a product, a sponsor must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application, much like the IND, prior to the commencement of human clinical trials. In the European Union, for example, a request for a Clinical Trial Authorization, or CTA, must be submitted to the competent regulatory authorities and the competent Ethics Committees in the European Union Member States in which the clinical trial takes place, much like FDA and the IRB, respectively. Once the CTA request is

approved in accordance with the European Union and the European Union Member State's requirements, clinical trial development may proceed.

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

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

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

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

The requirements and processes governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCPs and the applicable regulatory requirements of the country or countries in which the clinical trial is performed, as well as the ethical principles that have their origin in the Declaration of Helsinki (whichever provides the greater protection to the clinical trial participants).

U.S. regulations affecting certain federally funded programs, such as Medicare and Medicaid:

Manufacturers with products that are reimbursed by U.S. federally funded programs such as Medicare and Medicaid are subject to regulation by CMS and enforcement by the U.S. Department of Health and Human Services Office of the Inspector General, or HHS OIG. In the event our product candidates are approved, regulation by CMS and enforcement by HHS OIG would be relevant to us. Some of these laws, referred to as false claims laws, prohibit the submission or causing the submission of false or fraudulent claims for reimbursement to federal, state and other health care payors and programs. Other laws, referred to as anti-kickback laws, prohibit soliciting, offering, receiving or paying remuneration in order to induce the referral of a patient or ordering, purchasing, leasing or arranging for, or recommending ordering, purchasing or leasing of, items or services that are paid for by federal, state and other health care payors and programs.

The federal Anti-Kickback Law prohibits providers and others from directly or indirectly soliciting, receiving, offering or paying any remuneration with the intent of generating referrals or orders for services or

items covered by a government health care program. Many states have enacted similar laws. Courts have interpreted this law very broadly, including by holding that a violation has occurred if even one purpose of the remuneration is to generate referrals, even if there are other lawful purposes. There are statutory and regulatory exceptions, or safe harbors, that outline arrangements that are deemed lawful. However, the fact that an arrangement does not fall within a safe harbor does not necessarily render the conduct illegal under the Anti-Kickback Law. In sum, even common business arrangements, such as discounted terms and volume incentives for customers in a position to recommend or choose drugs for patients, such as physicians and hospitals, can result in substantial legal penalties, including, among others, exclusion from Medicare and Medicaid programs, and arrangements with referral sources must be structured with care to comply with applicable requirements. Also, certain business practices, such as payment of consulting fees to health care providers, sponsorship of educational or research grants, charitable donations, interactions with health care providers that prescribe products for uses not approved by the FDA and financial support for continuing medical education programs, must be conducted within narrowly prescribed and controlled limits to avoid the possibility of wrongfully influencing health care providers to prescribe or purchase particular products or as a reward for past prescribing. Violations of the Anti-Kickback Law may be punished by civil and criminal penalties or exclusion from participation in federal health care programs, including Medicare and Medicaid.

It is a violation of the FCA for any entity to present or cause to be presented knowingly false claims for payment to the federal government. In addition, the Health Care Reform Law amended the FCA to create a cause of action against any person who knowingly makes a false statement material to an obligation to pay money to the government or knowingly conceals or improperly decreases an obligation to pay or transmit money or property to the government. For the purposes of these recent amendments, an obligation includes an identified overpayment, which is defined broadly to include any funds that a person receives or retains under Medicare and Medicaid to which the person, after applicable reconciliation, is not entitled. The FCA is commonly used to sue those who submit allegedly false Medicare or Medicaid claims, as well as those who induce or assist others to submit a false claim. False claims can result not only from non-compliance with the express requirements of applicable governmental reimbursement programs, such as Medicaid or Medicare, but also from non-compliance with other laws, such as the Anti-Kickback Law or laws that require quality care in service delivery. The fraud and abuse regulations have been subject to varying interpretations, as well as heightened enforcement activity over the past few years, and significant enforcement activity has been the result of relators, who serve as whistleblowers by filing complaints in the name of the United States (and if applicable, particular states) under federal and state false claims laws. Under the federal FCA, relators can be entitled to receive up to 30% of total recoveries. Also, violations of the FCA can result in treble damages and civil penalties. Most states have adopted similar state false claims laws, and these state laws have their own penalties that may be in addition to federal FCA penalties.

The Health Care Reform Law significantly strengthened the federal FCA and federal Anti-Kickback Law provisions, which could lead to the possibility of increased whistleblower or relator suits, and among other things made clear that a federal Anti-Kickback Law violation can be a basis for federal FCA liability.

Environmental regulations

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

Employees

As of December 31, 2017, we had 60 full-time employees, 19 of whom hold Ph.D. or M.D. degrees, 22 of whom are engaged in research and development activities, four of whom are engaged in clinical and regulatory

activities and 34 of whom are engaged in business development, legal, finance, information systems, human resources or administrative support activities.

Facilities

We lease our corporate headquarters, which consists of approximately 6,000 square feet in Cambridge, Massachusetts. Our lease expires in January 2018. We also lease approximately 6,000 square feet of additional office and laboratory space in Cambridge, Massachusetts, as well as several smaller office spaces. In January 2018, we entered into a new lease agreement for approximately 9,500 square feet of laboratory space in Cambridge, Massachusetts, with an initial term of five years and the option to extend for one additional two-year term. We are currently exploring a future location for our corporate headquarters.

Legal proceedings

From time to time, we may be involved in various legal proceedings arising out of our operations. We are not currently a party to any 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.

MANAGEMENT

Executive officers and directors

Set forth below are the names, ages and positions of our executive officers and directors as of January 1, 2018.

Name	Age	Position(s) held
Executive Officers		
Ilan Ganot	44	Co-founder, Chief Executive Officer and Director
Gilad Hayeem	50	Co-founder, President and Director
Alvaro Amorrortu	45	Chief Operating Officer
Carl Morris, Ph.D.	47	Chief Scientific Officer
Joel Schneider, Ph.D.	33	Chief Technology Officer and Head of Exploratory Research and Development
Jorge A. Quiroz, M.D.	48	Chief Medical Officer
Jennifer Ziolkowski	43	Chief Financial Officer, Treasurer and Assistant Secretary
Non-Employee Directors		
Andrey Zarur, Ph.D.	47	Co-founder and Chairman of the Board of Directors
Matthew Arnold	48	Director
Robert Huffines	52	Director
Adam Koppel, M.D., Ph.D.	48	Director
Rajeev Shah	40	Director
Adam Stone	38	Director
Lynne Sullivan	51	Director

Executive officers

Ilan Ganot is one of our co-founders and has served as our Chief Executive Officer and as a member of our board of directors since our inception in 2013. Previously, Mr. Ganot served as an investment banker at JPMorgan Chase & Co. from September 2011 to September 2013. From October 2008 to August 2011, Mr. Ganot served as a banker at Nomura Securities Co., Ltd., and from September 2003 to September 2008, at Lehman Brothers. Mr. Ganot received his M.B.A. from London Business School and holds law and business degrees from the Interdisciplinary Center Herzliya, Israel. Mr. Ganot also practiced corporate law in Israel and was a Captain in the Israeli Defense Forces. He is qualified to serve on our board of directors because of his personal dedication to improving treatments available for DMD patients and his extensive leadership experience in the financial sector.

Gilad Hayeem is one of our co-founders and has served as our President and as a member of our board of directors since our inception in 2013. Mr. Hayeem also has served as Chief Executive Officer of Waverly Capital, a family office, since January 2012. Mr. Hayeem received his M.B.A. from City, University of London and his undergraduate degree from the University of Leeds. Mr. Hayeem is qualified to serve on our board of directors because of his extensive knowledge of our company based on his role as co-founder and President and his extensive leadership experience.

Alvaro Amorrortu has served as our Chief Operating Officer since January 2017. Mr. Amorrortu served as our Senior Vice President of Operations from November 2015 to December 2016. Prior to joining us, he served as Vice President of Consulting for IMS Health from July 2015 to November 2015 and Vice President of Campbell Alliance (InVentiv Health Consulting) from July 2012 to June 2015. He was at the Monitor Group, a management consulting firm, from April 2003 to May 2012 where he held various positions, including Associate Partner. From 1995 to 2000, Mr. Amorrortu gained significant experience in project engineering and managing food-processing manufacturing facilities through various positions at Molinos Rio de la Plata and Trigalia (subsidiaries of Bunge Group and Cargill, respectively). Mr. Amorrortu received his M.B.A. from The Wharton School of the University of Pennsylvania and received an M.S. from the Instituto Tecnologico de Buenos Aires, Argentina.

Carl Morris, Ph.D. has served as our Chief Scientific Officer since June 2017, and previously served as our Senior Vice President of Research and Development from September 2015 to June 2017. Prior to joining us, Dr. Morris held various leadership positions within Pfizer Inc.'s Rare Disease Research Unit from January 2010 to August 2015, including serving as a Senior Director, Director and Senior Principal Scientist. Prior to Pfizer, Dr. Morris held various positions within the Tissue Repair unit at Wyeth Pharmaceuticals, Inc. Dr. Morris was an Assistant Professor at Boston University School of Medicine and a founding faculty member of the Muscle and Aging Research Unit. He is also co-founder and a member of the board of directors of Breed Nutrition Inc. Dr. Morris holds a B.A. in Biology from Franklin Pierce College and a Ph.D. in Physiology from UCLA.

Joel Schneider, Ph.D. has served as our Chief Technology Officer and Head of Exploratory Research and Development since June 2017. Dr. Schneider also served as an Analyst from March 2014 to March 2015, a Director from March 2015 to January 2017 and our Vice President of Research and Development from January 2017 to June 2017. Prior to joining Solid, Dr. Schneider completed a postdoctoral fellowship at Harvard University in the Department of Stem Cell and Regenerative Biology from January 2013 to 2014. He holds a Ph.D. in Cell Biology and Molecular Medicine from Rutgers University and a B.A. in Biology from Brandeis University.

Jorge A. Quiroz, M.D. has served as our Chief Medical Officer since January 2016. Prior to joining us, Dr. Quiroz served as the Head of Neurodevelopment & Psychiatry, Translational Medicine Neurosciences at F. Hoffmann-La Roche AG from 2014 and, prior to that, as Head of Psychiatry from 2012 to 2014 and Translational Medicine Leader from 2009 to 2011 at Hoffmann-La Roche. From 2007 to 2009, he served as the Director of Johnson & Johnson's Pharmaceutical Research & Development LLC and from 2005 to 2007 he served as its Associate Director. Dr. Quiroz holds a medical degree from the Pontifical Catholic University of Chile and he completed his medical training as a Research Fellow at the Laboratory of Molecular Pathophysiology, Mood and Anxiety Disorders Program, at the NIH in Bethesda, Maryland from February 2001 to May 2005. He is board certified in Psychiatry by the National Commission for Certification of Medical Specialties. He also holds an M.B.A. dual degree from Columbia University and the London Business School.

Jennifer Ziolkowski has served as our Chief Financial Officer, Treasurer and Assistant Secretary since May 2017. Prior to joining us, she served as the Head of Sales Operations, North America for Philips Healthcare from 2014 to 2017 and as its Senior Director of Finance, North America from 2012 to 2014. Ms. Ziolkowski served as Controller of Medical Consumables and Sensors from 2010 to 2012, Director of Finance of Imaging Systems from 2008 to 2010, Senior Director of Finance and Corporate Controller from 2007 to 2008 at TransMedics, Inc. and held various finance and corporate development leadership positions at Cytyc Corporation, a medical technology company, from 2001 to 2007. From 1996 to 2001, Ms. Ziolkowski gained significant experience at PricewaterhouseCoopers LLP where she served as a Senior Transaction Services Consultant and as Audit Senior and Staff in the Boston Technology Group. Ms. Ziolkowski holds a B.S. in Accounting from Boston College and is a Certified Public Accountant.

Non-employee directors

Andrey Zarur, Ph.D. is one of our co-founders and has served as the Chairman of our board of directors since our inception in 2013. Dr. Zarur co-founded GreenLight Biosciences in August 2008, and currently serves as its Chairman and Chief Executive Officer. From January 2006 to August 2014, he served as Managing General Partner of Kodiak Venture Partners. Dr. Zarur is also Chairman of the board for Lumicell Inc. Dr. Zarur holds an M.S. and a Ph.D. from Massachusetts Institute of Technology and an undergraduate degree from Universidad Nacional Autónoma de México. Mr. Zarur is qualified to serve on our board of directors based on his over 20 years of experience in leading companies from clinical-stage drug development to global commercialization.

Matthew Arnold is a founding member of Solid and has served as a member of our board of directors since our inception in 2013. A former energy executive, since 2009, Mr. Arnold has been actively working with startup

businesses in the United Kingdom and Europe, primarily in the technology and clean tech sectors. He holds an M.S. from the University of Virginia and a B.A. from Duke University. Mr. Arnold is qualified to serve on our board of directors because of his extensive management and board experience with startup companies and his background in finance.

Robert Huffines has served as a member of our board of directors since December 2013. Mr. Huffines joined J.P. Morgan in 1992 and currently serves as the Global Chairman of Investment Banking, a position he has held since February 2017. Throughout his career at J.P. Morgan, Mr. Huffines has held various leadership positions, including serving as Co-Head of the Global Healthcare Investment Banking Group from 2002 to 2010 and Vice Chairman from 2011 to January 2017. Mr. Huffines received an M.B.A. from the University of Virginia and a B.A. from the University of North Carolina. Mr. Huffines is qualified to serve on our board of directors based on his over 25 years of experience advising healthcare companies and his leadership experience.

Adam Koppel, M.D., Ph.D. has served as a member of our board of directors since October 2017. Dr. Koppel rejoined Bain Capital in 2016 as a Managing Director of Bain Capital Life Sciences. He initially joined Bain Capital Public Equity in 2003 where he was a leader within the healthcare sector until mid-2014. During the period mid-2014 to mid-2016, Dr. Koppel worked at Biogen where he served as EVP of Corporate Development and Chief Strategy Officer. Prior to joining Bain Capital in 2003, Dr. Koppel was an Associate Principal at McKinsey & Co. in New Jersey where he served a variety of healthcare companies. Dr. Koppel currently serves on the Board of Directors of Trevena, Inc. and Dicerna Pharmaceuticals, Inc. Dr. Koppel received an M.D. and Ph.D. in Neuroscience from the University of Pennsylvania School of Medicine. He also received an M.B.A. from The Wharton School at the University of Pennsylvania, where he was a Palmer Scholar. He graduated magna cum laude from Harvard University with an A.B. and A.M. in History and Science. Dr. Koppel is qualified to serve on our board of directors because of his extensive leadership experience, his public company board experience and his experience working in the healthcare sector.

Rajeev Shah has served as a member of our board of directors since March 2017. Mr. Shah is a Managing Director and Portfolio Manager at RA Capital Management, LLC, or RA Capital. Prior to joining RA Capital in 2004, Mr. Shah was a Senior Project Leader at Altus Pharmaceuticals Inc., a spin-off of Vertex Pharmaceuticals Inc., from 2001 to 2004. Mr. Shah is currently a member of the board of directors of the public companies Ra Pharmaceuticals, Inc. and Kalvista Pharmaceuticals, Inc. Mr. Shah holds a B.A. in Chemistry from Cornell University. Mr. Shah is qualified to serve on our board of directors because of his extensive leadership experience, his public company board experience and his experience investing in life science companies.

Adam Stone has served as a member of our board of directors since November 2015. Mr. Stone is currently the Chief Investment Officer of Perceptive Advisors, where he has worked since May 2006. Mr. Stone received a B.A. from Princeton University. Mr. Stone is qualified to serve on our board of directors because of his extensive experience developing early-stage biotech and health care companies.

Lynne Sullivan has served as a member of our board of directors since November 2015. Since September 2016, Ms. Sullivan has served as Biogen, Inc.'s Senior Vice President of Finance, where she also served as Vice President of Tax and Corporate Finance from February 2015 to March 2016 and Vice President of Tax from April 2008 to February 2015. Ms. Sullivan is currently a member of the board of directors of resTORbio, Inc. She received an M.S. in Taxation from Bentley University and a B.S.B.A. from Suffolk University. Ms. Sullivan was a Certified Public Account for over 20 years. Ms. Sullivan is qualified to serve on our board of directors because of her extensive experience in public accounting and financial expertise.

Scientific Advisory Board

We have established a scientific advisory board comprised of a world-class team of experts, which includes leading immunologists, molecular biologists, clinicians and gene therapy researchers. We regularly seek advice and input from these experienced leaders on matters related to our research and development programs. Our Scientific Advisory Board currently consists of Jeffrey Chamberlain, Ph.D. (University of Washington), Chairman of our Scientific Advisory Board, Jeff Bluestone, Ph.D. (University of California, San Francisco),

Ronald D. Cohn, M.D. (Hospital for Sick Children), Dongsheng Duan, Ph.D. (University of Missouri), Michael Lawlor, M.D., Ph.D. (Medical College of Wisconsin), Carrie Miceli, Ph.D. (University of California, Los Angeles), Geoffrey Slaff, Ph.D. and Lawrence A. Turka, M.D. (Massachusetts General Hospital). James M. Wilson, M.D., Ph.D., the former head of our Scientific Advisory Board, resigned on January 11, 2018 citing his emerging safety concerns about the possible risks of high systemic dosing of AAV.

Composition of the board of directors

Our board currently consists of nine members, each of whom serves as a director pursuant to the board composition provisions of our Third Amended and Restated LLC Agreement, or the LLC Agreement, of Solid Biosciences, LLC. The LLC Agreement will terminate upon our Corporate Conversion and, thereafter, our directors will be elected by vote of our common stockholders.

Director independence

Applicable NASDAQ rules require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, NASDAQ rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent and that audit committee members also satisfy independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act. The NASDAQ independence definition includes a series of objective tests, such as that the director is not, and has not been for at least three years, one of our employees and that neither the director nor any of his family members has engaged in various types of business dealings with us. In addition, under applicable NASDAQ rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Our board of directors has determined that all members of the board of directors, except Ilan Ganot, Gilad Hayeem and Andrey Zarur, are independent directors, as defined under applicable NASDAQ rules. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our common stock by each non-employee director.

Prior to the effectiveness of the registration statement of which this prospectus forms a part, we expect that the composition of our committees will comply with all applicable requirements of NASDAQ and the rules and regulations of the SEC. There are no family relationships among any of our directors or executive officers.

Classified board of directors

In accordance with the terms of our charter and bylaws, which will become effective prior to the effectiveness of the registration statement of which this prospectus forms a part, our board of directors will be divided into three classes, each of which will consist, as nearly as possible, of one-third of the total number of directors constituting our entire board of directors and directors in each class will serve staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following such election. Our directors will be divided among the three classes as follows:

- Class I, which will consist of Mr. Huffines, Dr. Koppel and Mr. Shah, whose terms will expire at the first annual meeting of stockholders to be held following the completion of this offering;
- Class II, which will consist of Mr. Arnold, Mr. Stone and Ms. Sullivan, whose terms will expire at the second annual meeting of stockholders to be held following the completion of this offering; and

- Class III, which will consist of Mr. Ganot, Mr. Hayeem and Dr. Zarur, whose terms will expire at the third annual meeting of stockholders to be held following the completion of this offering.

Our bylaws, which will become effective prior to the effectiveness of the registration statement of which this prospectus forms a part, will provide that the authorized number of directors may be changed only by resolution approved by a majority of our board of directors. Any additional directorships resulting from an increase in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors.

The division of our board of directors into three classes with staggered three-year terms may delay or prevent stockholder efforts to effect a change of our management or a change in control.

Role of our board of directors in risk oversight

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure. Our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements. Our compensation committee evaluates risks associated with our compensation practices and policies.

Committees of our board of directors

Audit committee

Our audit committee consists of Ms. Sullivan, Dr. Koppel and Mr. Shah, with Ms. Sullivan serving as chair of the audit committee. Our board of directors has determined that each of these individuals meets the independence requirements of the Sarbanes-Oxley Act, Rule 10A-3 under the Exchange Act, and the applicable listing standards of the NASDAQ. Each member of our audit committee can read and understand fundamental financial statements in accordance with the NASDAQ audit committee requirements. In arriving at this determination, the board has examined each audit committee member's employment and other experience. Our board of directors has determined that Ms. Sullivan qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the NASDAQ listing rules. In making this determination, our board has considered Ms. Sullivan's formal education and previous and current experience in financial roles. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

The functions of our audit committee include, among other things:

- evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;
- reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;
- monitoring the rotation of partners of our independent auditors on our engagement team as required by law;
- prior to engagement of any independent auditor, and at least annually thereafter, reviewing relationships that may reasonably be thought to bear on their independence, and assessing and otherwise taking the appropriate action to oversee the independence of our independent auditor;

- reviewing our annual and quarterly financial statements and reports, including the disclosures contained under the caption "Management's Discussion and Analysis of Financial Condition and Results of Operations," and discussing the statements and reports with our independent auditors and management;
- reviewing with our independent auditors and management any significant issues that arise regarding accounting principles and financial statement presentation and matters concerning the scope, adequacy and effectiveness of our financial controls;
- reviewing with management and our auditors any earnings announcements and other public announcements regarding material financial developments;
- establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;
- preparing the audit committee report that the SEC requires in our annual proxy statement;
- reviewing and providing oversight of any related-person transactions in accordance with our related-person transaction policy and reviewing and monitoring compliance with legal and regulatory requirements, including our code of business conduct and ethics;
- reviewing our major financial risk exposures, including the guidelines and policies to govern the process by which risk assessment and risk management is implemented;
- reviewing on a periodic basis our investment policy; and
- reviewing and evaluating on an annual basis the performance of the audit committee and the audit committee charter.

We believe that the composition and functioning of our audit committee complies with all applicable requirements of the Sarbanes-Oxley Act and all applicable SEC and NASDAQ rules and regulations.

Compensation committee

Our compensation committee consists of Dr. Koppel, Mr. Shah and Mr. Stone, with Dr. Koppel serving as chair of the compensation committee. Each of Mssrs. Shah and Stone is a non-employee director, as defined in Rule 16b-3 promulgated under the Exchange Act. Our board of directors has determined that each of these individuals is independent as defined under the applicable listing standards of NASDAQ, including the standards specific to members of a compensation committee. The functions of our compensation committee include, among other things:

- reviewing, modifying and approving or making recommendations to the full board of directors regarding our overall compensation strategy and policies;
- reviewing, modifying and approving or making recommendations to the full board of directors regarding the compensation and other terms of employment of our chief executive officer or our other executive officers;
- reviewing, modifying and approving or making recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- reviewing and approving or making recommendations to the full board of directors regarding the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;
- evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;

- reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our independent board members;
- establishing policies with respect to votes by our stockholders to approve executive compensation to the extent required by the Exchange Act and, if applicable, determining our recommendations regarding the frequency of advisory votes on executive compensation;
- reviewing and assessing the independence of compensation consultants, legal counsel and other advisors to the compensation committee as required by the Exchange Act;
- administering our equity incentive plans;
- establishing policies with respect to our equity compensation arrangements;
- reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policies and strategy in achieving expected benefits to us;
- reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- reviewing with management and approving our disclosures under the caption "Compensation Discussion and Analysis" in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;
- preparing the compensation committee report that the SEC requires in our annual proxy statement; and
- reviewing and evaluating on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations.

Nominating and corporate governance committee

Our nominating and corporate governance committee consists of Mr. Stone, Mr. Arnold and Ms. Sullivan, with Mr. Stone serving as chair of the nominating and corporate governance committee. Our board of directors has determined that each of these individuals is independent as defined under the applicable listing standards of NASDAQ and SEC rules and regulations. The functions of our nominating and corporate governance committee include, among other things:

- determining the minimum qualifications for service on our board of directors;
- evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- identifying, evaluating, nominating and recommending candidates for membership on our board of directors;
- evaluating nominations by stockholders of candidates for election to our board of directors;
- considering and assessing the independence of members of our board of directors;
- developing a set of corporate governance policies and principles and recommending to our board of directors any changes to such policies and principles;
- overseeing, at least annually, the self-evaluation process of the board of directors and its committees;
- overseeing our code of business conduct and ethics and approving any waivers thereof;
- considering questions of possible conflicts of interest of directors as such questions arise; and
- reviewing and evaluating on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations.

Compensation committee interlocks and insider participation

None of the current members of our compensation committee has ever been an executive officer or employee of ours. None of our executive officers currently serves, or has served during the last completed fiscal year, on the compensation committee or board of directors of any other entity that has one or more executive officers serving as a member of our board of directors or compensation committee.

Code of business conduct and ethics

Prior to the completion of this offering, we will adopt a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to directors, executive officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. The Code of Conduct will be available on the Investor Relations portion of our website at www.solidbio.com. The nominating and corporate governance committee of our board of directors will be responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. In addition, we intend to post on our website all disclosures that are required by law or the listing standards of NASDAQ concerning any amendments to, or waivers of, any provision of the Code of Conduct.

COMPENSATION OF OUR EXECUTIVE OFFICERS AND DIRECTORS

Named Executive Officers

Our named executive officers, or the Named Executive Officers, for the year ended December 31, 2017, are:

- Ilan Ganot, our Chief Executive Officer;
- Dr. Jorge A. Quiroz, our Chief Medical Officer; and
- Jennifer Ziolkowski, our Chief Financial Officer.

Compensation of our Named Executive Officers

Summary Compensation Table for Fiscal Year 2017

The following table contains information about the compensation paid to or earned by each of our Named Executive Officers during the most recently completed fiscal year.

Name and Principal Position	Year	Salary ($) (1)	Bonus ($) (2)	Stock Awards ($) (3)	All Other Compensation ($)	Total ($)
Ilan Ganot, Chief Executive Officer	2017	400,000	200,000	—	—	600,000
Jorge A. Quiroz, M.D., Chief Medical Officer	2017	360,500	144,200	428,344	4,420(4)	937,464
Jennifer Ziolkowski, Chief Financial Officer	2017	176,060(5)	124,700(6)	723,000	420(7)	1,024,180

(1) For 2018, base salary amounts for our Named Executive Officers were increased as follows: Mr. Ganot: $450,000; Dr. Quiroz: $375,000; and Ms. Ziolkowski: $300,000.
(2) Represents annual discretionary bonuses paid to the Named Executive Officers in respect of performance during the fiscal year ended December 31, 2017.
(3) The amount in this column represents the aggregate grant date fair value of the award as computed in accordance with Financial Accounting Standard Board Accounting Standards Codification Topic 718. The assumptions used in calculating the grant date fair value of the award reported in this column are set forth in Note 12 to our audited consolidated financial statements appearing elsewhere in this prospectus.
(4) Represents compensation for mobile phone subsidies, gym subsidies and a taxable gift provided by the Company, including an additional payment in respect of income taxes imposed upon Dr. Quiroz in connection with the taxable gift.
(5) Ms. Ziolkowski commenced employment with us in May 2017. This amount represents the portion of the year during which she was employed with us.
(6) Includes a $50,000 signing bonus paid to Ms. Ziolkowski in connection with the commencement of her employment with us.
(7) Represents compensation for mobile phone subsidies provided by the Company.

Employment Agreement with Mr. Ganot

On December 27, 2013, we entered into an employment agreement with Mr. Ganot. Mr. Ganot's employment agreement provided for an initial annual base salary of $300,000 as well as an entitlement to an annual incentive bonus in an amount determined by our board of managers. Mr. Ganot's employment with us is at will, although the agreement requires that either we or Mr. Ganot provide the other party at least six months' prior notice of intention to terminate Mr. Ganot's employment. However, we may terminate Mr. Ganot's

employment immediately for "cause" as defined in the employment agreement. Other than the foregoing notice period, Mr. Ganot's employment agreement does not provide for any severance payments or benefits upon a termination of his employment with us. Mr. Ganot is subject to certain restrictive covenants during the term of his employment and for the one-year period following termination, including employee and consultant non-solicitation and non-hire restrictions and non-competition provisions.

Offer Letter with Dr. Quiroz

On November 17, 2015, we entered into an offer letter with Dr. Quiroz. Dr. Quiroz's offer letter provided for an initial annual base salary of $350,000 as well as an entitlement to an annual incentive bonus of up to 40% of his base salary based upon achievement of individual and company-wide goals established by our board of managers in its sole discretion.

Under the offer letter, Dr. Quiroz received a signing bonus of $100,000, 50% of which he is required to repay if he resigns his employment other than for "good reason" (as defined in his offer letter) prior to the second anniversary of his employment commencement date. In addition, we agreed to assume certain obligations of Dr. Quiroz's prior employer with respect to Dr. Quiroz's graduate business school education, a leased apartment and a leased vehicle, up to a maximum of $250,000 in the aggregate. If Dr. Quiroz resigns his employment other than for good reason prior to the second anniversary of his employment commencement date, he will be required to repay 50% of the assumed obligations. We also agreed to reimburse Dr. Quiroz up to $120,000 in relocation expenses, plus an additional amount equal to the income taxes imposed on Dr. Quiroz in connection with such reimbursement.

In the event Dr. Quiroz's employment is terminated without "cause" (as defined in his offer letter) or Dr. Quiroz resigns for "good reason" (as defined in his offer letter), then, subject to his execution and non-revocation of a release of claims, he will receive continued payment of his base salary until the earlier of (i) six months following termination, and (ii) the date he obtains full-time employment. If his employment is terminated within 12 months following a change of control, Dr. Quiroz will receive an additional payment equal to 20% of his then current base salary. Dr. Quiroz is subject to certain restrictive covenants during the term of his employment and for the one-year period following termination, including employee and consultant non-solicitation and non-hire restrictions, customer non-solicitation and non-competition provisions.

Offer Letter with Ms. Ziolkowski

On April 17, 2017, we entered into an offer letter with Jennifer Ziolkowski, our Chief Financial Officer. Ms. Ziolkowski's agreement provided for an initial annual base salary of $280,000 and an annual incentive bonus of up to 40% of her base salary based upon achievement of individual and company-wide goals established by our board. Ms. Ziolkowski's agreement also provided for a one-time signing bonus of $50,000.

Ms. Ziolkowski's agreement provided for a grant of 150,000 Series D common units of Solid Biosciences, LLC. The units subject to these awards vest 25% on the first anniversary of Ms. Ziolkowski's start date and then in semi-annual installments over the 36-month period thereafter. In the event that we are acquired by a third party and Ms. Ziolkowski's employment is terminated by us without "Cause" (as defined in her employment agreement) within 12 months of the event, (A) Ms. Ziolkowski is entitled to receive continued payment of her base salary until the earlier of (i) three months following termination and (ii) the date she obtains full-time employment, and (B) all of Ms. Ziolkowski's outstanding unvested unit awards will become fully vested.

Ms. Ziolkowski is subject to certain restrictive covenants during the term of her employment and for the one-year period following termination, including employee and consultant non-solicitation and non-hire restrictions, customer non-solicitation and non-competition provisions.

Outstanding Equity Awards at Fiscal Year-End

The following table sets forth information regarding equity awards held by our Named Executive Officers as of December 31, 2017.

Name	Number of shares or units that have not vested (#)	Market value of shares or units that have not vested ($)(1)
Jorge A. Quiroz, M.D.	225,887(2)	789,680
	45,184(3)	212,817
	45,184(4)	215,528
Jennifer Ziolkowski	150,000(5)	484,500
	50,000(6)	238,500

(1) Calculated based on an independent third-party valuation.

(2) Represents Series D common units of Solid Biosciences, LLC granted on January 4, 2016. 25% of award vests on each of the first four anniversaries of grant date subject to continued employment through each vesting date. 100% of the units vest if Dr. Quiroz's employment is terminated without cause during the 12-month period after a change in control.

(3) Represents Series D common units of Solid Biosciences, LLC, with 11,296 vesting on September 12, 2018, and an additional 11,296 units vesting on each anniversary thereafter, subject to continued service through the vesting date and in no event shall more than 45,184 units become vested units. 100% of the units vest if Dr. Quiroz's employment is terminated without cause during the 12-month period after a change in control.

(4) Represents Series D common units of Solid Biosciences, LLC, with 11,296 vesting on the one-year anniversary of the date upon which the first dosing of a patient occurs in a clinical trial conducted by us, and an additional 5,648 units on each semi-annual anniversary thereafter, subject to continued employment through the vesting date and in no event shall more than 45,184 units become vested units. 100% of the units vest if Dr. Quiroz's employment is terminated without cause during the 12-month period after a change in control, provided that the first dosing condition is satisfied. If the dosing date has not occurred by December 7, 2027, all of the units become vested, subject to his continued employment through such date.

(5) Represents Series D common units of Solid Biosciences, LLC, with 37,500 units vesting on May 15, 2018, and an additional 18,250 units vesting on each semi-annual anniversary thereafter. 100% of the units vest if Ms. Ziolkowski's employment is terminated without cause during the 12-month period after a change in control.

(6) Represents Series D common units of Solid Biosciences, LLC, with 12,500 units vesting on the one-year anniversary of the date upon which we successfully complete an equity financing of $50 million or more at a price per unit not less than the price at which units were sold in the prior equity financing, and an additional 6,250 units on each semi-annual anniversary thereafter, subject to continued employment through the vesting date and in no event shall more than 50,000 units become vested units. 100% of the units vest if Ms. Ziolkowski employment is terminated without cause during the 12-month period after a change in control, provided that the equity financing condition is satisfied. If the equity financing condition has not occurred by December 7, 2027, all of the units become vested, subject to her continued employment through such date.

Equity Incentive Plans

Solid Biosciences, LLC Amended and Restated Equity Incentive Plan

We maintain the Solid Biosciences, LLC Amended and Restated Equity Incentive Plan, or the Existing Plan, under which we may grant Series D Common Units of the Company to our employees, consultants and other service providers. We will cease granting awards under the Existing Plan upon the implementation of the 2018 Plan, described below.

Our board of managers administers the Existing Plan. The board of managers is authorized to grant awards to eligible employees, consultants and other service providers. We intend to freeze the Existing Plan in connection with this offering. Following the date the Existing Plan is frozen, no further awards will be granted under the Existing Plan, but awards granted prior to the freeze date will continue in accordance with their terms and the terms of the Existing Plan.

The aggregate number of Series D Common Units that may be issued under the Existing Plan may not exceed 2,971,949. All of our current employees, consultants and other service providers are eligible to be granted awards under the Existing Plan. Eligibility for awards under the Existing Plan is determined by the board of managers in its discretion.

The board of managers may terminate or amend the Existing Plan at any time, subject to such approvals of the holders of the Company's units as may be required pursuant to the terms of the LLC Agreement.

2018 Omnibus Incentive Plan

In anticipation of this offering, our board of managers has adopted the Solid Biosciences Inc. 2018 Omnibus Incentive Plan, or 2018 Plan, contingent upon the consummation of this offering. Our unitholders have approved the 2018 Plan contingent upon the consummation of this offering. We believe that a new omnibus incentive plan is appropriate in connection with an initial public offering of our common stock not only to continue to enable us to grant awards to management to reward and incentivize their performance and retention, but also to have a long-term equity plan that is appropriate for us as a public company.

The material terms of the 2018 Plan are summarized below. The following summary is qualified in its entirety by reference to the complete text of the 2018 Plan, a copy of which will be filed as an exhibit to the registration statement of which this prospectus forms a part.

Administration of the plan

Our board of managers has appointed the compensation committee of our board of directors as the committee under the 2018 Plan with the authority to administer the 2018 Plan. We refer to our board of directors or compensation committee, as applicable, as the Administrator. The Administrator is authorized to grant awards to eligible employees, consultants and non-employee directors.

Number of authorized shares and award limits

The aggregate number of our shares of common stock that may be issued or used for reference purposes under the 2018 Plan may not exceed 5,001,000 shares (subject to adjustment as described below). Our shares of common stock that are subject to awards will be counted against the overall limit as one share for every share granted or covered by an award. If any award is cancelled, expires or terminates unexercised for any reason, the shares covered by such award will again be available for the grant of awards under the 2018 Plan, except that any shares that are not issued as the result of a net exercise or settlement or that are used to pay any exercise price or tax withholding obligation will not be available for the grant of awards. Shares of common stock that we repurchase on the open market with the proceeds of an option exercise price also will not be available for the grant of awards. Awards that may be settled solely in cash will not be deemed to use any shares.

The maximum number of our shares of common stock that may be granted pursuant to awards under the 2018 Plan during any fiscal year to any non-employee director is 1,000,200 shares. The foregoing individual participant limit is cumulative; that is, to the extent that shares of common stock that may be granted to an individual in a fiscal year are not granted, the number of shares of common stock that may be granted to such individual is increased in the subsequent fiscal years during the term of the 2018 Plan until used.

The Administrator will, in accordance with the terms of the 2018 Plan, make appropriate adjustments to the above aggregate and individual limits (other than cash limitations), to the number and/or kind of shares or other property (including cash) underlying awards and to the purchase price of shares underlying awards, in each case, to reflect any change in our capital structure or business by reason of any stock split, reverse stock split, stock dividend, combination or reclassification of shares, any recapitalization, merger, consolidation, spin off, split off, reorganization or any partial or complete liquidation, any sale or transfer of all or part of our assets or business, or any other corporate transaction or event that would be considered an "equity restructuring" within the meaning of FASB ASC Topic 718. In addition, the Administrator may take similar action with respect to other extraordinary events.

Eligibility and participation

All of our current and prospective employees and consultants, as well as our non-employee directors, are eligible to be granted non-qualified stock options, restricted stock, performance-based cash awards and other stock-based awards under the 2018 Plan. Only our and our subsidiaries' employees are eligible to be granted incentive stock options, or ISOs, under the 2018 Plan. Eligibility for awards under the 2018 Plan is determined by the Administrator in its discretion. In addition, each member of our board of directors who is not an employee of the company or any of our affiliates is expected to be eligible to receive awards under the 2018 Plan.

Types of awards

Stock options. The 2018 Plan authorizes the Administrator to grant ISOs to eligible employees and non-qualified stock options to purchase shares to employees, consultants, prospective employees, prospective consultants and non-employee directors. The Administrator will determine the number of shares of common stock subject to each option, the term of each option, the exercise price (which may not be less than the fair market value of the shares of common stock at the time of grant, or 110% of fair market value in the case of ISOs granted to 10% stockholders), the vesting schedule and the other terms and conditions of each option. Options will be exercisable at such times and subject to such terms as are determined by the Administrator at the time of grant. The maximum term of options under the 2018 Plan is ten years (or five years in the case of ISOs granted to 10% stockholders). Upon the exercise of an option, the participant must make payment of the full exercise price, either in cash or by check, bank draft or money order; solely to the extent permitted by law and authorized by the Administrator, through the delivery of irrevocable instructions to a broker, reasonably acceptable to us, to promptly deliver to us an amount equal to the aggregate exercise price; or on such other terms and conditions as may be acceptable to the Administrator (including, without limitation, the relinquishment of options or by payment in full or in part in the form of shares of common stock).

Restricted stock. The 2018 Plan authorizes the Administrator to grant restricted stock. Recipients of restricted stock enter into an agreement with us subjecting the restricted stock to transfer and other restrictions and providing the criteria or dates on which such awards vest and such restrictions lapse. The restrictions on restricted stock may lapse and the awards may vest over time, based on performance criteria or other factors (including, without limitation, performance goals that are intended to comply with the performance-based compensation exception under Section 162(m), as discussed below), as determined by the Administrator at the time of grant. Except as otherwise determined by the Administrator, a holder of restricted stock has all of the attendant rights of a stockholder including the right to receive dividends, if any, subject to and conditioned upon vesting and restrictions lapsing on the underlying restricted stock, the right to vote shares and, subject to and conditioned upon the vesting and restrictions lapsing for the underlying shares, the right to tender such shares. However, the Administrator may in its discretion provide at the time of grant that the right to receive dividends on restricted stock will not be subject to the vesting or lapsing of the restrictions on the restricted stock.

Other stock-based awards. The 2018 Plan authorizes the Administrator to grant awards of shares of common stock and other awards that are valued in whole or in part by reference to, or are payable in or otherwise based on, shares of common stock, including, but not limited to, shares of common stock awarded purely as a

bonus and not subject to any restrictions or conditions; shares of common stock in payment of the amounts due under an incentive or performance plan sponsored or maintained by us or an affiliate; stock appreciation rights; stock equivalent units; restricted stock units; performance awards entitling participants to receive a number of shares of common stock (or cash in an equivalent value) or a fixed dollar amount, payable in cash, stock or a combination of both, with respect to a designated performance period; or awards valued by reference to book value of our shares of common stock. In general, other stock-based awards that are denominated in shares of common stock will include the right to receive dividends, if any, subject to and conditioned upon vesting and restrictions lapsing on the underlying award, but the Administrator may in its discretion provide at the time of grant that the right to receive dividends on a stock-denominated award will not be subject to the vesting or lapsing of the restrictions on the performance award.

Performance-based cash awards

The 2018 Plan authorizes the Administrator to grant cash awards that are payable or otherwise based on the attainment of pre-established performance goals during a performance period. As noted above, following the Reliance Period, performance-based cash awards granted under the 2018 Plan that are intended to satisfy the performance-based compensation exception under Section 162(m) will vest based on attainment of specified performance goals established by the Administrator. These performance goals will be based on the attainment of a certain target level of, or a specified increase in (or decrease where noted), criteria selected by the Administrator.

Such performance goals may be based upon the attainment of specified levels of company, affiliate, subsidiary, division, other operational unit, business segment or administrative department performance relative to the performance of other companies. The Administrator may designate additional business criteria on which the performance goals may be based or adjust, modify or amend those criteria, to the extent permitted by Section 162(m). Unless the Administrator determines otherwise, to the extent permitted by Section 162(m), the Administrator will disregard and exclude the impact of special, unusual or non-recurring items, events, occurrences or circumstances; discontinued operations or the disposal of a business; the operations of any business that we acquire during the fiscal year or other applicable performance period; or a change in accounting standards required by generally accepted accounting principles or changes in applicable law or regulations.

Effect of certain transactions; Change in control

In the event of a change in control, as defined in the 2018 Plan, except as otherwise provided by the Administrator, unvested awards will not vest. Instead, the Administrator may, in its sole discretion provide that outstanding awards will be: assumed and continued; purchased based on the price per share paid in the change in control transaction (less, in the case of options and stock appreciation rights, or SARs, the exercise price), as adjusted by the Administrator for any contingent purchase price, escrow obligations, indemnification obligations or other adjustments to the purchase price; and/or in the case of stock options or other stock-based appreciation awards where the change in control price is less than the applicable exercise price, cancelled. However, the Administrator may in its sole discretion provide for the acceleration of vesting and lapse of restrictions of an award at any time including in connection with a change in control.

Non-transferability of awards

Except as the Administrator may permit, at the time of grant or thereafter, awards granted under the 2018 Plan are generally not transferable by a participant other than by will or the laws of descent and distribution. Shares of common stock acquired by a permissible transferee will continue to be subject to the terms of the 2018 Plan and the applicable award agreement.

Term

Awards under the 2018 Plan may not be made after December 18, 2027, but awards granted prior to such date may extend beyond that date. We may seek stockholder reapproval of the performance goals in the 2018

Plan. If such stockholder approval is obtained, on or after the first stockholders' meeting in the fifth year following the year of the last stockholder approval of the performance goals in the 2018 Plan, awards under the 2018 Plan may be based on such performance goals in order to qualify for the "performance-based compensation" exception under Section 162(m).

Amendment and termination

Subject to the rules referred to in the balance of this paragraph, our board of directors or the Administrator (to the extent permitted by law) may at any time amend, in whole or in part, any or all of the provisions of the 2018 Plan, or suspend or terminate it entirely, retroactively or otherwise. Except as required to comply with applicable law, no such amendment, suspension or termination may reduce the rights of a participant with respect to awards previously granted without the consent of such participant. In addition, without the approval of stockholders, no amendment may be made that would: increase the aggregate number of shares of common stock that may be issued under the 2018 Plan; increase the maximum individual participant share limitations for a fiscal year or year of a performance period; change the classification of individuals eligible to receive awards under the 2018 Plan; extend the maximum term of any option; reduce the exercise price of any option or SAR or cancel any outstanding "in-the-money" option or SAR in exchange for cash; substitute any option or SAR in exchange for an option or SAR (or similar other award) with a lower exercise price; alter the performance goals; or require stockholder approval in order for the 2018 Plan to continue to comply with Section 162(m) or Section 422 of the Internal Revenue Code of 1986, as amended, or the Code.

Registration of shares

Following consummation of this offering, we intend to file a registration statement on Form S-8 under the Securities Act of 1933, as amended, or the Securities Act, to register the full number of shares of common stock that will be available for issuance under the 2018 Plan, as described in the section titled "—2018 Plan—Number of Authorized Shares and Award Limits" above.

Federal income tax implications of the incentive plans

The federal income tax consequences arising with respect to awards granted under the Existing Plan and 2018 Plan will depend on the type of award. From the recipients' standpoint, as a general rule, ordinary income will be recognized at the time of payment of cash, or delivery of actual shares. Future appreciation on shares held beyond the ordinary income recognition event will be taxable at capital gains rates when the shares are sold. We, as a general rule, will be entitled to a tax deduction that corresponds in time and amount to the ordinary income recognized by the recipient, and we will not be entitled to any tax deduction in respect of capital gain income recognized by the recipient. Exceptions to these general rules may arise under the following circumstances: (i) if shares, when delivered, are subject to a substantial risk of forfeiture by reason of failure to satisfy any employment or performance-related condition, ordinary income taxation and our tax deduction will be delayed until the risk of forfeiture lapses (unless the recipient makes a special election to ignore the risk of forfeiture); (ii) if an employee is granted an ISO, no ordinary income will be recognized, and we will not be entitled to any tax deduction, if shares acquired upon exercise of the ISO are held longer than the later of one year from the date of exercise and two years from the date of grant; (iii) for awards granted after the reliance period, we may not be entitled to a tax deduction for compensation attributable to awards granted to one of our Named Executive Officers (other than our Chief Financial Officer), if and to the extent such compensation does not qualify as "performance-based" compensation under Section 162(m), and such compensation, along with any other non-performance-based compensation paid in the same calendar year, exceeds $1 million; and (iv) an award may be taxable at 20% above ordinary income tax rates at the time it becomes vested, even if that is prior to the delivery of the cash or stock in settlement of the award, if the award constitutes "deferred compensation" under Section 409A of the Code, and the requirements of Section 409A of the Code are not satisfied. The foregoing provides only a general description of the application of federal income tax laws to certain awards under the Incentive Plans, and is not intended as tax guidance to participants in the Incentive Plans, as the tax consequences

may vary with the types of awards made, the identity of the recipients and the method of payment or settlement. This summary does not address the effects of other federal taxes (including possible "golden parachute" excise taxes) or taxes imposed under state, local, or foreign tax laws.

Non-employee director compensation

We do not currently have a formal policy with respect to compensation payable to our non-employee managers for service as managers. During 2017, except for the Chairman of our Board, our non-employee managers did not receive any cash compensation for their services as managers or as board committee members. In 2017, Dr. Zarur received aggregate cash compensation of $352,333 for his services as Chairman. None of our non-employee managers received any equity award grants in 2017.

The table below shows the compensation paid to our non-employee managers during 2017.

Name	Fees Earned or Paid in Cash ($)	Equity Awards ($) (1)	All Other Compensation ($)	Total ($)
Andrey Zarur, Ph.D.	200,000(2)	—	$ 152,333(2)	352,333(2)
Robert Huffines	—	—	—	—
Lynne Sullivan	—	—	—	—
Matthew Arnold	—	—	—	—
Adam Stone	—	—	—	—
Rajeev Shah	—	—	—	—
Adam Koppel, M.D., Ph.D.	—	—	—	—

(1) As of December 31, 2017, none of our non-employee managers held any equity awards or unvested units.

(2) Represents advisory fees paid to Dr. Zarur in exchange for services as Chairman of our board of managers, including an approximately $150,000 discretionary bonus paid to Dr. Zarur in respect of performance during the fiscal year that ended December 31, 2017 and an additional $2,333 representing a taxable gift provided by the Company.

Following the consummation of this offering, we intend to implement a director compensation program pursuant to which our non-employee directors will receive the following compensation for their service on our board of directors:

- An annual retainer of $35,000;
- An additional annual retainer of $15,000 for serving as chair or $7,500 for serving as a member of the Audit Committee;
- An additional annual retainer of $10,000 for serving as chair or $5,000 for serving as a member of the Compensation Committee;
- An additional annual retainer of $8,000 for serving as chair or $4,000 for serving as a member of the Nominating and Corporate Governance Committee; and
- An annual grant of restricted stock made under the 2018 Plan having a fair market value of $50,000, all of which shall vest on the earlier to occur of the one-year anniversary of the grant date and immediately prior to the first annual meeting of our stockholders occurring after the grant date subject, in all cases, to each such director's continued service as a member of the Board from the grant date to the applicable vesting date.

CERTAIN RELATIONSHIPS AND RELATED-PERSON TRANSACTIONS

In addition to the executive officer and director compensation arrangements discussed above under "Compensation of our executive officers and directors," below we describe transactions since January 1, 2014 to which we have been or will be a participant, in which the amount involved in the transaction exceeds or will exceed $120,000 and in which any of our directors, executive officers or beneficial holders of more than 5% of any class of our capital stock, or 5% Security Holders, or any immediate family member of, or person sharing the household with, any of these individuals, had or will have a direct or indirect material interest.

Equity financings

Solid Biosciences, LLC

On March 29, 2017, we entered into a unit purchase agreement, or the Senior Preferred Unit Purchase Agreement, which provided for the sale of 2,500,000 of our Series 1 Senior Preferred Units to certain investors at a price of $10.00 per unit for an aggregate purchase price of $25.0 million. 625,000 of such units were sold to affiliates of RA Capital Management, LLC, or RA Capital. Mr. Shah, a member of our board of directors, is a Portfolio Manager and Managing Director at RA Capital. 249,999 of Series 1 Senior Preferred Units were sold to affiliates of Perceptive Advisors. Mr. Stone, a member of our board of directors, is the Chief Investment Officer of Perceptive Advisors, and Perceptive Advisors is a 5% Security Holder. 166,667 of such units were sold to an affiliate of Biogen, Inc., or Biogen. Ms. Sullivan, a member of our board of directors, is the Senior Vice President of Finance of Biogen, and Biogen is a 5% Security Holder.

The Senior Preferred Unit Purchase Agreement, as amended on September 1, 2017, additionally provided that the holders of the Series 1 Senior Preferred Units were required to purchase $25.0 million of Series 2 Senior Preferred Units, in the event we achieve certain preclinical milestones. In addition, at their option, the holders had the ability to purchase the Series 2 Senior Preferred Units at any time prior to December 1, 2017. On October 26, 2017, the Senior Preferred Unit Purchase Agreement was further amended to provide for the sale of 4,886,000 Series 2 Senior Preferred Units at a purchase price of $11.257 per unit for an aggregate purchase price of $55.0 million. As part of this Series 2 Preferred Financing, which closed on October 26, 2017, 1,110,470 of such units were sold to affiliates of RA Capital, 444,180 of such units were sold to an affiliate of Perceptive Advisors, 296,120 of such units were sold to an affiliate of Biogen, 1,110,470 of such units were sold to an affiliate of Bain Capital Life Sciences and 222,080 of such units were sold to each of Mr. Arnold and Mr. Hayeem, members of our board of managers. Dr. Koppel, a member of our board of directors, is a Managing Director of Bain Capital Life Sciences, and Bain Capital Life Sciences is a 5% Security Holder.

J.P. Morgan Securities, LLC, acted as placement agent in connection with our offering of securities under the Senior Preferred Unit Purchase Agreement and received customary placement agent fees for its services. Mr. Huffines is an employee of J.P. Morgan Securities, LLC. JPMC Strategic Investments II Corporation, a 5% Security Holder, is an affiliate of J.P. Morgan Securities, LLC.

Solid GT, LLC

On November 2, 2015, Solid GT entered into a unit purchase agreement which provided for the sale of 134,920 of its Class D Voting Units to certain investors at a price of $315.00 per unit for an aggregate purchase price of approximately $42.5 million. 47,619 of such units were sold to Biogen, 47,619 of such units were sold to affiliates of Perceptive Advisors, and 6,349 of such units were sold to Matthew Arnold, a member of our board of directors. On March 29, 2017, pursuant to a merger agreement between the Company and Solid GT, or the Merger Agreement, the operations of Solid GT were merged into the Company and all outstanding units of Solid GT, including those held by related persons, were converted into units of Solid Biosciences, LLC. See "—Merger and recapitalization."

Merger and recapitalization

We historically owned 100% of the voting units of our wholly owned subsidiary, Solid GT. Solid GT was organized in Delaware in August 2014. In November 2015, Solid GT issued voting units to new investors (as

discussed above under "—Equity Financings—Solid GT, LLC"), which decreased our voting ownership in Solid GT to 77%. On March 29, 2017, pursuant to the Merger Agreement, we merged the operations of Solid GT into the company and Solid GT ceased to exist as a separate legal entity. In connection with the Merger, units of the company and units of Solid GT were converted into new series of units of the company. Units of the company and Solid GT that were held by our executive officers, directors and 5% Security Holders were converted on the same basis as all other holders of such units as forth in the Merger Agreement and the LLC Agreement.

Limited liability company agreement of Solid Biosciences, LLC

We are party to the LLC Agreement with our current members. The LLC Agreement will terminate upon the Corporate Conversion. See "Management—Composition of the board of directors." Under the terms of the LLC Agreement, Series A Common Unit holders were entitled to designate two individuals to serve on our board of managers. Pursuant to this provision, the two board appointees were Mssrs. Arnold and Huffines. Mr. Huffines is an employee of J.P. Morgan Securities LLC, a participating underwriter in this offering. An affiliate of J.P. Morgan Securities LLC owns in excess of 10% of our issued and outstanding common stock. See "Underwriting—Conflicts of Interest" for a description of services that the underwriters have provided to us.

Amended and restated registration rights agreement

We are party to an Amended and Restated Registration Rights Agreement, or the Registration Rights Agreement, dated March 29, 2017, with certain holders of our units, which includes our 5% Security Holders and entities affiliated with certain of our directors. The Registration Rights Agreement provides these holders with the right to request, following this offering, that their shares of common stock be registered for resale in certain circumstances. See "Description of capital stock—Registration rights."

Corporate conversion

We currently operate as a Delaware limited liability company under the name Solid Biosciences, LLC. Prior to the effectiveness of the registration statement of which this prospectus forms a part, Solid Biosciences, LLC will convert into a Delaware corporation pursuant to a statutory conversion and change its name to Solid Biosciences Inc. In addition, entities affiliated with certain of our unitholders will be merged with and into us. As required by the LLC Agreement, the Corporate Conversion will be approved by the requisite number of outstanding units of Solid Biosciences, LLC.

In connection with the Corporate Conversion, Solid Biosciences, LLC unitholders will receive 26,498,559 shares of common stock (including 1,132,425 shares of restricted stock) for all units held immediately prior to the Corporate Conversion. The existing units held by our executive officers, directors and 5% Security Holders will be converted on the same basis as all other holders of such units.

Equity grants to executive officers and directors

Solid Biosciences, LLC

On May 7, 2014, we granted 114,667 Series A Common Units to Dr. Schneider. On September 1, 2015, we granted 114,000 Series A Common Units to Dr. Morris. On January 29, 2016, we granted 171,000 Series A Common Units to Mr. Amorrortu. On March 29, 2017, we granted 50,000 Series D Common Units to Dr. Morris. On May 31, 2017, we granted 150,000 Series D Common Units to Ms. Ziolkowski. On September 12, 2017, we granted 45,184 Series D Common Units to Dr. Quiroz and 22,707 Series D Common Units to Mr. Amorrortu. On December 7, 2017, we granted 22,706 Series D Common Units to Mr. Amorrortu, 45,184 Series D Common Units to Dr. Quiroz and 50,000 Series D Common Units to Ms. Ziolkowski. No payment was made to Solid in connection with the above grants.

Solid GT, LLC

On December 15, 2015, Solid GT granted 1,388 Class C Non-Voting Units to Mr. Amorrortu, 6,904 Class C Non-Voting Units to Dr. Quiroz, and 2,778 Class C Non-Voting Units to Dr. Schneider. No payment was made to Solid GT in connection with the above grants. On March 29, 2017, all Class C Non-Voting Units were converted to units of Solid Biosciences, LLC in connection with the Merger and Recapitalization described above.

Other arrangements

Since November 2016, we have employed Annie Ganot, the wife of Ilan Ganot, as Director, Patient Advocacy. Mr. Ganot is our CEO and a member of our board of directors. Ms. Ganot receives an annual salary of less than $200,000 and received a signing bonus in connection with the start of her employment.

Indemnification agreements

We will enter into agreements to indemnify our directors and executive officers. These agreements will, among other things, require us to indemnify these individuals for certain expenses (including attorneys' fees), judgments, fines and settlement amounts reasonably incurred by such persons in any action or proceeding, including any action by or in our right, on account of any services undertaken by any such person on behalf of our company or that person's status as a member of our board of directors to the maximum extent allowed under Delaware law.

Indications of Interest

Certain of our existing stockholders, including certain of our 5% Security Holders, have agreed to purchase an aggregate of approximately 3,955,000 shares of our common stock in this offering at the initial public offering price. The underwriters will receive the same underwriting discounts and commissions on the shares purchased by these stockholders as they will on the other shares sold to the public in this offering.

Policy for approval of related-person transactions

Prior to this offering, we have not had a formal policy regarding approval of transactions with related persons. In connection with this offering, our board of managers has adopted a related-person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification for the review of any transaction, arrangement or relationship in which we are a participant, the amount involved exceeds $120,000 and one of our executive officers, directors, director nominees or 5% stockholders (or their immediate family members), each of whom we refer to as a "related person," has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a "related-person transaction," the related person must report the proposed related-person transaction to our general counsel. The policy calls for the proposed related-person transaction to be reviewed by and if deemed appropriate approved by, the audit committee of our board of directors. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the audit committee will review and, in its discretion, may ratify the related-person transaction. The policy also permits the chair of the audit committee to review, and if deemed appropriate approve, proposed related-person transactions that arise between audit committee meetings, subject to ratification by the audit committee at its next meeting. Any related-person transactions that are ongoing in nature will be reviewed annually.

A related-person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the audit committee after full disclosure of the related person's interest in the transaction. As appropriate for the circumstances, the committee will review and consider:

- the related person's interest in the related-person transaction;
- the approximate dollar amount involved in the related-person transaction;
- the approximate dollar amount of the related person's interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;

- the purpose of, and the potential benefits to us of, the related-person transaction; and
- any other information regarding the related-person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The audit committee may approve or ratify the transaction only if the audit committee determines that, under all of the circumstances, the transaction is not inconsistent with our best interests. The audit committee may impose any conditions on the related-person transaction that it deems appropriate.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by the compensation committee of our board of directors in the manner specified in its charter.

PRINCIPAL STOCKHOLDERS

The following table sets forth information regarding the beneficial ownership of our common stock as of January 1, 2018 by (i) each person whom we know to beneficially own more than 5% of our outstanding common stock (a "5% stockholder"), (ii) each director, (iii) each executive officer and (iv) all directors and executive officers as a group. Unless otherwise indicated, the address of each executive officer and director is c/o Solid Biosciences, 161 First Street, Third Floor, Cambridge, MA 02412.

The number of shares of common stock "beneficially owned" by each stockholder is determined under rules issued by the SEC regarding the beneficial ownership of securities. This information is not necessarily indicative of beneficial ownership for any other purpose. Under these rules, beneficial ownership of shares of our common stock includes (1) any shares as to which the person or entity has sole or shared voting power or investment power and (2) any shares as to which the person or entity has the right to acquire beneficial ownership within 60 days after January 1, 2018. Each holder's percentage ownership before this offering is based on 26,498,559 shares of common stock outstanding as of January 1, 2018, after giving effect to the Corporate Conversion. Each holder's percentage ownership after this offering is based on 34,311,059 shares of common stock to be outstanding immediately after the consummation of this offering. The percentages assume no exercise by the underwriters of their option to purchase additional shares.

Unless otherwise indicated below, and subject to community property laws where applicable, to our knowledge, all persons named in the table have sole voting and investment power with respect to their shares of common stock.

	Before Offering		After Offering	
Name of Beneficial Owner	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned	Number of Shares Beneficially Owned	Percentage of Shares Beneficially Owned
5% Stockholders:				
JPMC Strategic Investments II Corporation (1)	3,031,601	11.4%	3,031,601	8.8%
Perceptive Life Sciences Master Fund LTD (2)	2,907,222	11.0%	3,907,222	11.4%
Biogen New Ventures Inc. (3)	1,772,486	6.7%	1,772,486	5.2%
BCLS SB Investco, LP (4)	1,689,444	6.4%	1,989,444	5.8%
Entities affiliated with RA Capital Management, LLC (5)	1,689,444	6.4%	2,689,444	7.8%
Executive Officers and Directors:				
Ilan Ganot (6)	1,443,040	5.4%	1,443,040	4.2%
Gilad Hayeem (7)	3,578,399	13.5%	3,578,399	10.4%
Alvaro Amorrortu	180,746	*	180,746	*
Carl Morris, Ph.D.	111,545	*	111,545	*
Joel Schneider, Ph.D.	146,646	*	146,646	*
Jorge A. Quiroz, M.D.	268,342	1.0%	268,342	*
Jennifer Ziolkowski	169,700	*	169,700	*
Andrey Zarur, Ph.D.	691,205	2.6%	691,205	2.0%
Matthew Arnold	3,396,293	12.8%	3,396,293	9.9%
Robert Huffines	—	*	—	*
Adam Koppel, M.D., Ph.D. (8)	1,689,444	6.4%	1,989,444	5.0%
Rajeev Shah (5)	1,689,444	6.4%	2,689,444	5.0%
Adam Stone (9)	—	*	—	*
Lynne Sullivan	—	*	—	*
All directors and executive officers as a group (14 persons)	**13,364,804**	**50.4%**	**14,664,804**	**42.7%**

* Less than one percent.

(1) Consists of shares held by JPMC Strategic Investments II Corporation, or JPMC Strategic Investments. The address of JPMC Strategic Investments is 270 Park Avenue, New York, NY 10017.

(2) Consists of shares held by Perceptive Life Sciences Master Fund LTD, or Perceptive, including 1,000,000 shares that Perceptive has agreed to purchase in this offering. Perceptive Advisors LLC is the advisor of Perceptive, and Joseph Edelman is the managing member of Perceptive Advisors LLC. Perceptive Advisors LLC and Mr. Edelman may be deemed to beneficially own the shares held by Perceptive. The address of Perceptive is 51 Astor Place, 10th Floor, New York, NY 10003.

(3) Consists of shares held by Biogen New Ventures Inc., or Biogen New Ventures. Biogen New Ventures is a wholly owned subsidiary of Biogen MA Inc., which is a wholly owned subsidiary of Biogen Inc. The address of Biogen New Ventures is 250 Binney Street, Cambridge, MA 02142.

(4) Consists of shares held by BCLS SB Investco, LP ("BCLS"), including 300,000 shares that BCLS has agreed to purchase in this offering. The governance, investment strategy and decision-making process with respect to investments held by BCLS is directed by Bain Capital Life Sciences Investors, LLC, whose managers are Jeffrey Schwartz and Adam Koppel. As a result, each of Bain Capital Life Sciences Investors, LLC, Mr. Schwartz and Dr. Koppel may be deemed to share voting and dispositive power over the shares held by BCLS. The address of BCLS is c/o Bain Capital Life Sciences, LP, 200 Clarendon Street, Boston, Massachusetts 02116.

(5) Consists of (a) 1,368,981 shares held by RA Capital Healthcare Fund, L.P. ("RA Capital Fund"), and (b) 320,463 shares held by Blackwell Partners LLC—Series A ("Blackwell") and (c) 1,000,000 shares that RA Capital Fund and Blackwell have agreed to purchase in this offering. RA Capital Management, LLC ("RA Capital") is the general partner of RA Capital Fund and the investment manager to Blackwell. Investment decisions with respect to the shares held by RA Capital Fund and Blackwell are made by a portfolio management team at RA Capital of which Rajeev Shah, a member of our board of directors, is a member. Mr. Shah disclaims beneficial ownership of all shares held by RA Capital Fund and Blackwell, except to the extent of his pecuniary interest therein. The address for each of RA Capital Fund, Blackwell, and RA Capital is c/o 20 Park Plaza, Suite 1200, Boston, MA 02116.

(6) Consists of (a) 1,091,495 shares held by Mr. Ganot as an individual, (b) 60,631 shares held by Mr. Ganot and Ms. Ganot as joint tenants with right of survivorship and (c) 290,914 shares held by Mr. Adam Ganot and Ms. Ganot, as trustees for the Ilan Ganot 2017 Irrevocable Trust. Excludes 1,593,755 shares held by The Popps Trust for which Mr. Ganot and Mr. Hayeem act as trustees, but as to which Mr. Ganot has no pecuniary interest.

(7) Consists of (a) 1,984,644 shares held by Mr. Hayeem as an individual and (b) 1,593,755 shares held by The Popps Trust, a trust established for the benefit of Mr. Hayeem's family for which Mr. Hayeem is the Investment Advisor. In connection with estate planning activities, Mr. Hayeem sold units of the Company equivalent to approximately 353,050 shares to a sub-trust of an employee-benefit trust established by a former employer of Mr. Hayeem. Such sub-trust has as its beneficiaries Mr. Hayeem and his family. Because Mr. Hayeem does not exercise investment or voting control of the shares held by such sub-trust, such shares do not appear in the table above.

(8) Consists of shares held by BCLS. Dr. Koppel is a manager of Bain Capital Life Sciences Investors, LLC and as a result, by virtue of the relationships described in footnote (4) above, may be deemed to share beneficial ownership of the shares held by BCLS. The address of Dr. Koppel is c/o Bain Capital Life Sciences, LP, 200 Clarendon Street, Boston, Massachusetts 02116.

(9) Mr. Stone is Chief Investment Officer of Perceptive Advisors LLC. Mr. Stone disclaims beneficial ownership of the shares held by Perceptive. The address of Mr. Stone is 51 Astor Place, 10th Floor, New York, NY 10003.

DESCRIPTION OF CAPITAL STOCK

The following description is intended as a summary of our certificate of incorporation (which we refer to as our "charter") and our bylaws, each of which will become effective prior to the effectiveness of the registration statement of which this prospectus forms a part and which will be filed as exhibits to the registration statement of which this prospectus forms a part, and to the applicable provisions of the Delaware General Corporation Law. The description of our common stock and preferred stock reflects the completion of the Corporate Conversion. Because the following is only a summary, it does not contain all of the information that may be important to you. For a complete description, you should refer to our charter and bylaws.

General

Our charter authorizes 300,000,000 shares of common stock, $0.001 par value per share, and 10,000,000 shares of preferred stock, $0.001 par value per share.

As of September 30, 2017, after giving effect to the Series 2 Preferred Financing and the Corporate Conversion, there were 26,338,522 shares of our common stock outstanding (including 1,776,377 shares of restricted common stock) and approximately 104 stockholders of record. No shares of our preferred stock are designated, issued or outstanding.

Common stock

Voting rights

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Our stockholders do not have cumulative voting rights in the election of directors. Accordingly, holders of a majority of the voting shares are able to elect all of the directors.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of our common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds.

We have not declared or paid any cash dividends on our capital stock since our inception. We intend to retain future earnings, if any, to finance the operation and expansion of our business and do not anticipate paying any cash dividends in the foreseeable future.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and preferences

Holders of our common stock have no preemptive, conversion, subscription or other rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of our preferred stock that we may designate in the future.

Fully paid and nonassessable

All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, fully paid and nonassessable.

Preferred stock

Our board of directors will have the authority, without further action by our stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series and to fix the rights, preferences, privileges and restrictions thereof. These rights, preferences and privileges could include dividend rights, conversion rights, voting rights, terms of redemption, liquidation preferences and sinking fund terms, any or all of which may be greater than the rights of common stock. The issuance of our preferred stock could adversely affect the voting power of holders of common stock and the likelihood that such holders will receive dividend payments and payments upon our liquidation. In addition, the issuance of preferred stock could have the effect of delaying, deferring or preventing a change in control of our company or other corporate action. Immediately after consummation of this offering, no shares of preferred stock will be outstanding, and we have no present plan to issue any shares of preferred stock.

Registration rights

We are party to the Registration Rights Agreement, dated March 29, 2017, with certain of our stockholders, or the Investors. The Registration Rights Agreement provides for demand and piggyback registration rights for the Investors. All expenses of registration (other than underwriting discounts and commissions) under the Registration Rights Agreement will be borne by us.

Demand registration rights

Beginning six months after the date of this prospectus, the Investors are entitled to demand registration rights. Under the terms of the Registration Rights Agreement, we will be required, upon the written request of Investors holding at least 20% of the securities eligible for registration then outstanding, to file a registration statement and use our best efforts to effect as soon as practicable the registration of such shares. We are required to effect only two demand registrations pursuant to the Registration Rights Agreement. However, if we become eligible to register the sale of securities on Form S-3 under the Securities Act, the Investors have the right to demand unlimited registrations under the Registration Rights Agreement (but not to exceed two registrations on Form S-3 in any calendar year) provided that the securities for sale on Form S-3 have an aggregate price to the public of $2.0 million.

Piggyback registration rights

If we register any of our equity securities either for our own account or for the account of other security holders, the Investors are entitled to piggyback registration rights and may include their shares in the registration. The underwriters may advise us to limit the number of shares included in any underwritten offering to the number of shares which we and the underwriters determine in our sole discretion will not jeopardize the success of the offering. If this occurs, the aggregate number of securities held by the Investors that may be included in the underwriting shall be allocated among all requesting Investors in proportion to the amount of securities sought to be sold by each Investor.

Fees; Indemnification

Under the Registration Rights Agreement, we will be responsible, subject to certain exceptions, for the expenses of any registration of securities pursuant to the agreement, other than underwriting discounts and commissions.

The Registration Rights Agreement contains customary cross-indemnification provisions, under which we are obligated to indemnify the Investors in the event of material misstatements or omissions in the registration statement or any violation of the Securities Act, Exchange Act, state securities law or any rule or regulation promulgated thereunder attributable to us, and they are obligated to indemnify us, severally and not jointly, for material misstatements, omissions or any violation of the Securities Act, Exchange Act, state securities law or any rule or regulation promulgated thereunder attributable to them.

Termination of registration rights

The demand registration rights and the piggyback registration rights granted under the Registration Rights Agreement will terminate, with respect to each Investor, as of the date when all registrable securities held by and issued to such Investor may be sold under Rule 144 under the Securities Act, provided such Investor owns less than 1% of the outstanding common stock of the Company.

Anti-takeover effects of provisions of our charter, our bylaws and Delaware law

Some provisions of Delaware law, our charter and our bylaws, contain provisions that could have the effect of delaying, deterring or preventing another party from acquiring or seeking to acquire control of us through the use of the following: acquisition of us by means of a tender offer; acquisition of us by means of a proxy contest or otherwise; or removal of our incumbent officers and directors. These provisions may delay, deter or prevent a change in control or other takeover of our company that our stockholders might consider to be in their best interests, including transactions that might result in a premium being paid over the market price of our common stock and also may limit the price that investors are willing to pay in the future for our common stock. These provisions may also have the effect of preventing changes in our management.

These provisions are intended to discourage certain types of coercive takeover practices and inadequate takeover bids and to encourage anyone seeking to acquire control of us to negotiate first with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure us outweigh the disadvantages of discouraging these proposals because negotiation of these proposals could result in an improvement of their terms.

Charter and bylaws provisions

Our charter and our bylaws, include a number of provisions that could deter hostile takeovers or delay or prevent changes in control of our company, including the following:

- *Board of Directors Vacancies:* Our charter and bylaws authorize only our board of directors to fill vacant directorships, including newly created seats. In addition, the number of directors constituting our board of directors may only be set by a resolution adopted by a majority vote of our entire board of directors. These provisions would prevent a stockholder from increasing the size of our board of directors and then gaining control of our board of directors by filling the resulting vacancies with its own nominees. This makes it more difficult to change the composition of our board of directors but promotes continuity of management.
- *Classified Board:* Our charter and bylaws provide that our board of directors will be classified into three classes of directors, each with staggered three-year terms. A third party may be discouraged from making a tender offer or otherwise attempting to obtain control of us as it is more difficult and time consuming for stockholders to replace a majority of the directors on a classified board of directors. See “Management—Composition of the board of directors.”
- *Stockholder Action; Special Meetings of Stockholders:* Our charter provides that our stockholders may not take action by written consent, but may only take action at annual or special meetings of our stockholders. As a result, a holder controlling a majority of our capital stock would not be able to amend our bylaws or remove directors without holding a meeting of our stockholders called in accordance with our bylaws. Further, our bylaws and charter will provide that special meetings of our stockholders may be called only by a majority of our board of directors, the Chairman of our board of directors or our Chief Executive Officer, thus prohibiting a stockholder from calling a special meeting. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.

- *Advance Notice Requirements for Stockholder Proposals and Director Nominations:* Our bylaws provide advance notice procedures for stockholders seeking to bring matters before our annual meeting of stockholders or to nominate candidates for election as directors at our annual meeting of stockholders. Our bylaws also specify certain requirements regarding the form and content of a stockholder's notice. These provisions might preclude our stockholders from bringing matters before our annual meeting of stockholders or from making nominations for directors at our annual meeting of stockholders if the proper procedures are not followed. We expect that these provisions might also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer's own slate of directors or otherwise attempting to obtain control of our company.
- *Supermajority Voting:* The Delaware General Corporation Law, or the DGCL, provides, generally, that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Our bylaws may be amended or repealed by a majority vote of our board of directors or the affirmative vote of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast in an annual election of directors. In addition, the affirmative vote of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast in an election of directors is required to amend or repeal or to adopt certain provisions of our charter.
- *No Cumulative Voting:* The DGCL provides that stockholders are not entitled to the right to cumulate votes in the election of directors unless a corporation's certificate of incorporation provides otherwise. Our charter does not provide for cumulative voting.
- *Removal of Directors Only for Cause:* Our charter provides that stockholders may remove directors only for cause and only by the affirmative vote of the holders of at least two-thirds of our outstanding common stock.
- *Exclusive Forum:* Our charter provides that the Court of Chancery of the State of Delaware will be the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the DGCL, our charter or our bylaws; any action to interpret, apply, enforce or determine the validity of our charter or our bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable.

Delaware law

We are subject to the provisions of Section 203 of the DGCL, regulating corporate takeovers. In general, DGCL Section 203 prohibits a publicly held Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years following the date on which the person became an interested stockholder unless:

- prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- upon consummation of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, (i) shares owned by persons who are directors and also officers and (ii) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or

- at or subsequent to the date of the transaction, the business combination is approved by the board of directors of the corporation and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least two-thirds of the outstanding voting stock that is not owned by the interested stockholder.

Generally, a business combination includes a merger, asset or stock sale, or other transaction or series of transactions together resulting in a financial benefit to the interested stockholder. An interested stockholder is a person who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own 15% or more of a corporation's outstanding voting stock. We expect the existence of this provision to have an anti-takeover effect with respect to transactions our board of directors does not approve in advance. We also anticipate that DGCL Section 203 may also discourage attempts that might result in a premium over the market price for the shares of common stock held by stockholders.

Limitations on liability, indemnification of officers and directors and insurance

Our charter and bylaws contain provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by Delaware law.

Listing

We have been approved to list our common stock on the NASDAQ Global Select Market, under the symbol "SLDB."

Transfer agent and registrar

The transfer agent and registrar for the shares of our common stock will be Computershare Trust Company, N.A. The transfer agent and registrar's address is 250 Royall Street, Canton, MA 02021.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of our common stock in the public market after this offering, or the perception that those sales may occur, could cause the prevailing market price for our common stock to fall or impair our ability to raise equity capital in the future. As described below, only a limited number of shares of our common stock will be available for sale in the public market for a period of several months after consummation of this offering due to contractual and legal restrictions on resale described below. Future sales of our common stock in the public market either before (to the extent permitted) or after restrictions lapse, or the perception that those sales may occur, could adversely affect the prevailing market price of our common stock at such time and our ability to raise equity capital at a time and price we deem appropriate. Although we have been approved to list our common stock on the NASDAQ Global Select Market under the symbol "SLDB," we cannot assure you that there will be an active public market for our common stock.

Sale of restricted shares

Based on the number of shares of our common stock outstanding as of September 30, 2017, after giving effect to the Series 2 Preferred Financing and the Corporate Conversion, upon the closing of this offering and assuming no exercise of the underwriters' option to purchase additional shares of common stock, we will have outstanding an aggregate of approximately 34,151,022 shares of common stock. Of these shares, all of the 7,812,500 shares of common stock to be sold in this offering, and any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable in the public market without restriction or further registration under the Securities Act, unless the shares are held by any of our "affiliates" as such term is defined in Rule 144 of the Securities Act. All remaining shares of common stock held by existing stockholders immediately prior to the consummation of this offering will be "restricted securities" as such term is defined in Rule 144. These restricted securities were issued and sold by us in private transactions and are eligible for public sale only if registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including the exemptions provided by Rule 144 or Rule 701, which rules are summarized below. As a result of the contractual 180-day lock-up period described below and the provisions of Rule 144 and 701 of the Securities Act, the restricted securities will be available for sale in the public markets as follows:

Date Available for Sale	Shares Eligible for Sale	Description
Date of Prospectus	7,812,500	Shares sold in the offering and shares saleable under Rule 144 that are not subject to a lock-up
180 Days after Date of Prospectus	26,338,522	Lock-up released; shares saleable under Rules 144 and 701

Rule 144

In general, under Rule 144, as currently in effect, once we have been subject to the public company reporting requirements of the Exchange Act, for at least 90 days, a person (or persons whose shares are required to be aggregated) who is not deemed to have been one of our "affiliates" for purposes of Rule 144 at any time during the three months preceding a sale, and who has beneficially owned restricted securities within the meaning of Rule 144 for at least six months, including the holding period of any prior owner other than one of our "affiliates," is entitled to sell those shares in the public market (subject to the lock-up agreement referred to below, if applicable) without complying with the manner of sale, volume limitations or notice provisions of Rule 144, but subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than "affiliates," then such person is entitled to sell such shares in the public market without complying with any of the requirements of Rule 144 (subject to the lock-up agreement referred to below, if applicable). In general, under Rule 144, as currently in effect, once we have been subject to the public company

reporting requirements of the Exchange Act for at least 90 days, our "affiliates," as defined in Rule 144, who have beneficially owned the shares proposed to be sold for at least six months, including the holding period of any prior owner other than one of our "affiliates," are entitled to sell in the public market, upon expiration of any applicable lock-up agreements and within any three-month period, a number of those shares of our common stock that does not exceed the greater of:

- 1% of the number of common shares then outstanding, which will equal approximately 341,510 shares of common stock immediately after this offering (calculated assuming no exercise of the underwriters' option to purchase additional shares and no exercise of outstanding options or warrants); or
- the average weekly trading volume of our common stock on the NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to such sale.

Such sales under Rule 144 by our "affiliates" or persons selling shares on behalf of our "affiliates" are also subject to certain manner of sale provisions, notice requirements and to the availability of current public information about us. Notwithstanding the availability of Rule 144, the holders of all of our restricted securities have entered into lock-up agreements as referenced above and their restricted securities will become eligible for sale (subject to the above limitations under Rule 144) upon the expiration of the restrictions set forth in those agreements.

Rule 701

In general, under Rule 701 as currently in effect, any of our employees, directors, officers, consultants or advisors who acquired common stock from us in connection with a written compensatory stock or option plan or other written agreement in compliance with Rule 701 under the Securities Act before the effective date of the registration statement of which this prospectus is a part (to the extent such common stock is not subject to a lock-up agreement) is entitled to rely on Rule 701 to resell such shares beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act in reliance on Rule 144, but without compliance with the holding period requirements contained in Rule 144. Accordingly, subject to any applicable lock-up agreements, beginning 90 days after we become subject to the public company reporting requirements of the Exchange Act, under Rule 701 persons who are not our "affiliates," as defined in Rule 144, may resell those shares without complying with the minimum holding period or public information requirements of Rule 144, and persons who are our "affiliates" may resell those shares without compliance with Rule 144's minimum holding period requirements.

Equity incentive plans

Our board of directors and stockholders previously adopted the Existing Plan. In connection with this offering, our board of directors and stockholders intend to adopt the 2018 Plan. For a description of our Existing Plan and 2018 Plan, see "Compensation of our executive officers and directors—Equity incentive plans."

In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act to register the total number of shares of our common stock that may be issued under our 2018 Plan. That registration statement will become effective upon filing, and 5,001,000 shares of our common stock covered by such registration statement are eligible for sale in the public market immediately after the effective date of such registration statement, subject to Rule 144 volume limitations applicable to affiliates, vesting restrictions and the lock-up agreements described below.

Registration rights

Beginning six months after the date of this prospectus, the holders of approximately 24.2 million shares of our common stock will, after the expiration of the lock-up period, be entitled to certain rights with respect to the registration of the offer and sale of those shares under the Securities Act. For a description of these registration rights, please see the section titled "Description of capital stock—Registration rights." If the offer and sale of these shares are registered, they will be freely tradable without restriction under the Securities Act.

Lock-up agreements

In connection with this offering, we, our executive officers directors, and holders of all of our outstanding common stock have agreed that, for a period of 180 days from the date of this prospectus, we and they will not, without the prior written consent of J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC, dispose of or hedge any shares or any securities convertible into or exchangeable for our common stock, subject to certain exceptions. J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC in their sole discretion may release any of the securities subject to these lock-up agreements at any time. If the restrictions under the lock-up agreements are waived, shares of our common stock may become available for resale into the market, subject to applicable law, which could reduce the market price for our common stock. See “Underwriting.”

MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES TO NON-U.S. HOLDERS

The following discussion is a summary of the material U.S. federal income tax consequences to non-U.S. holders (as defined below) of the purchase, ownership and disposition of our common stock issued pursuant to this offering. This discussion does not purport to be a complete analysis of all potential tax effects to non-U.S. holders of our common stock. The effects of other U.S. federal tax laws, such as estate and gift tax laws, and any applicable state, local or foreign tax laws are not included in this discussion, and non-U.S. holders should consult their own tax advisors as to these matters. This discussion is based on the Code, Treasury Regulations promulgated thereunder, judicial decisions and published rulings and administrative pronouncements of the Internal Revenue Service, or the IRS, in effect as of the date of this prospectus. These authorities may change or be subject to differing interpretations. Any such change may be applied retroactively in a manner that could adversely affect a non-U.S. holder of our common stock. We have not sought and will not seek any rulings from the IRS regarding the matters discussed below. There can be no assurance that the IRS or a court will not take a contrary position regarding the tax consequences of the purchase, ownership and disposition of our common stock.

This discussion is limited to non-U.S. holders that hold our common stock as a "capital asset" within the meaning of Section 1221 of the Code (generally, property held for investment). This discussion does not address all U.S. federal income tax consequences relevant to a non-U.S. holder's particular circumstances, including the impact of the unearned income Medicare contribution tax and the alternative minimum tax rules. In addition, it does not address consequences relevant to non-U.S. holders subject to particular rules, including, without limitation:

- U.S. expatriates and certain former citizens or long-term residents of the United States;
- persons holding our common stock as part of a hedge, straddle or other risk reduction strategy or as part of a conversion transaction or other integrated investment;
- banks, insurance companies or other financial institutions;
- real estate investment trusts or regulated investment companies;
- brokers, dealers or traders in securities;
- "controlled foreign corporations," "passive foreign investment companies" or corporations that accumulate earnings to avoid U.S. federal income tax;
- partnerships or other entities or arrangements treated as partnerships for U.S. federal income tax purposes;
- tax-exempt organizations or governmental organizations;
- persons deemed to sell our common stock under the constructive sale provisions of the Code;
- persons who hold or receive our common stock pursuant to the exercise of any employee stock option or otherwise as compensation; and
- pension funds.

If a partnership (or other entity or arrangement treated as a partnership for U.S. federal income tax purposes) holds our common stock, the tax treatment of a partner in the partnership will depend on the status of the partner, the activities of the partnership and certain determinations made at the partner level. Accordingly, partnerships holding our common stock and the partners in such partnerships should consult their own tax advisors regarding the U.S. federal income tax consequences to them.

THIS DISCUSSION IS FOR INFORMATION PURPOSES ONLY AND IS NOT INTENDED AS TAX ADVICE. INVESTORS SHOULD CONSULT THEIR OWN TAX ADVISORS WITH RESPECT

TO THE APPLICATION OF THE U.S. FEDERAL INCOME TAX LAWS TO THEIR PARTICULAR SITUATIONS AS WELL AS ANY TAX CONSEQUENCES OF THE PURCHASE, OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK ARISING UNDER THE U.S. FEDERAL ESTATE OR GIFT TAX LAWS OR UNDER THE LAWS OF ANY STATE, LOCAL OR NON-U.S. TAXING JURISDICTION OR UNDER ANY APPLICABLE INCOME TAX TREATY.

Definition of a non-U.S. holder

For purposes of this discussion, a "non-U.S. holder" is any beneficial owner of our common stock that is neither a "U.S. person" nor a partnership for U.S. federal income tax purposes. A U.S. person is any of the following:

- an individual who is a citizen or resident of the United States;
- a corporation (or other entity treated as a corporation for U.S. federal income tax purposes) created or organized under the laws of the United States, any state thereof or the District of Columbia;
- an estate, the income of which is subject to U.S. federal income tax regardless of its source; or
- a trust that (i) is subject to the primary supervision of a U.S. court and the control of one or more U.S. persons (within the meaning of Section 7701(a)(30) of the Code), or (ii) has made a valid election under applicable Treasury Regulations to continue to be treated as a U.S. person.

Distributions

As described in the section of this prospectus captioned "Dividend policy," we do not anticipate making distributions to holders of our common stock in the foreseeable future.

If we do, however, make distributions on our common stock, such distributions of cash or property on our common stock (other than certain pro rata distributions of our stock) generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles.

Subject to the discussion below regarding backup withholding and payments made to certain foreign accounts, dividends paid to a non-U.S. holder of our common stock that are not effectively connected with the non-U.S. holder's conduct of a trade or business within the United States will generally be subject to U.S. federal withholding tax at a rate of 30% of the gross amount of the dividends (or such lower rate as may be specified by an applicable income tax treaty).

Amounts not treated as dividends for U.S. federal income tax purposes will first constitute a return of capital and be applied against and reduce a non-U.S. holder's adjusted tax basis in its common stock, but not below zero. Any excess will be treated as capital gain and will be treated as described below in the section relating to the sale or other taxable disposition of the common stock.

Non-U.S. holders may be entitled to a reduction in or an exemption from withholding on dividends as a result of either (i) qualifying for the benefits of an applicable income tax treaty or (ii) holding our common stock in connection with the conduct of a trade or business within the United States and receiving the dividends in connection with that trade or business. To claim such a reduction in or exemption from withholding, the non-U.S. holder must provide the applicable withholding agent with a properly executed (a) IRS Form W-8BEN or W-8BEN-E (or applicable successor form) claiming an exemption from or reduction of the withholding tax under the benefit of an applicable income tax treaty, (b) IRS Form W-8ECI (or applicable successor form) stating that the dividends are effectively connected with the conduct by the non-U.S. holder of a trade or business within the United States, or (c) a suitable substitute form, as may be applicable. These certifications must be provided to the applicable withholding agent prior to the payment of dividends and must be updated periodically. Non-U.S.

holders that do not timely provide the applicable withholding agent with the required certification, but that qualify for a reduced rate or exemption under an applicable income tax treaty, may obtain a refund of any excess amounts withheld by timely filing an appropriate claim for refund with the IRS.

Subject to the discussion below regarding backup withholding and payments made to certain foreign accounts, if dividends paid to a non-U.S. holder are effectively connected with the non-U.S. holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the non-U.S. holder maintains a permanent establishment in the United States to which such dividends are attributable), then, although exempt from withholding of U.S. federal income tax (provided the non-U.S. holder provides appropriate certification, as described above), the non-U.S. holder will be subject to U.S. federal income tax on such dividends on a net income basis at the regular graduated U.S. federal income tax rates. In addition, a non-U.S. holder that is or is treated as a corporation for U.S. federal income tax purposes may be subject to an additional branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on its effectively connected earnings and profits for the taxable year that are attributable to such dividends, as adjusted for certain items. Non-U.S. holders should consult their own tax advisors regarding their entitlement to benefits under any applicable income tax treaty.

Sale or other taxable disposition

Subject to the discussion below regarding backup withholding and payments made to certain foreign accounts, a non-U.S. holder will not be subject to U.S. federal income tax on any gain realized upon the sale or other taxable disposition of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, the non-U.S. holder maintains a permanent establishment in the United States to which such gain is attributable);
- the non-U.S. holder is a non-resident alien individual present in the United States for 183 days or more during the taxable year of the disposition and certain other requirements are met; or
- our common stock constitutes a U.S. real property interest within the meaning of Section 897 of the Code by reason of our status as a U.S. real property holding corporation, or USRPHC, for U.S. federal income tax purposes.

Gain described in the first bullet point above will generally be subject to U.S. federal income tax on a net income basis at the regular graduated U.S. federal income tax rates. A non-U.S. holder that is a foreign corporation also may be subject to an additional branch profits tax at a rate of 30% (or such lower rate specified by an applicable income tax treaty) on a portion of its effectively connected earnings and profits for the taxable year that are attributable to such gain, as adjusted for certain items.

A non-U.S. holder described in the second bullet point above will be subject to U.S. federal income tax at a rate of 30% (or such lower rate as may be specified by an applicable income tax treaty) on any gain derived from the sale or other taxable disposition, which (even though the individual is not considered a resident of the United States) may be offset by certain U.S. source capital losses of the non-U.S. holder provided the non-U.S. holder timely files U.S. federal income tax returns with respect to such losses.

With respect to the third bullet point above, we believe we are not currently, and do not anticipate that we will become, a USRPHC.

Non-U.S. holders should consult their own tax advisors regarding potentially applicable income tax treaties that may provide for different rules.

Information reporting and backup withholding

A non-U.S. holder generally will not be subject to backup withholding with respect to payments of dividends on our common stock we make to the non-U.S. holder, provided the applicable withholding agent does

not have actual knowledge or reason to know such holder is a U.S. person and the holder certifies its non-U.S. status by providing a valid IRS Form W-8BEN, W-8BEN-E or W-8ECI, or other applicable certification (or applicable successor form), or otherwise establishes an exemption. However, information returns will be filed with the IRS in connection with any dividends on our common stock paid to the non-U.S. holder, regardless of whether any tax was actually withheld. Copies of these information returns may also be made available under the provisions of a specific treaty or agreement to the tax authorities of the country in which the non-U.S. holder resides or is established.

Information reporting and backup withholding may apply to the proceeds of a sale of our common stock within the United States, and information reporting may (although backup withholding will generally not) apply to the proceeds of a sale or other taxable disposition of our common stock outside the United States conducted through certain U.S.-related financial intermediaries, in each case, unless the beneficial owner certifies under penalty of perjury that it is a non-U.S. person on IRS Form W-8BEN, W-8BEN-E, W-8ECI or other applicable form or successor form (and the payer does not have actual knowledge or reason to know that the beneficial owner is a U.S. person) or otherwise establishes an exemption.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules may be allowed as a refund or a credit against a non-U.S. holder's U.S. federal income tax liability, provided the required information is timely furnished to the IRS.

Additional withholding tax on payments made to foreign accounts

Withholding taxes may be imposed under the provisions of the law generally known as the Foreign Account Tax Compliance Act, or FATCA, on certain types of payments made to non-U.S. financial institutions and certain other non-U.S. entities. Specifically, a 30% withholding tax may be imposed on dividends on, or gross proceeds from the sale or other disposition of, our common stock paid to a "foreign financial institution" or a "non-financial foreign entity" (each as defined in the Code), unless (i) the foreign financial institution undertakes certain diligence and reporting obligations, (ii) the non-financial foreign entity either certifies it does not have any "substantial U.S. owners" (as defined in the Code) or furnishes identifying information regarding each substantial U.S. owner or (iii) the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. If the payee is a foreign financial institution and is subject to the diligence and reporting requirements in (i) above, it must enter into an agreement with the U.S. Department of the Treasury requiring, among other things, that it undertakes to identify accounts held by certain "specified U.S. persons" or "U.S.-owned foreign entities" (each as defined in the Code), annually reports certain information about such accounts and withholds 30% on payments to non-compliant foreign financial institutions and certain other account holders. An intergovernmental agreement between the United States and an applicable foreign country, or future Treasury Regulations or other guidance, may modify these requirements. Accordingly, the entity through which our common stock is held will affect the determination of whether such withholding is required.

Under the applicable Treasury Regulations and recent guidance from the IRS, withholding under FATCA generally applies to payments of dividends on our common stock, and will apply to payments of gross proceeds from the sale or other disposition of our common stock on or after January 1, 2019, and to certain "passthru" payments made on or after the later of January 1, 2019 and the date final Treasury Regulations are issued defining such passthru payments. The FATCA withholding tax will apply to all withholdable payments without regard to whether the beneficial owner of the payment would otherwise be entitled to an exemption from imposition of withholding tax pursuant to an applicable tax treaty with the United States or U.S. domestic law. We will not pay additional amounts to holders of our common stock in respect of any amounts withheld.

Prospective investors should consult their own tax advisors regarding the potential application of withholding under FATCA to their investment in our common stock.

UNDERWRITING (CONFLICTS OF INTEREST)

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC are acting as book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the representatives. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the initial public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of Shares
J.P. Morgan Securities LLC	3,125,000
Goldman Sachs & Co. LLC	2,343,750
Leerink Partners LLC	1,562,500
Nomura Securities International, Inc.	390,625
Chardan Capital Markets LLC	390,625
Total	7,812,500

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $0.6720 per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to $0.2240 per share from the initial public offering price. After the initial offering of the shares to the public, the offering price and other selling terms may be changed by the underwriters. Sales of shares made outside of the United States may be made by affiliates of the underwriters. The offering of the shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

Certain of our existing stockholders have agreed to purchase an aggregate of approximately 3,955,000 shares of our common stock in this offering at the initial public offering price. The underwriters will receive the same underwriting discounts and commissions on the shares purchased by these stockholders as they will on the other shares sold to the public in this offering.

The underwriters have an option to buy up to 1,171,875 additional shares of common stock from us to cover sales of shares by the underwriters that exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $1.12 per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without option to purchase additional shares exercise	With full option to purchase additional shares exercise
Per share	$ 1.12	$ 1.12
Total	$ 8,750,000	$ 10,062,500

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $3,800,000. We have agreed to reimburse the underwriters for expenses of up to $50,000 related to clearance of this offering with FINRA.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in this offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or file or confidentially submit with the SEC a registration statement under the Securities Act relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case, without the prior written consent of J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC for a period of 180 days after the date of this prospectus, subject to certain exceptions including, (i) any shares of our common stock to be sold in this offering, (ii) any shares of our common stock issued upon the exercise of options granted under equity compensation plans described in this prospectus, (iii) upon the exercise of any warrant or option or the conversion of the units outstanding as of the date of the this prospectus, (iv) up to 5% of our outstanding securities issued in connection with mergers, acquisitions, licenses or commercial collaboration or strategic transactions, or (v) the filing of a registration statement on Form S-8 (or a successor form) relating to an equity compensation plan described in this prospectus.

Our directors, executive officers and holders of all of our outstanding common stock have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 180 days after the date of this prospectus, or the restricted period, may not, without the prior written consent of J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC, (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including without limitation, common stock or such other securities that may be deemed to be beneficially owned by such directors, executive officers and stockholders in accordance with the rules and regulations of the SEC and securities that may be issued upon exercise of a stock option or warrant) or publicly disclose the intention to make any offer, sale, pledge or disposition, (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise, or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock, in each case, subject to certain exceptions, including:

(A) transactions relating to shares of common stock or other securities purchased in this offering (provided that the seller is not an officer or director of ours) or in open market transactions during the restricted period,

(B) the exercise, including by “net” exercise, so long as exercised in accordance with clauses (C) and (D) below, of any options or warrants to acquire shares of common stock or the conversion of any convertible

security into common stock described in this prospectus, or issued pursuant to an equity plan described in this prospectus, it being understood that any shares of common stock received shall be subject to the restrictions on transfer set forth in the lock-up agreements,

(C) the sale or transfer us of such number of shares of common stock acquired in connection with the exercise of options or warrants on a "net" exercise basis described in the foregoing clause,

(D) the sale or transfer us of such number of shares of common stock necessary to generate only such amount of cash needed for the payment of taxes (including estimated taxes) due as a result of the exercise of such options or warrant described in clause (B),

(E) transfers of shares of common stock as a bona fide gift or gifts or pursuant to a negotiated divorce settlement,

(F) transfers pursuant to a qualified domestic relations order,

(G) distributions or transfers of shares of common stock or other securities to subsidiaries, limited or general partners, members, stockholders or affiliates of, or any investment fund or other entity that controls or manages, the transferor,

(H) transfers of shares of common stock or other securities to any immediate family member, trusts for the direct or indirect benefit of the transferor or the immediate family members of the transferor or any of their successors upon death, or any partnerships or limited liability company, the partners or members of which consist of or are for the direct or indirect benefit of the transferor and/or immediate family members or other dependents of the transferor, (for these purposes, "immediate family" means any relationship by blood, marriage or adoption, not more remote than first cousin),

(I) transfers of shares of common stock or other securities by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the transferor in a transaction not involving a disposition for value,

(J) any forfeiture, sale or other transfer to us of any shares of common stock or other securities in connection with the termination of the transferor's employment with or services to the company, provided that no public announcement reporting a reduction in the beneficial ownership shall be voluntarily made, and any required announcement, including any announcement under the Exchange Act, shall clearly indicate the reason for such reduction, or

(K) exchange of common or preferred units of the company into shares of common stock in connection with the consummation of the Corporate Conversion, it being understood that any such shares of common stock received upon such exchange shall be subject to the restrictions on transfer set forth in the lock-up agreement;

provided that in the case of any transfer or distribution pursuant to clauses (E), (G), (H) and (I), each donee, distributee or transferee shall execute and deliver to J.P. Morgan Securities LLC, Goldman Sachs & Co. LLC and Leerink Partners LLC a lock-up letter in the form of this paragraph; and provided, further, that in the case of any transfer or distribution pursuant to clauses (A) through (E) and (G) through (I), no filing by any party (donor, donee, transferor or transferee) under the Exchange Act or other public announcement reporting a reduction in the beneficial ownership shall be required or shall be made voluntarily in connection with such transfer or distribution (other than a filing on a Form 5 or 13F filing made after the expiration of the restricted period and any required Schedule 13G (or 13G/A)).

The lock-up agreements will not apply to the establishment of a trading plan by any director, executive officer or stockholder pursuant to Rule 10b5-1 under the Exchange Act for the transfer of shares of common stock, provided that such plan does not provide for the transfer of common stock during the restricted period referred to above and no public announcement or filing under the Exchange Act, if any, is required of or is voluntarily made by or on behalf of such director, executive officer or stockholder or us regarding such plan.

The lock-up agreements will not apply to any transfers, sales, tenders or other dispositions of common stock or any security convertible into or exercisable or exchangeable for common stock pursuant to a bona fide third-party tender offer, merger, amalgamation, consolidation or other similar transaction made to or involving all holders of the common stock or such other securities pursuant to a change of control of our ownership (including, without limitation, the entry into any lock-up, voting or similar agreement pursuant to which such directors, executive officers and stockholders may agree to transfer, sell, tender or otherwise dispose of common stock or other such securities in favor of any such transaction); provided that if such tender offer, merger, amalgamation, consolidation or other similar transaction is not completed, any common stock or any security convertible into or exercisable or exchangeable for common stock subject to this lock-up agreement shall remain subject to the restrictions contained in this lock-up agreement.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act.

We have been approved to list our common stock on the NASDAQ Global Select Market under the symbol “SLDB.”

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be “covered” shorts, which are short positions in an amount not greater than the underwriters’ option to purchase additional shares referred to above, or may be “naked” shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act of 1933, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on the NASDAQ Global Select Market, in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors, including:

- the information set forth in this prospectus and otherwise available to the representatives;
- our prospects and the history and prospects for the industry in which we compete;

- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Conflicts of interest

An affiliate of J.P. Morgan Securities LLC, an underwriter in this offering, owns in excess of 10% of our issued and outstanding common stock. Under the Rules of FINRA, J.P. Morgan Securities LLC is deemed to have a conflict of interest with us and accordingly, this offering is being made in compliance with the requirements of Rule 5121 of FINRA. In accordance with this rule, Goldman Sachs & Co. LLC has assumed the responsibilities of acting as a qualified independent underwriter. In its role as qualified independent underwriter, Goldman Sachs & Co. LLC has participated in due diligence and the preparation of this prospectus and the registration statement of which this prospectus is a part. Goldman Sachs & Co. LLC will not receive any additional fees for serving as a qualified independent underwriter in connection with this offering. J.P. Morgan Securities LLC will not confirm sales of the shares to any account over which it exercises discretionary authority without the prior written approval of the customer.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services.

Certain of the underwriters and their affiliates have provided in the past to us and our affiliates and may provide from time to time in the future certain commercial banking, financial advisory, investment banking and other services for us and such affiliates in the ordinary course of their business, for which they have received and may continue to receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their respective affiliates, officers, directors and employees may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future.

One of our directors is an employee of J.P. Morgan Securities LLC, an underwriter participating in this offering. See also “Certain Relationships and Related-Person Transactions—Limited liability company agreement of Solid Biosciences, LLC.”

In March 2017, entities affiliated with Leerink Partners LLC purchased an aggregate of 41,667 units of our Series 1 Senior Preferred Units on the same terms as the other investors, which shares will automatically convert into 35,353 shares of our common stock upon Corporate Conversion. In March 2017, entities affiliated with Leerink Partners LLC purchased an aggregate of 17,042 units of our Series C Common Units on the same terms as the other investors, which shares will automatically convert into 14,460 shares of our common stock upon Corporate Conversion. In October 2017, entities affiliated with Leerink Partners LLC purchased an aggregate of 74,030 units of our Series 2 Senior Preferred Units on the same terms as the other investors, which shares will automatically convert into 62,813 shares of our common stock upon the Corporate Conversion. The Financial Industry Regulatory Authority, Inc. deems these securities to be underwriting compensation. The shares are subject to FINRA's Rule 5110(g), which prohibits transfer for a period of 180 days following the effective date of the offering.

The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of the company's securities and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in the company's securities.

Selling restrictions

European Economic Area

In relation to each Member State of the European Economic Area that has implemented the Prospectus Directive (each, a "Relevant Member State"), an offer to the public of our common shares may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of our common shares may be made at any time under the following exemptions under the Prospectus Directive:

(a) to any legal entity that is a qualified investor as defined in the Prospectus Directive;

(b) to fewer than 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), subject to obtaining the prior consent of the Representatives for any such offer; or

(c) in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to our common shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and our common shares to be offered so as to enable an investor to decide to purchase our common shares, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State; and the expression "Prospectus Directive" means Directive 2003/71/EC (as amended), including by Directive 2010/73/EU, and includes any relevant implementing measure in the Relevant Member State.

Canada

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 *Prospectus Exemptions* or subsection 73.3(1) of the *Securities Act* (Ontario), and are permitted clients, as defined in National Instrument 31-103 *Registration Requirements, Exemptions and Ongoing Registrant Obligations*. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 *Underwriting Conflicts* (NI 33-105), the representatives are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

United Kingdom

This document is only being distributed to and is only directed at (i) persons who are outside the United Kingdom or (ii) to investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, or the Order, or (iii) high net worth entities, and other persons to whom it may lawfully be communicated, falling with Article 49(2)(a) to (d) of the Order (all such persons together being referred to as "relevant persons"). The securities are only available to, and any invitation, offer or agreement to subscribe, purchase or otherwise acquire such securities will be engaged in only with, relevant persons. Any person who is not a relevant person should not act or rely on this document or any of its contents.

Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document, nor any other offering or marketing material relating to the shares or this offering, may be publicly distributed or otherwise made publicly available in Switzerland. Neither this document nor any other offering or marketing material relating to this offering, the Company, the shares has been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

Hong Kong

The shares may not be offered or sold by means of any document other than (i) in circumstances that do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), or (ii) to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap.571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances that do not result in the document being a "prospectus" within the meaning of the Companies Ordinance (Cap.32, Laws of Hong Kong), and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to shares that are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (ii) to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 by a relevant person that is: (a) a corporation (which is not an accredited investor) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiaries' rights and interest in that trust shall not be transferable for six months after that corporation or that trust has acquired the shares under Section 275 except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions, specified in Section 275 of the SFA; (2) where no consideration is given for the transfer; or (3) by operation of law.

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Law of Japan, or the Financial Instruments and Exchange Law, and each underwriter has agreed that it will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term, as used in this prospectus means any person resident in Japan, including any corporation or other entity organized under the laws of Japan) or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Law and any other applicable laws, regulations and ministerial guidelines of Japan.

United Arab Emirates

This prospectus relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or DFSA. This prospectus is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth herein and has no responsibility for this prospectus. The shares to which this prospectus relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus, you should consult an authorized financial advisor.

LEGAL MATTERS

Proskauer Rose LLP will pass upon the validity of the shares of common stock offered hereby for us. The underwriters are represented by Davis Polk & Wardwell LLP.

EXPERTS

The financial statements as of December 31, 2015 and 2016 and for each of the two years in the period ended December 31, 2016 included in this prospectus have been so included in reliance on the report (which contains an explanatory paragraph relating to the Company's ability to continue as a going concern as described in Note 1 to the financial statements) of PricewaterhouseCoopers LLP, an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

Change in our public accounting firm

In September 2016, we dismissed Katz, Nannis + Solomon, P.C., or KN+S, as our independent accountants. The decision to dismiss KN+S as our independent registered public accounting firm was approved by the board of managers of Solid Biosciences, LLC.

KN+S had reported on our consolidated financial statements as of and for the year ended December 31, 2015.

The report of KN+S on our 2015 consolidated financial statements did not contain any adverse opinion or disclaimer of opinion, nor was such report qualified or modified as to uncertainty, audit scope or accounting principles.

During the year ended December 31, 2015 and through the date of dismissal, there were no disagreements between us and KN+S on any matter of accounting principles or practices, financial statement disclosure or auditing scope or procedure, which disagreement, if not resolved to the satisfaction of KN+S, would have caused it to make reference to the subject matter of the disagreement in connection with its reports.

None of the reportable events described under Item 304(a)(1)(v) of Regulation S-K occurred during the years ended December 31, 2015 and through the date of dismissal of KN+S.

We engaged PricewaterhouseCoopers LLP, or PwC, as our independent registered public accounting firm on March 6, 2017 to audit our consolidated financial statements as of and for the years ended December 31, 2015 and 2016.

During our year ended December 31, 2015 and in the subsequent interim period through March 31, 2017, other than in the normal course of the audit, neither we nor anyone on our behalf consulted with PwC regarding either: (i) the application of accounting principles to a specified transaction, either completed or proposed, or the type of audit opinion that might be rendered on our financial statements, and neither a written report was provided to us or oral advice was provided to us that PwC concluded was an important factor considered by us in reaching a decision as to the accounting, auditing or financial reporting issue; or (ii) any matter that was either the subject of a disagreement or reportable event as defined in Regulation S-K, Item 304(a)(1)(iv) and Item 304(a)(1)(v), respectively.

We delivered a copy of this disclosure to KN+S and requested that they furnish us a letter addressed to the SEC stating whether they agree with the above statements. In their letter to the SEC dated July 24, 2017, attached as Exhibit 16.1 to the registration statement of which this prospectus forms a part, KN+S states that they agree with the statements above concerning their firm.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of our common stock being offered by this prospectus. This prospectus, which constitutes part of that registration statement, does not contain all of the information set forth in the registration statement or the exhibits and schedules that are part of the registration statement. Some items included in the registration statement are omitted from the prospectus in accordance with the rules and regulations of the SEC. For further information with respect to us and the common stock offered in this prospectus, we refer you to the registration statement and the accompanying exhibits and schedules filed therewith. Statements contained in this prospectus regarding the contents of any contract or any other document that is filed as an exhibit to the registration statement are not necessarily complete, and each such statement is qualified in all respects by reference to the full text of such contract or other document filed as an exhibit to the registration statement.

A copy of the registration statement and the accompanying exhibits and any other document we file may be inspected without charge at the public reference facilities maintained by the SEC at 100 F Street, N.E., Washington, D.C. 20549 and copies of all or any part of the registration statement may be obtained from that office upon the payment of the fees prescribed by the SEC. The public may obtain information on the operation of the public reference facilities in Washington, D.C. by calling the SEC at 1-800-SEC-0330. Our filings with the SEC are available to the public from the SEC's website at www.sec.gov.

Upon the completion of this offering, we will be subject to the information and periodic reporting requirements of the Exchange Act and, in accordance therewith, we will file proxy statements, periodic information and other information with the SEC. All documents filed with the SEC are available for inspection and copying at the public reference room and website of the SEC referred to above. We maintain a website at www.solidbio.com. You may access our reports, proxy statements and other information free of charge at this website as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not incorporated by reference and is not a part of this prospectus.

INDEX TO CONSOLIDATED FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

To the Members, Unitholders and Board of Managers of
Solid Biosciences, LLC

In our opinion, the accompanying consolidated balance sheets and the related consolidated statements of operations, of comprehensive loss, of redeemable preferred units and members' deficit and of cash flows present fairly, in all material respects, the financial position of Solid Biosciences, LLC and its subsidiaries as of December 31, 2016 and 2015, and the results of their operations and their cash flows for the years then ended in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these financial statements in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the consolidated financial statements, the Company has incurred recurring losses from operations since inception, has an accumulated deficit, and will require additional financing to fund future operations that raise substantial doubt about its ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The financial statements do not include any adjustments that might result from the outcome of this uncertainty.

/s/ PricewaterhouseCoopers LLP

Boston, Massachusetts
August 4, 2017

SOLID BIOSCIENCES, LLC
CONSOLIDATED BALANCE SHEETS
(In thousands, except unit and per unit data)

	December 31,		September 30, 2017	
	2015	2016	Actual (unaudited)	Pro forma (unaudited)
Assets				
Current assets:				
Cash and cash equivalents	$ 28,595	$ 7,678	$ 15,017	$ 15,017
Available-for-sale securities	26,792	29,980	14,553	14,553
Prepaid expenses and other current assets	309	2,314	1,182	1,182
Restricted cash	—	—	65	65
Total current assets	55,696	39,972	30,817	30,817
Property and equipment, net	—	452	2,272	2,272
Restricted cash	—	165	97	97
Deferred offering costs	—	47	2,259	2,259
Total assets	$ 55,696	$ 40,636	$ 35,445	$ 35,445
Liabilities, Redeemable Preferred Units and Members' Deficit/ Stockholders' Equity				
Current liabilities:				
Accounts payable	$ 608	$ 2,984	$ 6,403	6,403
Accrued expenses and other current liabilities	1,312	3,889	4,921	4,921
Redeemable Preferred unit tranche right	12,004	—	—	—
Series 1 Senior Preferred unit tranche right	—	—	527	527
Total current liabilities	13,924	6,873	11,851	11,851
Total liabilities	13,924	6,873	11,851	11,851
Commitments and Contingencies (Note 13)				
Redeemable Preferred Units, 60,000,000 units authorized at December 31, 2015 and 2016 and no units authorized at September 30, 2017 (unaudited); 13,680,000 and 17,100,000 units issued and outstanding at December 31, 2015 and 2016, respectively, and no units issued and outstanding at September 30, 2017 (unaudited); aggregate liquidation preference of $55,746 and $0 at December 31, 2016 and September 30, 2017 (unaudited), respectively; no shares authorized, issued or outstanding, pro forma as of September 30, 2017 (unaudited)	61,697	71,649	—	—
Series 2 Senior Preferred Units, no units authorized at December 31, 2015 and 2016 and 1,973,430 units authorized at September 30, 2017 (unaudited); no units issued and outstanding at December 31, 2015 and 2016 and September 30, 2017 (unaudited); no shares authorized, issued or outstanding, pro forma as of September 30, 2017 (unaudited)	—	—	—	—
Series 1 Senior Preferred Units, no units authorized at December 31, 2015 and 2016 and 2,500,000 units authorized at September 30, 2017 (unaudited); no units issued and outstanding December 31, 2015 and 2016 and 2,500,000 units issued and outstanding at September 30, 2017 (unaudited); aggregate liquidation preference of $25,000 at September 30, 2017 (unaudited); no shares authorized, issued or outstanding, pro forma as of September 30, 2017 (unaudited)	—	—	25,000	—
Junior Preferred Units, no units authorized at December 31, 2015 and 2016 and 4,414,356 units authorized at September 30, 2017 (unaudited); no units issued and outstanding at December 31, 2015 and 2016 and 4,414,356 units issued and outstanding at September 30, 2017 (unaudited); aggregate liquidation preference of $42,500 at September 30, 2017 (unaudited); no shares authorized, issued or outstanding, pro forma as of September 30, 2017 (unaudited)	—	—	44,177	—
Members' deficit/stockholders' equity				
Series A, B, C and D Common Units, 20,000,000 units authorized at December 31, 2015 and 2016 and 20,189,509 units authorized at September 30, 2017 (unaudited); 5,015,917 units and 5,123,917 units issued and outstanding at December 31, 2015 and 2016 and 19,241,003 units issued and outstanding at September 30, 2017 (unaudited)	208	558	64,191	—
Common stock; $0.001 par value per share, no shares authorized, issued or outstanding at December 31, 2015 and 2016 and September 30, 2017 (unaudited); 300,000,000 shares authorized, 22,192,762 shares issued and outstanding, pro forma as of September 30, 2017 (unaudited)	—	—	—	22
Additional paid-in-capital	—	—	—	133,346
Accumulated other comprehensive income (loss)	(10)	23	(3)	(3)
Accumulated deficit	(67,711)	(84,941)	(109,771)	(109,771)
Total (members' deficit)/stockholders' equity	(67,513)	(84,360)	(45,583)	23,594
Non-controlling interest	47,588	46,474	—	—
Total (deficit)/stockholders' equity	(19,925)	(37,886)	(45,583)	23,594
Total liabilities, redeemable preferred units and members' deficit/stockholders' equity	$ 55,696	$ 40,636	$ 35,445	$ 35,445

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

SOLID BIOSCIENCES, LLC
CONSOLIDATED STATEMENTS OF OPERATIONS
(In thousands, except unit and per unit data)

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	4,192	20,116	13,048	27,959
General and administrative	2,372	5,460	3,807	11,737
Total operating expenses	6,564	25,576	16,855	39,696
Loss from operations	(6,564)	(25,576)	(16,855)	(39,696)
Other income (expense):				
Revaluation of preferred unit tranche rights	(103)	1,163	1,163	(68)
Interest income	3	369	270	165
Other income	—	271	168	908
Total other income (expense), net	(100)	1,803	1,601	1,005
Net loss	$ (6,664)	$ (23,773)	$ (15,254)	$ (38,691)
Net loss attributable to non-controlling interest	(287)	(2,234)	(1,471)	(1,060)
Net loss attributable to Solid Biosciences, LLC	$ (6,377)	$ (21,539)	$ (13,783)	$ (37,631)
Decretion (accretion) of preferred units to redemption value	(68)	4,309	1,198	(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 unitholders	$ (6,445)	$ (17,230)	$ (12,585)	$ (24,830)
Net loss per unit attributable to common unitholders, basic and diluted	$ (7.61)	$ (10.14)	$ (7.50)	$ (1.99)
Weighted average common units outstanding, basic and diluted	846,569	1,698,904	1,677,909	12,446,769
Unaudited pro forma net loss per share attributable to common stockholders, basic and diluted		$ (1.62)		$ (2.05)
Unaudited pro forma weighted average common shares outstanding, basic and diluted		14,052,917		19,233,147

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

SOLID BIOSCIENCES, LLC
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands)

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Net loss	$(6,664)	$(23,773)	$(15,254)	$(38,691)
Other comprehensive loss:				
Unrealized gain (loss) on available-for-sale securities	(10)	33	64	(26)
Comprehensive loss	(6,674)	(23,740)	(15,190)	(38,717)
Comprehensive loss attributable to non-controlling interest	(287)	(2,234)	(1,471)	(1,060)
Comprehensive loss attributable to Solid Biosciences, LLC	$(6,387)	$(21,506)	$(13,719)	$(37,657)

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

SOLID BIOSCIENCES, LLC
CONSOLIDATED STATEMENTS OF REDEEMABLE PREFERRED UNITS AND MEMBERS' DEFICIT
(In thousands except for unit data)

	Redeemable Preferred Units	Amount	Series 1 Senior Preferred Units	Amount	Junior Preferred Units	Amount	Series A, B, C and D Common Units	Amount	Accumulated other comprehensive income (loss)	Accumulated Members' Deficit	Total Members' Deficit	Non-controlling Interest	Total Deficit
Balance at December 31, 2014	6,840,000	$ 30,781	—	—	—	—	4,729,667	$ 68	—	$ (61,266)	$ (61,198)	$ 2,499	$(58,699)
Issuance of preferred units	6,840,000	6,840	—	—	—	—	—	—	—	—	—	—	—
Reclassification of tranche right upon issuance of preferred units	—	24,008	—	—	—	—	—	—	—	—	—	—	—
Accretion in redemption value of preferred units	—	68	—	—	—	—	—	—	—	(68)	(68)	—	(68)
Issuance of Series A common units	—	—	—	—	—	—	305,000	—	—	—	—	—	—
Repurchase of Series A common units	—	—	—	—	—	—	(18,750)	—	—	—	—	—	—
Equity based compensation expense	—	—	—	—	—	—	—	140	—	—	140	624	764
Issuance of non-controlling interest in Solid GT	—	—	—	—	—	—	—	—	—	—	—	44,752	44,752
Unrealized loss on available for sale securities	—	—	—	—	—	—	—	—	$ (10)	—	(10)	—	(10)
Net loss	—	—	—	—	—	—	—	—	—	(6,377)	(6,377)	(287)	(6,664)
Balance at December 31, 2015	13,680,000	61,697	—	—	—	—	5,015,917	208	(10)	(67,711)	(67,513)	47,588	(19,925)
Issuance of preferred units	3,420,000	3,420	—	—	—	—	—	—	—	—	—	—	—
Reclassification of tranche right upon issuance of preferred units	—	10,841	—	—	—	—	—	—	—	—	—	—	—
Decretion in redemption value of preferred units	—	(4,309)	—	—	—	—	—	—	—	4,309	4,309	—	4,309
Issuance of Series A common units	—	—	—	—	—	—	108,000	—	—	—	—	—	—
Equity based compensation expense	—	—	—	—	—	—	—	350	—	—	350	1,120	1,470
Unrealized gain on available for sale securities	—	—	—	—	—	—	—	—	33	—	33	—	33
Net loss	—	—	—	—	—	—	—	—	—	(21,539)	(21,539)	(2,234)	(23,773)
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	—	—	—	—	—	—	—	4,207	—	—	4,207	300	4,507
Net loss	—	—	—	—	—	—	—	—	—	(37,631)	(37,631)	(1,060)	(38,691)
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	—	—	—	—	—	—	641,140	—	—	—	—	—	—
Unrealized loss on available for sale securities	—	—	—	—	—	—	—	—	(26)	—	(26)	—	(26)
Balance at September 30, 2017 (unaudited)	—	—	2,500,000	$ 25,000	4,414,356	$ 44,177	19,241,003	$ 64,191	$ (3)	$ (109,771)	$ (45,583)	—	$(45,583)

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

SOLID BIOSCIENCES, LLC
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Cash flows from operating activities:				
Net loss	$ (6,664)	$(23,773)	$(15,254)	$(38,691)
Adjustments to reconcile net loss to net cash used in operating activities:				
Amortization of premium on available for sale securities	5	505	379	194
Equity-based compensation expense	764	1,470	1,122	4,507
Depreciation expense	—	56	22	225
Loss / (gain) from revaluation of preferred unit tranche right	103	(1,163)	(1,163)	68
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	(309)	(2,005)	(2,216)	1,132
Accounts payable	585	2,213	278	2,279
Accrued expenses and other current liabilities	1,312	2,577	2,286	1,044
Net cash used in operating activities	(4,204)	(20,120)	(14,546)	(29,242)
Cash flows from investing activities:				
Purchases of property and equipment	—	(392)	(231)	(1,950)
Proceeds from sales and maturities of available for sale securities	—	22,035	15,113	26,350
Purchases of available for sale securities	(26,806)	(25,695)	(24,583)	(11,143)
Changes in restricted cash	—	(165)	(165)	3
Net cash provided by (used in) investing activities	(26,806)	(4,217)	(9,866)	13,260
Cash flows from financing activities:				
Proceeds from issuance of Series 1 Senior preferred units	—	—	—	24,500
Payment of deferred offering costs	—	—	—	(1,179)
Proceeds from issuance of redeemable preferred units	6,840	3,420	—	—
Proceeds from issuance of non-controlling interest in Solid GT	44,752	—	—	—
Net cash provided by financing activities	51,592	3,420	—	23,321
Net increase (decrease) in cash and cash equivalents	20,582	(20,917)	(24,412)	7,339
Cash and cash equivalents at beginning of period	8,013	28,595	28,595	7,678
Cash and cash equivalents at end of period	$ 28,595	$ 7,678	$ 4,183	$ 15,017
Supplemental disclosure of non-cash investing and financing activities:				
Reclassification of preferred unit tranche liability to preferred units upon settlement	$ 24,008	$ 10,841	—	—
Decretion (accretion) to redemption value for redeemable preferred units	$ (68)	$ 4,309	$ 1,198	$ (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 accrued expenses	—	$ 47	—	$ 1,080
Property and equipment included in accounts payable	—	$ 116	$ 10	$ 211
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 consolidated financial statements.

SOLID BIOSCIENCES, LLC
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(Information as of September 30, 2017 and for the nine months ended September 30, 2016 and 2017 is unaudited)
(Amounts in thousands, except unit and per unit data)

1. Nature of the Business and Basis of Presentation

Solid Biosciences, LLC (the "Company") was organized under the laws of the State of Delaware 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'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 (RPDD) 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 for SGT-001 in the United States during the fourth quarter of 2017.

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 other pharmaceutical and biotechnology companies. In addition, the Company is dependent upon the services of its employees, partners and consultants.

The accompanying consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business. Through December 31, 2016 and September 30, 2017, the Company has funded its operations primarily with proceeds from the sale of redeemable preferred units. The Company has incurred recurring losses from operations since its inception, including a net loss of $23,773 for the year ended December 31, 2016 and $38,691 for the nine months ended September 30, 2017. In addition, as of December 31, 2016 and September 30, 2017, the Company had an accumulated members' deficit of $84,941 and $109,771, respectively. The Company expects to continue to generate operating losses for the foreseeable future. The Company expects that its cash, cash equivalents and available-for-sale securities of $29,570 as of September 30, 2017 together with the $55,000 of net proceeds raised through the issuance of the Series 2 Senior

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1. Nature of the Business and Basis of Presentation—(Continued)

Preferred Units, as discussed below, will be sufficient to fund its operating expenses and capital expenditure requirements through June 30, 2018. The future viability of the Company beyond that point is dependent on its ability to obtain additional financing to fund future operations. The circumstances described above raise substantial doubt about the Company's ability to continue as a going concern as of December 31, 2016 and September 30, 2017. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

On October 26, 2017, the Company issued 4,886,000 Series 2 Senior Preferred Units (the "Series 2 Senior Preferred Units") at an issuance price of $11.26 for net proceeds of $55,000.

The Company is also seeking to complete an initial public offering of its common stock. Upon the closing of a qualified public offering on specific terms, the Company's outstanding preferred units and common units will automatically convert into common shares. See Note 10, *Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units* for additional information.

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, 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 the financing, the terms of any financing may adversely affect the holdings or the rights of the Company's unitholders. 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 eventual 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.

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 are included in the Company's 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 continues 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 has 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 consolidated statements of operations based on a profit and loss sharing arrangement between the Company and the non-controlling interests. The Company also presents the change in equity related to equity-based compensation issued to Solid GT employees by Solid GT, in non-controlling interest. See Note 12, *Equity-Based Compensation* for additional information.

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. See Note 3, *Merger and Recapitalization*, for additional information.

The proportionate share of the loss attributed to the non-controlling interest amounted to $287 and $2,234 and $1,471 and $1,060 for the years ended December 31, 2015 and 2016 and the nine months ended September 30, 2016 and 2017, respectively.

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1. Nature of the Business and Basis of Presentation—(Continued)

The carrying value of the non-controlling interest was $47,588 and $46,474 at December 31, 2015 and 2016. There was no non-controlling interest at September 30, 2017.

The accompanying consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the United States of America ("GAAP"). The accompanying consolidated financial statements include the accounts of Solid Biosciences, LLC and its wholly owned or controlled subsidiaries. All intercompany accounts and transactions have been eliminated.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of the Company's consolidated financial statements in conformity with GAAP requires management to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, the recognition of research and development expenses and the valuation of restricted common units and the preferred unit tranche rights. 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.

Unaudited Interim Financial Information

The accompanying consolidated balance sheet as of September 30, 2017, the consolidated statements of operations, comprehensive loss and cash flows for the nine months ended September 30, 2016 and 2017, and the consolidated statement of redeemable preferred units and members' deficit for the nine months ended September 30, 2017 are unaudited. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of September 30, 2017, and the results of its operations and its cash flows for the nine months ended September 30, 2016 and 2017. The financial data and other information disclosed in these notes related to the nine months ended September 30, 2016 and 2017 are unaudited. The results for the nine months ended September 30, 2017 are not necessarily indicative of results to be expected for the year ending December 31, 2017 or any other interim periods, or any future year or period.

Unaudited Pro Forma Information

The accompanying unaudited pro forma consolidated balance sheet as of September 30, 2017 has been prepared to give effect to the Company's conversion to a corporation whereby all outstanding Series 1 Senior Preferred Units, Junior Preferred Units and Series A, B, C and D Common Units are converted on a one-for-0.8485 basis into shares of common stock as if the proposed Corporate Conversion had occurred on September 30, 2017.

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(Information as of September 30, 2017 and for the nine months ended September 30, 2016 and 2017 is unaudited)
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2. Summary of Significant Accounting Policies—(Continued)

In the accompanying consolidated statements of operations, unaudited pro forma basic and diluted net loss per unit attributable to common unitholders for the year ended December 31, 2016 and the nine months ended September 30, 2017 have been prepared to give effect to the Company's conversion to a corporation whereby all outstanding Series 1 Senior Preferred Units, Junior Preferred Units and Series A, B, C and D Common Units are converted into shares of common stock as if the proposed Corporate Conversion had occurred on the later of January 1, 2016 or the issuance date of the preferred and common units.

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 $100 and $65 in separate restricted bank accounts as a security deposit for the Company's credit card program and for the lease of the Company's facility, respectively, as of December 31, 2016. The Company has classified these deposits as long-term assets on its balance sheets at such date. There was no restricted cash at December 31, 2015.

The Company held restricted cash of $97 and $65 in separate restricted bank accounts as a security deposit for the Company's credit card program and for the lease of the Company's facility, respectively, as of September 30, 2017. The Company has included the amount of $97 as a long-term asset and the amount of $65 as a current asset as of September 30, 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 securities are reported as a separate component of members' deficit. 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 consolidated statement of operations. If any adjustment to fair value reflects a decline in the value of the investment that the Company considers to be "other than temporary," the Company reduces the investment to fair value through a charge to the consolidated statement of operations. No such adjustments were necessary during the periods presented.

Concentration of Credit Risk and of Significant Suppliers

Financial instruments that potentially expose the Company to concentrations of credit risk consist primarily of cash and cash equivalents. Periodically, the Company maintains deposits in accredited financial institutions in

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2. Summary of Significant Accounting Policies—(Continued)

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 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, available-for-sale securities and the preferred unit tranche rights 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.

Deferred Offering Costs

The Company capitalizes certain legal and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded as a reduction of the proceeds generated as a result of the offering. Should the planned equity financing be abandoned, the deferred offering costs will be expensed immediately as a charge to operating expense in the consolidated statements of operations. Deferred offering costs amounted to $47 at December 31, 2016 and $2,259 at September 30, 2017. There were no deferred offering costs at December 31, 2015.

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2. Summary of Significant Accounting Policies—(Continued)

Property and Equipment

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the useful life of the asset. Laboratory equipment is depreciated over five years. Computer equipment is depreciated over three years. Computer software is depreciated over two years. Furniture and office equipment are depreciated over five years. Leasehold improvements are amortized over the shorter of the lease term or the estimated useful life of the related asset. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations.

Impairment of Long-Lived Assets

Long-lived assets, comprised of property and equipment, to be held and used are tested for recoverability whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. Factors that the Company considers in deciding when to perform an impairment review include significant underperformance of the business in relation to expectations, significant negative industry or economic trends and significant changes or planned changes in the use of the assets. If an impairment review is performed to evaluate a long-lived asset for recoverability, the Company compares forecasts of undiscounted cash flows expected to result from the use and eventual disposition of the long-lived asset to its carrying value. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of an asset are less than its carrying amount. The impairment loss would be based on the excess of the carrying value of the impaired asset over its fair value, determined based on discounted cash flows. To date, the Company has not recorded any impairment losses or disposals on long-lived assets.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses include salaries, equity-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company’s research and development activities, including allocated facility-related expenses and external costs of outside vendors engaged to conduct both pre-clinical studies and clinical trials. Non-refundable pre-payments for goods or services that will be used or rendered for future research and development activities are deferred and capitalized. Such amounts are recognized as expense as the goods or services are delivered or the related services are performed, or until it is no longer expected that the goods will be delivered or the services rendered.

Research Contract Costs and Accruals

The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable, and related payments are recorded as research and development expenses as incurred. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates are made in determining the accrued balances at the end of any reporting period. Actual results could differ from the

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2. Summary of Significant Accounting Policies—(Continued)

Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Patent Costs

All patent-related costs incurred for filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses.

Equity-Based Compensation

The Company measures restricted common units 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. Generally, the Company issues restricted common units 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 measures restricted common unit 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 nonemployees 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 units.

The Company classifies equity-based compensation expense in its consolidated statements of operations in the same manner in which the award recipients' payroll costs are classified or in which the award recipients' service payments are classified.

The fair value of each restricted common unit was determined based on a number of objective and subjective factors consistent with the methodologies outlined in the American Institute of Certified Public Accountants Practice Aid, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, including the contemporaneous valuations of the Company's common units, the Company's financial condition and operating results, the material risks related to the Company's business, the Company's stage of development and business strategy and the likelihood of achieving a liquidity event for the holders of the Company's common units such as an initial public offering given prevailing market conditions.

Income Taxes

The Company is treated as a partnership for income tax purposes and is not subject to U.S. federal or state income taxation. As a result, the Company has not recorded any U.S. federal or state income tax benefits for the net losses incurred in each reporting period or for any earned research and development tax credits. To date, the operating losses incurred by the Company have been passed through to its members.

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2. Summary of Significant Accounting Policies—(Continued)

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 members' deficit that result from transactions and economic events other than those with members. The Company's only element of other comprehensive income (loss) in all periods presented was unrealized gains (losses) from available-for-sale securities.

Net Loss per Unit

The Company applies the two-class method to calculate its basic and diluted net loss per unit attributable to common unitholders, as its preferred units and certain unvested common units are considered participating securities. The two-class method determines net income (loss) per unit for each class of common and participating securities according to participation rights in undistributed earnings. The two-class method requires income available to common unitholders 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. As holders of the Company's participating securities, which include Redeemable Preferred Units, Series 1 Senior Preferred Units, Junior Preferred Units and certain unvested common units, do not have a contractual obligation to fund the losses of the Company, the net loss is not allocated between common units and participating securities.

The exchange of Series A Common Units to Series B and Series D Common Units as the result of Merger and Recapitalization described in Note 3 is treated similar to a stock split for the purposes of presenting weighted-average units outstanding. The Company's weighted-average number of common units for the periods prior to Merger and Recapitalization, therefore, have been retroactively adjusted to reflect the exchange of vested Series A Common Units into vested Series B and vested Series D Common Units. Accordingly, for the period subsequent to the Merger and Recapitalization, weighted-average units outstanding include newly issued Series A Common Units, vested Series B, vested Series D Common Units and Series C Common Units. Although each series of units has different rights, losses are shared equally among each of the series of common units and therefore, net loss per unit is the same for each series of common units.

The Company's basic and diluted net loss per unit are the same because the Company has generated a net loss in all periods presented and potentially dilutive securities are excluded from diluted net loss per unit because they have an anti-dilutive impact.

Preferred Unit Tranche Rights

Included in the terms of the Redeemable Preferred Unit Purchase Agreement was a Redeemable Preferred Unit Tranche Right granted to the holders of the Redeemable Preferred Units. Included in the terms of the Series 1 Senior Preferred Unit Purchase Agreement was a Series 1 Senior Preferred Unit Tranche Right granted to the holders of the Series 1 Senior Preferred Units.

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2. Summary of Significant Accounting Policies—(Continued)

The Redeemable Preferred Unit Tranche Right and the Series 1 Preferred Unit Tranche Right, together the Tranche Rights, obligate the holders to purchase additional preferred units under certain conditions. The Tranche Rights also provide the holders with the right to purchase these additional units. The Tranche Rights meet the definition of a freestanding financial instrument as the Tranche Rights are legally detachable and separately exercisable from the Redeemable Preferred Units and the Series 1 Senior Preferred Units. The Tranche Rights are initially recorded at fair value and are subsequently re-measured at fair value each reporting period. Changes in the fair market value are recognized as a component of other income (expense), net, in the consolidated statements of operations.

Funding from Charitable Organizations

The Company has received funding from charitable organizations to perform research and development services to identify therapies for people with DMD. The amounts received are recognized as services are performed and research expenses are incurred. These are included in other income in the consolidated statements of operations as the arrangement between the Company and the charitable organizations are not part of the Company's on-going, major or central operations. Any amount received in advance of services performed is recorded in accrued expenses and other current liabilities in the consolidated balance sheets if the services are expected to be performed within the next twelve months.

The Company recognized other income of $271, $168 and $908 for the year ended December 31, 2016, and the nine months ended September 30, 2016 and 2017, respectively, which is included in the consolidated statements of operations. There was no other income recorded for the year ended December 31, 2015.

Contingencies

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

Recently Adopted Accounting Pronouncements

In March 2016, the FASB issued ASU No. 2016-09, *Compensation—Stock Compensation (Topic 718): Improvements to Employee Share-Based Payment Accounting* ("ASU 2016-09"). ASU 2016-09 includes multiple provisions intended to simplify various aspects of the accounting for share-based payments, including the income tax consequences, classification of awards as either equity or liabilities, an option to recognize gross share

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2. Summary of Significant Accounting Policies—(Continued)

compensation expense with actual forfeitures recognized as they occur, as well as certain classifications on the statement of cash flows. The Company elected to early adopt the standard on January 1, 2016. The adoption of ASU 2016-09 had no material impact on the Company's financial position, results of operations or cash flows. The Company elected to account for forfeitures as they occur rather than apply an estimated forfeiture rate to share-based compensation expense.

In August 2014, the FASB issued ASU No. 2014-15, *Disclosure of Uncertainties About an Entity's Ability to Continue as a Going Concern* ("ASU 2014-15"). ASU 2014-15 amends Accounting Standards Codification ("ASC") 205-40, *Presentation of Financial Statements—Going Concern*, by providing guidance on determining when and how reporting entities must disclose going-concern uncertainties in their financial statements, including requiring management to perform interim and annual assessments of an entity's ability to continue as a going concern within one year of the date of issuance of the entity's financial statements and providing certain disclosures if there is substantial doubt about the entity's ability to continue as a going concern. The standard is effective for public companies for annual periods ending after December 15, 2016 and interim periods within annual periods beginning after December 15, 2016. The Company has adopted this standard for the year ended December 31, 2016 and its adoption had no impact on the Company's financial position, results of operations or cash flows.

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers*, which supersedes the revenue recognition requirements in ASC 605-25, *Multiple-Element Arrangements* and most industry-specific guidance. The new standard requires that an entity recognize revenue to depict the transfer of promised goods or services to customers in an amount that reflects the consideration to which the company expects to be entitled in exchange for those goods or services. The update also requires additional disclosure about the nature, amount, timing, and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. This new guidance will be effective for annual reporting periods (including interim reporting periods within those years) beginning on January 1, 2018. Early adoption in 2017 is permitted. Companies have the option of applying this new guidance retrospectively to each prior reporting period presented or retrospectively with the cumulative effect of initially applying this update recognized at the date of initial application. The Company elected to early adopt the standard on January 1, 2017. The Company does not have any revenue generating arrangements and the adoption of this standard had no impact on the Company's financial position, results of operations or cash flows.

Recently Issued Accounting Pronouncements

In May 2017, the FASB issued 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 guidance is effective for annual periods beginning after December 15, 2017, with early adoption permitted, including adoption in any interim period for which financial statements have not yet been issued. Upon adoption of this standard, the Company will apply modification accounting in accordance with the standard.

In November 2016, the FASB issued ASU 2016-18, *Statement of Cash Flows*, which requires that amounts generally described as restricted cash and restricted cash equivalents should be included with cash and cash

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2. Summary of Significant Accounting Policies—(Continued)

equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU 2016-18 is effective for fiscal years beginning after December 15, 2018 and should be applied using a retrospective transition method to each period presented. Early adoption is permitted. The Company is in the process of evaluating the impact of ASU 2016-17 on its financial statements.

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 for fiscal years, and for interim periods within those fiscal years, beginning after December 15, 2017, with early adoption permitted. The adoption of this standard is not expected to have a material impact on our statements of cash flows upon adoption.

In February 2016, the FASB issued ASU No. 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 (ASC Topic 842) 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. Early adoption is permitted. The Company is currently evaluating the impact that the adoption of ASU 2016-02 will have on its consolidated financial statements.

3. Merger and Recapitalization

On March 29, 2017, the Company 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 the Company pursuant to the merger agreement between the Company and Solid GT (the "Merger Agreement"), collectively referred to as the "Merger and Recapitalization." As part of the Merger and Recapitalization, the Company (a) issued 2,500,000 Series 1 Senior Preferred Units to new investors at $10.00 per unit resulting in gross proceeds to the Company of $25,000, (b) merged operations of Solid GT into the Company, effected through the exchange of Solid GT units held by non-controlling interests of the Company into new classes of the Company units, and (c) exchanged existing Redeemable Preferred Units and Series A Common Units of the Company 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, the Company issued 2,500,000 Series 1 Senior Preferred Units to new investors at $10.00 per unit resulting in gross proceeds to the Company of $25,000.

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3. Merger and Recapitalization—(Continued)

See Note 10, *Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units*, for additional information.

(b) Merger of Solid GT into the Company

Prior to the Merger and Recapitalization, the Company issued Class B Non-Voting and Class D Voting Units of Solid GT to holders which represent non-controlling interests of the Company. 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 the Company; 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 the Company

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 the Company. The Solid GT Class C Restricted Units were held by employees and consultants of Solid GT. See Note 12, *Equity-Based Compensation*, for additional information.

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 impacts net loss attributable to common unitholders. See Note 15, *Net Loss Per Unit*, for additional information.

(c) Exchange of the Company's existing Redeemable Preferred Units and Series A Common Units

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

- 17,100,000 Redeemable Preferred Units of the Company were exchanged for 12,219,299 Series A Common Units of the Company. See Note 10, *Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units*, for additional information.
- 4,560,000 Series A Common Units of the Company were exchanged for 3,258,480 Series B Common Units of the Company. See Note 11, *Members Deficit*, for additional information.
- 563,917 Series A Common Units of the Company were exchanged for 402,963 Series D Common Units of the Company. See Note 11, *Members Deficit*, for additional information.

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3. Merger and Recapitalization—(Continued)

The table below displays the pre-merger and post-merger capitalization structure of the Company:

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 December 31, 2015 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Available for sale securities	$ —	$ 26,792	$—	$ 26,792
Liabilities:				
Redeemable Preferred Unit tranche liability	$ —	$—	$ 12,004	$ 12,004

	Fair Value Measurements as of December 31, 2016 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Available for sale securities	$ —	$ 29,980	$ —	$ 29,980

	Fair Value Measurements as of September 30, 2017 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
		(unaudited)		
Assets:				
Available for sale securities	$ —	$ 14,553	$ —	$ 14,553
Liabilities:				
Series 1 Senior Preferred Unit tranche liability	$ —	$—	$ 527	$ 527

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4. Fair Value of Financial Assets and Liabilities—(Continued)

As of December 31, 2015 and 2016 and September 30, 2017, the fair values of the Company's available-for-sale securities, which consisted of US government agency securities and corporate bond securities were determined using Level 2 inputs. During the years ended December 31, 2015 and 2016 and the nine months ended September 30, 2016 and 2017, there were no transfers between Level 1, Level 2 and Level 3. A reconciliation of the liabilities measured at fair value using Level 3 significant unobservable inputs is included in Note 9, *Preferred Unit Tranche Rights*.

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 December 31, 2015 and 2016 and September 30, 2017, the fair value of available-for-sale securities by type of security was as follows:

	December 31, 2015			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
US government agency securities	$ 22,273	$ 2	$ (7)	$22,268
Corporate bond securities	4,529	—	(5)	4,524
	$ 26,802	$ 2	$ (12)	$26,792

	December 31, 2016			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
US government agency securities	$ 11,579	$ 11	$ —	$11,590
Corporate bond securities	18,378	21	(9)	18,390
	$ 29,957	$ 32	$ (9)	$29,980

	September 30, 2017 (unaudited)			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
US government agency securities	$ 5,699	$ —	$ (1)	$ 5,698
Corporate bond securities	8,857	—	(2)	8,855
	$ 14,556	$ —	$ (3)	$14,553

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5. Available-for-Sale Securities—(Continued)

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

	December 31, 2015		December 31, 2016	
	Amortized Cost	Fair Value	Amortized Cost	Fair Value
Due in one year or less	$ 10,044	$10,047	$ 28,732	$28,757
Due after one year through two years	16,758	16,745	1,225	1,223
Total available-for-sale securities	$ 26,802	$26,792	$ 29,957	$29,980

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

	September 30, 2017	
	Amortized Cost	Fair Value
	(unaudited)	
Due in one year or less	$ 14,556	$14,553
Total available-for-sale securities	$ 14,556	$14,553

The average maturity of the Company's available-for-sale securities as of December 31, 2015 and 2016 and September 30, 2017 was approximately one year, 0.5 years and 0.3 years, respectively.

6. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

	December 31,		
	2015	2016	September 30, 2017 (unaudited)
Prepaid research and development expenses	$221	$2,079	$ 639
Prepaid expenses and other assets	88	235	543
	$309	$2,314	$ 1,182

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7. Property and Equipment

Property and equipment consists of the following:

	December 31, 2015	December 31, 2016	September 30, 2017 (unaudited)
Furniture and fixtures	$—	$ 61	$ 61
Laboratory equipment	—	195	2,313
Leasehold improvements	—	68	68
Computer equipment	—	68	77
Computer software	—	—	23
Construction in process	—	116	11
	—	508	2,553
Less accumulated depreciation	—	56	281
	$—	$452	$ 2,272

Depreciation expense was $56, $22 and $225 for the year ended December 31, 2016 and for the nine months ended September 30, 2016 and 2017, respectively. There was no depreciation for the year ended December 31, 2015.

8. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following:

	December 31, 2015	December 31, 2016	September 30, 2017 (unaudited)
Accrued research and development	$ 892	$ 1,953	$ 1,469
Accrued compensation	260	1,167	1,373
Deferred funding from charitable organizations	—	345	313
Accrued other	160	424	1,766
	$ 1,312	$ 3,889	$ 4,921

9. Preferred Unit Tranche Rights

Included in the terms of the Redeemable Preferred Unit Purchase Agreement and the Series 1 Senior Preferred Unit Agreement were Tranche Rights which obligate the investors to purchase additional preferred units under certain conditions. The Tranche Rights also provide the investors with the right to purchase these additional units. The Company concluded that the Tranche Rights met the definition of a freestanding financial instrument as the Tranche Rights were legally detachable and separately exercisable from the Redeemable Preferred Units and the Series 1 Senior Preferred Units. Therefore, the Company allocated the net proceeds to each Tranche Right and the Redeemable Preferred Units or the Series 1 Senior Preferred Units based on the fair value at the date of issuance with the remaining proceeds being allocated to the Redeemable Preferred Units or Series 1 Senior Preferred Units.

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9. Preferred Unit Tranche Rights—(Continued)

For the year ended December 31, 2015 and through the final settlement date in October 2016, the Company estimated the fair value of the Redeemable Preferred Unit Tranche Right based on the probability of closing the tranches and the estimated future value of the Redeemable Preferred Units. The Redeemable Preferred Unit Tranche Right was recorded as a liability as the purchase price of the additional Redeemable Preferred Units is less than the estimated fair value of the Redeemable Preferred Units at the expected settlement date. Upon settlement, the Redeemable Preferred Unit Tranche Right is reclassified to Redeemable Preferred Units. In October 2016, the Redeemable Preferred Unit Tranche Right was settled and no Redeemable Preferred Unit Tranche Right was outstanding subsequent to October 2016.

The estimated fair value of the Series 1 Senior Preferred Unit Tranche Right was determined using a probability-weighted present value model that considered the probability of closing the tranche through achievement of the preclinical milestones, estimated to be 50% on the date of issue and 60% at September 30, 2017, and the estimated future value of Series 1 Senior Preferred Units at closing. The Company converted future values to present value using a discount rate appropriate for probability adjusted cash flows. The estimates are based, in part, on subjective assumptions. Changes to these assumptions can have a significant impact on the fair value of the Series 1 Senior Preferred Unit Tranche Right. The Series 1 Senior Preferred Unit Tranche Right is outstanding as of September 30, 2017.

A roll-forward of the tranche right is as follows:

	Redeemable Preferred Unit Tranche Right	Series 1 Senior Preferred Unit Tranche Right
Balance at December 31, 2014	$ 35,909	$ —
Change in fair value	103	—
Reclassification to preferred units	(24,008)	—
Balance at December 31, 2015	12,004	—
Change in fair value	(1,163)	—
Reclassification to preferred units	(10,841)	—
Balance at December 31, 2016	—	—
Issuance	—	459
Change in fair value	—	68
Balance at September 30, 2017 (unaudited)	$ —	$ 527

10. Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units

Redeemable Preferred Units

The Company has issued redeemable preferred units (“Redeemable Preferred Units”). The Redeemable Preferred Units are classified outside of members’ deficit because the units contain redemption features that are not solely within the control of the Company.

In December 2013, the Company issued 3,420,000 Redeemable Preferred Units at an issuance price of $1.00 per unit for proceeds of $3,420.

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10. Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units—(Continued)

In December 2014, the Company issued 3,420,000 Redeemable Preferred Units at an issuance price of $1.00 per unit for proceeds of $3,420.

In October 2015, the Company issued 6,840,000 Redeemable Preferred Units at an issuance price of $1.00 per unit for proceeds of $6,840.

In November and December 2016, the Company issued an aggregate of 3,420,000 Redeemable Preferred Units at $1.00 per unit for proceeds of $3,420.

On March 29, 2017, the Redeemable Preferred Units were exchanged to Series A Common Units. See Note 3, *Merger and Recapitalization*, for additional information. The Redeemable Preferred Units, which are carried at fair value due to their fair value redemption feature, were remeasured for a final time to their redemption value on March 29, 2017 and then were reclassified to members' deficit.

Redeemable Preferred Units consisted of the following:

	Authorized	Issued and Outstanding	Carrying Value	Liquidation Preference
At December 31, 2015	60,000,000	13,680,000	$61,697	$ 61,697
At December 31, 2016	60,000,000	17,100,000	$71,649	$ 55,746
At September 30, 2017 (unaudited)	—	—	—	—

The holders of the Redeemable Preferred Units had the following rights and preferences:

Tranche Right

The Redeemable Preferred Unit Tranche Right obligates the holders to purchase, and provides the holders with the right to purchase, additional Redeemable Preferred Units, under certain circumstances. The Redeemable Preferred unitholders purchased these additional units in 2015 and 2016. In October 2016, the Redeemable Preferred Unit Tranche Right was settled with the closing of the Redeemable Preferred Unit financing. See Note 9, *Preferred Unit Tranche Rights*, for additional information.

Redemption

The Redeemable Preferred Units were redeemable on or after December 27, 2022 at the option of the Redeemable Preferred unitholder. The Redeemable Preferred Units were redeemable at the fair market value on the redemption date.

Conversion

The Redeemable Preferred Units had no conversion rights.

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10. Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units—(Continued)

Voting Rights

The holders of Redeemable Preferred Units are entitled to vote as a single class with the holders of the Series A Common Units on certain matters, including the election of managers, with each Redeemable Preferred Unit and Series A Common Unit carrying one vote per unit.

Distributions

The Company's Board of Managers has authority to determine the amount, if any, of proceeds available for distribution to the unitholders. Prior to the conversion of the Redeemable Preferred Units on March 29, 2017, such proceeds were to be distributed in accordance with the following order of priority:

- First, to the holders of Redeemable Preferred Units, pro rata in proportion to the remaining amount to be distributed to each such holder, until each such holder has received distributions in an amount equal to the cumulative capital contributions since inception in respect of the Redeemable Preferred Units.
- Thereafter, to all Redeemable Preferred Unitholders, Series A Common Units held by the Company's founders, Series A Common Units issued to non-founders between December 27, 2013 and December 26, 2014, and vested Series A Restricted Common Unitholders issued subsequent to December 26, 2014 pro rata in proportion to their percentage interest at the time of distribution.

No distributions were made in 2015 or 2016 or during the nine months ended September 30, 2017.

Liquidation

In the event of any liquidation, dissolution, or winding-up of the Company, the assets of the Company will be distributed in accordance with the same order of priority as distributions.

Series 1 Senior Preferred Units

On March 29, 2017, the Company issued 2,500,000 Series 1 Senior Preferred Units at an issuance price of $10.00 per unit for proceeds of $25,000. See Note 3, *Merger and Recapitalization*, for additional information.

Series 1 Senior Preferred Units consist of the following:

	Authorized	Issued and Outstanding	Carrying Value	Liquidation Preference	Common Units Issuable Upon Conversion
At September 30, 2017 (unaudited)	2,500,000	2,500,000	$25,000	$ 25,000	2,500,000

Junior Preferred Units

On March 29, 2017, 134,920 Solid GT Class D Units were exchanged for 4,414,356 Junior Preferred Units of the Company. See Note 3, *Merger and Recapitalization*, for additional information.

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10. Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units—(Continued)

Junior Preferred Units consisted of the following:

	Authorized	Issued and Outstanding	Carrying Value	Liquidation Preference
At September 30, 2017 (unaudited)	4,414,356	4,414,356	$44,177	$ 42,500

The holders of the Series 1 Senior Preferred Units and Junior Preferred Units have the following rights and preferences:

Tranche Right

The holders of Series 1 Senior Preferred Units are obligated to purchase 1,973,430 Series 2 Senior Preferred Units at $12.67 per unit for gross proceeds of $25,000 in the event the Company achieves certain pre-clinical milestones. In addition, the holders of a majority of the Series 1 Senior Preferred Units have the right to require the holders of the Series 1 Senior Preferred Units to purchase the Series 2 Senior Preferred Units at any time prior to September 1, 2017, which in August 2017, was extended to December 1, 2017. The Series 1 Tranche Right is subject to certain transfer rights. See Note 9, *Preferred Unit Tranche Rights*, for additional information.

On October 26, 2017, the Series 1 Tranche Right was settled. See Note 18, *Subsequent Events*, for additional information.

The holders of the Junior Preferred Units do not have any tranche rights.

Redemption

The Series 1 Senior Preferred Units are redeemable on or after March 29, 2022 at the option of the holder at a redemption price equal to the original purchase price of $10.00 per unit plus any declared but unpaid distributions. The Company has presented Series 1 Senior Preferred Units outside of permanent equity since the redemption of Series 1 Senior Preferred Units is outside the control of the Company.

The consent of the Junior Preferred unitholders along with Series 1 Senior Preferred unitholders can effect a deemed liquidation event. Therefore, the Company has presented the Junior Preferred Units outside of permanent equity.

Voting Rights

The holders of the Series 1 Senior Preferred Units and Junior Preferred Units are entitled to vote together, and not as separate classes, with each Series 1 Senior Preferred Unit, Junior Preferred Unit, Series A Common Unit and Series B Common Unit carrying one vote per unit.

Subject to maintaining certain ownership levels, the Series 1 Senior Preferred unitholders as a class are entitled to elect one of the eight board members while such units are outstanding. The Junior Preferred unitholders as a class are entitled to elect two of the eight board members while such units are outstanding.

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10. Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units—(Continued)

Dividends

The holders of Series 1 Senior Preferred Units are entitled to an 8% annual dividend based on the Series 1 Senior Preferred Unit issuance price of $10.00 per unit, when and if declared by the Board of Managers. No dividends were declared or paid to Series 1 Senior Preferred unitholders.

The holders of the Junior Preferred Units are entitled to an 8% annual dividend based on the Junior Preferred Unit issuance price of $9.63 per unit, when and if declared by the Board of Managers. No dividends were declared or paid to Junior Preferred unitholders.

Distributions

The Company's Board of Managers has authority to determine the amount, if any, of proceeds available for distribution. Such proceeds are to be distributed in accordance with the following order of priority:

- First, the Series 1 Senior Preferred and the Junior Preferred unitholders are entitled to an amount distributed, on a pro rata basis, equal to the Series 1 Senior Preferred Unit price of $10.00 per unit and any declared but unpaid Series 1 Senior Preferred dividends and the Junior Preferred Unit price of $9.63 per unit and any declared but unpaid Junior Preferred dividends, respectively.
- Second, the Series A, B, C and D Common unitholders are entitled to an amount distributed, on a pro rata basis, subject to certain limitations, until the cumulative amount distributed with respect to one Series A Common Unit, Series B Common Unit, Series C Common Unit and vested Series D Common Unit equals the cumulative amount distributed to one Junior Preferred Unit.
- Third, the Junior Preferred unitholders and the Series A, B, C and vested D Common unitholders are entitled to an amount distributed on a pro rata basis, subject to certain limitations, until the cumulative amount distributed with respect to one Junior Preferred Unit, Series A Common Unit, Series B Common Unit, Series C Common Unit and vested Series D Common Unit equals the cumulative amount distributed to one Series 1 Senior Preferred Unit.
- Fourth, the Junior Preferred and the Series A, B, C and vested D Common unitholders are entitled to participate on a pro rata basis in cumulative distributions, subject to certain limitations, in the remaining proceeds available for distribution.

As a result of the issuance of the Series 2 Senior Preferred Units on October 26, 2017, the Series 2 Senior Preferred unitholders are entitled to cumulative amounts distributed equal to the amount paid per unit for the Series 2 Senior Preferred Units and any declared but unpaid Series 2 Senior Preferred cumulative dividends, prior to and with priority over any distributions to any other unitholders. In addition, upon the issuance of the Senior Series 2 Preferred units, the holders of the Junior Preferred Units no longer share pro rata in the order of distributions with the Senior Series 1 Preferred unitholders and are subordinate to distributions made to Series 1 Senior Preferred unitholders.

No distributions were made during the nine months ended September 30, 2017.

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10. Redeemable Preferred Units, Series 1 Senior Preferred Units and Junior Preferred Units—(Continued)

Liquidation

In the event of any liquidation, dissolution, or winding-up of the Company, the assets of the Company will be distributed in accordance with the same order of priority that applies to distributions.

Conversion

The holders of the Series 1 Senior Preferred Units have the right to convert their units into Series C Common units on a one-to-one basis prior to March 29, 2022.

Upon the closing of a qualified public offering on specific terms, or upon the request of the holders of a majority of each of the outstanding Series 1 Senior Preferred Units and Junior Preferred Units, the Company's outstanding preferred units and common units will automatically convert into common shares.

11. Members' Deficit

Series A, B, C and D Common Units

Series A, B, C and D Common Units consisted of the following:

	December 31, 2015		
	Authorized	Issued and Outstanding	Carrying Value
Series A Common Units	20,000,000	5,015,917	$ 208

	December 31, 2016		
	Authorized	Issued and Outstanding	Carrying Value
Series A Common Units	20,000,000	5,123,917	$ 558

	September 30, 2017 (unaudited)		
	Authorized	Issued and Outstanding	Carrying Value
Series A Common Units	12,219,299	12,219,299	$ 55,964
Series B Common Units	3,258,480	3,258,480	3,312
Series C Common Units	1,635,916	1,635,916	2,053
Series D Common Units	3,075,814	2,127,308	2,862
	20,189,509	19,241,003	$ 64,191

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11. Members' Deficit—(Continued)

Series A Common Units

Founders Series A Common Units

On December 27, 2013, the Company issued 4,560,000 restricted Series A Common Units to its founders with time-based vesting conditions. Unvested units of Series A Common Units may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award. At December 31, 2015 and 2016, 2,280,000 and 3,420,000 restricted Series A Common Units were vested. The aggregated intrinsic value of the restricted Series A Common Units that vested during the year ended December 31, 2016 was $3,306. There were no restricted Series A Common Units that vested during the nine months ended September 30, 2017.

On March 29, 2017, in connection with the Merger and Recapitalization, the 4,560,000 founders' restricted Series A Common Units were exchanged for 3,258,480 restricted Series B Common units. All restricted Series B Common Units will continue to vest pursuant to the original vesting terms under the restricted Series A Common Units agreements and the Company will continue to recognize compensation expense over the related service period.

In addition, in connection with the exchange of the founders' restricted Series A Common Units into restricted Series B Common Units, the Company recognized $2,710 of equity based compensation expense for vested units, which represents the incremental fair value of the units before and after the Merger and Recapitalization. The Company will record additional compensation expense in the amount of $904 over the remaining vesting period of the Series B Common units of which $602 has been recognized as of September 30, 2017.

Non-Founder Series A Common Units

In March and November 2014, the Company issued 169,667 restricted Series A Common Units at a per unit value of $2.59 to certain employees and consultants.

In September and November 2015, the Company issued 305,000 restricted Series A Common Units at a per unit values between $2.39 and of $2.65 to certain employees.

In May and September 2016, the Company issued 60,000 restricted Series A Common Units at a per unit values between $2.03 and $2.14 to certain employees.

In December 2016, the Company issued 48,000 restricted Series A Common Units at a per unit value of $2.25 to certain employees.

On March 29, 2017, in connection with the Merger and Recapitalization, 563,917 non-founder restricted Series A Common Units were exchanged for 402,963 restricted Series D Common Units. All restricted Series D Common Units will continue to vest pursuant to their original vesting period, which was generally four years, under the restricted Series A Common Units agreement, and the Company will continue to recognize compensation expense over the related service period.

In addition, in connection with the exchange of the non-founders' restricted Series A Common Units into restricted Series D Common Units, the Company recognized $140 of equity-based compensation expense for

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11. Members' Deficit—(Continued)

vested units, which represents the incremental fair value of the units before and after the Merger and Recapitalization. The Company will record additional compensation expense in the amount of $115 over the remaining vesting period of the Series D Common units of which $32 has been recognized as of September 30, 2017.

The holders of the Series A, B, C and D Common Units are entitled to the following rights and priorities:

Voting Rights

Holders of Series A and B Common Units have the right to one vote per unit held by such member. The Series A Common unitholders as a class are entitled to elect two of the eight board members while such units are outstanding. The Series B Common unitholders as a class are entitled to elect three of the eight board members while such units are outstanding

Holders of Series C and D Common Units do not have the right to vote for the election of board members.

Redemption

The Series A, B, C and D Common Units are not redeemable.

Distributions and Liquidation Preference

The holders of the Series A, B, C and D Common Units are entitled to participate in distributions after preferential distributions are made to the Series 1 Senior Preferred and Junior Preferred unitholders as follows:

- The Series A, B, C and D Common unitholders are entitled to participate in distributions on a pro rata basis, subject to certain limitations, until the cumulative amount distributed with respect to one Series A Common Unit, Series B Common Unit, Series C Common Unit and vested Series D Common Unit equals the cumulative amount distributed to one Junior Preferred Unit.
- The Junior Preferred unitholders and the Series A, B, C and D Common unitholders are entitled to participate in distributions on a pro rata basis, subject to certain limitations, until the cumulative amount distributed with respect to one Junior Preferred Unit, Series A Common Unit, Series B Common Unit, Series C Common Unit and vested Series D Common Unit equals the cumulative amount distributed to one Series 1 Senior Preferred Unit.
- All unitholders are entitled to participate on a pro rata basis in cumulative distributions, subject to certain limitations, in the remaining proceeds available for distribution.

No distributions were made to the Series A, B, C or D Common unitholders during the years ended December 31, 2015 and 2016 and during the nine months ended September 30, 2017.

12. Equity-Based Compensation

The Company adopted the Solid Ventures, LLC Equity Incentive Plan (the "Plan") on January 1, 2015, which provided for the issuances of up to 1,140,000 Series A Common Units under the Plan. The Company has granted

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12. Equity-Based Compensation—(Continued)

Series A Common Units with time-based vesting conditions. Unvested Series A Common Units may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award. As of December 31, 2016, 576,083 units were available for future grants under the Plan.

On March 29, 2017, the Company amended the Solid Ventures, LLC Equity Incentive Plan and changed the name of the Plan to the Solid Biosciences, LLC Amended and Restated Equity Incentive Plan (the "Amended Plan") and increased the number of Series D Common Units available for issuance under the Amended Plan from 1,140,000 to 2,971,949 units.

As of September 30, 2017, 844,640 Series D Common Units were available for future grants under the Amended Plan.

The following table summarizes the Company's restricted Series A Common Unit activity since December 31, 2015:

	Units	Weighted-Average Grant Date Fair Value
Unvested restricted Series A Common Units at December 31, 2015	413,500	$ 2.53
Issued	108,000	2.17
Vested	(115,987)	2.53
Unvested restricted Series A Common Units at December 31, 2016	405,513	2.43
Vested	(44,377)	2.49
Unvested restricted Series A Common Units at March 29, 2017 (unaudited)	361,136	2.43
Exchange of unvested restricted Series A Common Units to restricted Series D Common Units at March 29, 2017 (unaudited)	258,060	3.39
Issuance of unvested restricted Series D Common units	50,000	3.08
Unvested restricted Series D Common Units at March 31, 2017 (unaudited)	308,060	$ 3.34

The following table summarizes the Company's restricted Series D Common Unit activity since March 31, 2017. The opening balance includes the exchange of the Company's restricted Series A Common Units and the exchange of Solid GT's Class C Restricted Common Units to restricted Series D Common Units as a result of the merger and recapitalization described in Note 3, Merger and Recapitalization.

	Units	Weighted-Average Grant Date Fair Value
Unvested restricted Series D Common Units at March 31, 2017 (unaudited)	819,545	$ 3.34
Issuance of unvested restricted Series D Common units	643,276	3.68
Vested	(131,959)	3.36
Forfeited	(52,136)	3.23
Unvested restricted Series D Common Units at September 30, 2017 (unaudited)	1,278,726	$ 3.51

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12. Equity-Based Compensation—(Continued)

The aggregate intrinsic value of restricted Series A Common Units that vested during the year ended December 31, 2016 was $16. The aggregate intrinsic value of restricted Series D Common units that vested during the nine months ended September 30, 2017 was $911.

At December 31, 2016, there was $864 of unrecognized equity-based compensation related to unvested Series A Common Units, which is expected to be recognized over a weighted average period of 2.8 years. At September 30, 2017, there was $4,498 of unrecognized equity-based compensation related to Series D Common Units, which is expected to be recognized over a weighted average period of 3.0 years.

The Company's Board of Managers approved the issuance of up to 185,781 Series D Common Units to employees upon the achievement of certain events. If those events occur, the Series D Common Units will be issued and vest in accordance with their time-based vesting conditions, which is generally four years. The Company issued 67,891 of the 185,781 Series D Common Units in September 2017 when the Company submitted its initial IND to the FDA. These 67,891 units are included within the 643,276 units included within the table above.

The Solid GT LLC Agreement provides for the issuance of up to 55,555 Class C Restricted Common Units. The Company has granted Class C Restricted Common Units with time-based vesting conditions. Unvested Class C Restricted Common Units may not be sold or transferred by the holder. These restrictions lapse according to the time-based vesting conditions of each award. As of December 31, 2016, 22,073 units were available for future grants.

On March 29, 2017, the Solid GT LLC Equity Incentive Plan was terminated and all Class C Restricted Common Units were exchanged for Series D Common Units of the Company with no change in vesting conditions. No further Class C Restricted Common Unit activity has occurred subsequent to March 29, 2017.

The following table summarizes the Solid GT Class C Restricted Common Unit activity since December 31, 2015:

	Units	Weighted-Average Grant Date Fair Value
Unvested Class C Restricted Units at December 31, 2015	17,245	$ 92.86
Issued	12,830	110.30
Vested	(9,081)	86.01
Forfeited	(1,597)	97.24
Unvested Class C Restricted Units at December 31, 2016	19,397	107.24
Vested	(3,764)	100.20
Unvested Class C Restricted Units at March 29, 2017 (unaudited)	15,633	$ 108.94
Exchange of Unvested Class C Restricted Units into Series D Common Units of the Company at March 29, 2017 (unaudited)	511,485	$ 3.34
Unvested Restricted Series D Common Units at March 31, 2017 (unaudited)	511,485	$ 3.34

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12. Equity-Based Compensation—(Continued)

The aggregate intrinsic value of Solid GT Class C Common Units that vested during the years ended December 31, 2016 was $335. The aggregate intrinsic value of restricted Class C Restricted Common Units that vested during the nine months ended September 30, 2017 was $58.

At December 31, 2016, there was $2,853 of unrecognized equity-based compensation, which is expected to be recognized over a weighted average period of 2.6 years.

The Company recorded equity-based compensation expense related to the Company's restricted Series A Common Units, restricted Series D Common Units and Solid GT Class C Common Units, in the following expense categories of its consolidated statements of operations:

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Research and development expenses	$749	$1,262	$ 959	$ 784
General and administrative expenses	15	208	163	3,723
	$764	$1,470	$ 1,122	$ 4,507

13. Commitments and Contingencies

Operating Lease

The Company leases office and laboratory space under an operating lease agreement. The lease expires in January 2018 with no extension periods.

During the years ended December 31, 2015 and 2016 and the nine months ended September 30, 2016 and 2017, the Company recognized $108, $270, $204 and $791, respectively, of rental expense related to office and laboratory space.

Future minimum lease payments for this operating lease as of December 31, 2016 were as follows:

Year Ending December 31,	
2017	$ 288
2018	25
Total	$ 313

Letter of Credit

The Company has an outstanding letter of credit in the amount of $65 at December 31, 2016, which was required as a condition of the Company's office and laboratory lease.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters, including, but not limited to,

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13. Commitments and Contingencies—(Continued)

losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with members of its Board of Managers that will require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as managers. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnification arrangements.

The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2015 and 2016 and September 30, 2017.

Contingencies

In the first quarter of 2017, the Company terminated the development, manufacturing and testing agreement (the “Agreement”) it entered into in January 2016 with a third-party. The Company and the third-party are in dispute regarding the remaining amounts owned by the Company to the third-party under the Agreement. The range of possible loss is estimated to be between $600 and $1,500, and an estimated liability of $1,450 has been established for this matter in the accompanying consolidated balance sheet as of September 30, 2017.

14. License Agreements

University of Washington License Agreement

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

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones. There were no milestones achieved as of December 31, 2015 and 2016 and September 30, 2017. The Company must also pay royalties of a low single digit percentage of future sales by us and our sublicensees of products developed under the licensed patent rights. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to the Company and its sublicensees.

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. The Company may terminate the agreement at any time upon providing sixty days’ written notice to

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14. License Agreements—(Continued)

the University of Washington. The University of Washington may terminate the agreement upon the Company's uncured, material breach of the agreement or if the Company enters into an insolvency-related event.

The Company recorded research and development expense in the amount of $25 for the year ended December 31, 2015. There was no research and development expense for the year ended December 31, 2016, and $0 and $49 for the nine months ended September 30, 2016 and 2017 under the agreement.

The University of Missouri License Agreement

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

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones for each product developed based on the licensed patents. There were no milestones achieved as of December 31, 2015 and 2016 and September 30, 2017. The Company must pay a royalty of a low single digit percentage of future sales or by its sublicensees of products developed using the licensed patents. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

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

The license agreement remains in effect until the expiration of the last-to-expire patent or the abandonment of the last to be abandoned patent application licensed under the agreement. The University of Missouri may terminate the agreement, or render the license granted thereunder non-exclusive, in individual countries if the Company's sublicensees fail to achieve certain milestones. The Company may terminate the license agreement at any time upon providing six months' written notice to the University of Missouri and paying a termination fee. Each of the University of Missouri and the Company may also terminate the agreement for an uncured default or breach of the agreement by the other party. The Company's ability to cure such breach only applies to the first two notices of such breach provided by the University of Missouri, and thereafter, the University of Missouri may terminate the agreement for the Company's default or breach of the agreement upon thirty days' written notice without an opportunity to cure such default or breach.

The Company recorded research and development expense in the amount of $40 for the year ended December 31, 2015. There was no research and development expense for the year ended December 31, 2016 and $0 and $1 the nine months ended September 30, 2016 and 2017 under the agreement.

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14. License Agreements—(Continued)

The University of Michigan License Agreement

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

In consideration for the rights granted by the agreement, we paid a one-time license fee and a separate fee to cover past patent prosecution costs, which we recorded as a research and development expense in 2016. We are required to reimburse the University of Michigan for costs incurred in applying for, prosecuting and maintaining patents, and pay up to an aggregate of approximately $1 million upon the achievement of certain milestones. There were no milestones achieved as of December 31, 2016 and September 30, 2017. The Company must also pay a royalty of a low single digit percentage on future sales by us or our sublicensees of products developed using the licensed rights, with a minimum annual royalty after certain milestones are achieved. In addition, the Company must pay an annual maintenance fee in any year in which the minimum annual royalty is not reached.

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

The license agreement remains in effect until the expiration of the last-to-expire patent licensed under the agreement. The University of Michigan may terminate the agreement upon the Company’s uncured material breach of the agreement, including failure to make required payments under the agreement or to achieve certain milestones, or if the Company becomes insolvent or bankrupt. The Company may terminate the license agreement at any time upon providing sixty days’ written notice to the University of Michigan.

The Company recorded and research and development expense in the amount of $145, $145 and $4 for the year ended December 31, 2016 and the nine months ended September 30, 2016 and 2017, respectively, under the agreement.

Harvard College License Agreements

In 2016, the Company entered into a license agreement with the President and Fellows of Harvard College, or Harvard College, under which the Company obtained a non-exclusive, royalty-bearing, sublicensable, worldwide license to use certain intellectual property owned by Harvard College to develop, manufacture, and commercialize products for use in the treatment of DMD.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2016. The Company is required to pay an annual license maintenance fee until certain milestones are achieved, after which time the annual maintenance fee will increase annually. Such annual maintenance fee will further increase if the Company grants certain rights to a sublicensee or strategic partner with whom the Company collaborates on the development and

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14. License Agreements—(Continued)

commercialization of licensed products. The annual maintenance fee is creditable against royalty payments. The Company also must pay a milestone payment within thirty days after achieving certain milestones. There were no milestones achieved as of December 31, 2016 and September 30, 2017. The Company must pay a royalty of a low single digit percentage on future sales by us or our sublicensees of products developed using the licensed technology.

The license agreement remains in effect for an initial term of fifteen years, with automatic three-year renewal periods thereafter unless one of the parties provides notice of non-renewal. The Company may terminate the license agreement at any time upon providing sixty days' written notice to Harvard College. Harvard College may terminate the agreement in the event the Company becomes bankrupt or insolvent. Both Harvard College and the Company may also terminate the agreement for an uncured material breach of the agreement by the other party.

The Company recorded research and development expense in the amount of $45, $45 and $35 for the year ended December 31, 2016 and the nine months ended September 30, 2016 and 2017, respectively, under the agreement.

In August 2017, the Company entered into a license agreement with the President and Fellows of Harvard College, or Harvard College, under which the Company obtained a non-exclusive, royalty-bearing, sublicensable, worldwide license to use certain intellectual property owned by Harvard College to develop, manufacture, and commercialize products for use in the treatment of DMD.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2017. The Company is required to pay an annual license maintenance fee until certain milestones are achieved, after which time the annual maintenance fee will increase annually. Such annual maintenance fee will further increase if the Company grants certain rights to a sublicensee or strategic partner with whom the Company collaborates on the development and commercialization of licensed products. The annual maintenance fee is creditable against royalty payments. The Company also must pay a milestone payment within thirty days after achieving certain milestones. There were no milestones achieved as of September 30, 2017. The Company must pay a royalty of a low single digit percentage on future sales by us or our sublicensees of products developed using the licensed technology.

The license agreement remains in effect for an initial term of fifteen years, with automatic three-year renewal periods thereafter unless one of the parties provides notice of non-renewal. The Company may terminate the license agreement at any time upon providing sixty days' written notice to Harvard College. Harvard College may terminate the agreement in the event the Company becomes bankrupt or insolvent. Both Harvard College and the Company may also terminate the agreement for an uncured material breach of the agreement by the other party.

The Company recorded research and development expense in the amount of $18 for the nine months ended September 30, 2017 under the agreement.

Other License Agreements

In 2016, the Company entered into a license agreement with Life Technologies Corporation, or Life Technologies. In consideration for obtaining a non-exclusive, royalty-free, worldwide license to use certain

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14. License Agreements—(Continued)

technologies and associated know-how to develop product candidates, the Company paid a one-time, non-refundable license fee. This fee was recorded as a research and development expense in 2016. The license agreement will remain effective in perpetuity unless earlier terminated. Life Technologies has the right to terminate the agreement upon our material, uncured breach of the agreement or in the event that it determines that continued performance of the agreement may violate any laws. The Company is obligated to diligently pursue regulatory approval necessary for the development, manufacture and sale of the licensed products. The Company has the right to terminate the agreement at any time upon providing thirty days' written notice to Life Technologies.

15. Net Loss per Unit and Unaudited Pro Forma Net Loss per Share

Basic and diluted net loss per common unit were calculated as follows:

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

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Net loss	$ (6,664)	$ (23,773)	$(15,254)	$(38,691)
Net loss attributable to non-controlling interest	(287)	(2,234)	(1,471)	(1,060)
Net loss attributable to Solid Biosciences, LLC	$ (6,377)	$ (21,539)	$(13,783)	$(37,631)
Decretion (accretion) of preferred units to redemption value	(68)	4,309	1,198	(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 unitholders	$ (6,445)	$ (17,230)	$(12,585)	$(24,830)

The denominator is as follows:

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Weighted average common units outstanding, basic and diluted	846,569	1,698,904	1,677,909	12,446,769

Net loss per unit attributable to common unitholders, basic and diluted is as follows:

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Net loss per unit attributable to common unitholders, basic and diluted	$ (7.61)	$ (10.14)	$ (7.50)	$ (1.99)

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15. Net Loss per Unit and Unaudited Pro Forma Net Loss per Share—(Continued)

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

	Year Ended December 31,		Nine Months Ended September 30,	
	2015	2016	2016	2017
			(unaudited)	
Series A common units	1,924,718	1,104,391	1,923,170	—
Series B common units	—	—	—	814,620
Series D common units	—	—	—	1,278,726
	1,924,718	1,104,391	1,923,170	2,093,346

The unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2016 and the nine months ended September 30, 2017 have been prepared to give effect to the Company's conversion to a corporation whereby all outstanding preferred units and common units are converted into shares of common stock as if the proposed Corporate Conversion had occurred on the later of January 1, 2016 or the issuance date of the preferred and common units.

The unaudited pro forma net loss attributable to common stockholders used in the calculation of unaudited basic and diluted pro forma net loss per share attributable to common stockholders does not include the effects of the decretion/accretion of preferred units to redemption value, the revaluation of the preferred unit tranche rights or the redemption of preferred units because the calculation assumes the conversion of the preferred units into shares of common stock as if the proposed Corporate Conversion had occurred on the later of January 1, 2016 or the issuance date of the preferred units.

Upon conversion to a corporation, the Company will become subject to U.S. federal and state income taxes. Based on the Company’s history of generating operating losses and its anticipation of operating losses continuing in the foreseeable future, the Company has determined that it is more likely than not that the tax benefits from its operating losses would not be realized and has determined that a full valuation allowance against its net deferred tax assets would be recorded on a pro forma basis. Therefore, for the purposes of the pro forma tax provision, the Company has not recorded an income tax benefit for the net losses incurred by the Company during the year ended December 31, 2016 and the nine months ended September 30, 2017.

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15. Net Loss per Unit and Unaudited Pro Forma Net Loss per Share—(Continued)

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Year Ended December 31, 2016	Nine Months Ended September 30, 2017 (unaudited)
Numerator:		
Net loss attributable to common stockholders before benefit from income taxes	$ (17,230)	$ (24,830)
Add:		
Change in fair value of preferred unit tranche rights	(1,163)	68
(Decretion) accretion of preferred units to redemption value	(4,309)	959
Redemption of preferred units	—	(15,685)
Pro forma benefit from income taxes	—	—
Pro forma net loss attributable to common stockholders	$ (22,702)	$ (39,488)
Denominator:		
Weighted average common shares outstanding—basic and diluted	1,698,904	12,446,769
Pro forma adjustment to reflect the assumed conversion	12,354,013	6,786,378
Pro forma weighted average common shares outstanding—basic and diluted	14,052,917	19,233,147
Pro forma net loss per share attributable to common stockholders—basic and diluted	$ (1.62)	$ (2.05)

16. Retirement Plan

The Company has a defined contribution savings plan under Section 401(k) of the Internal Revenue Code. The plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis. Company contributions to the plan may be made at the discretion of the Company's board of managers. The Company made no contributions to the plan during the years ended December 31, 2015 and 2016 or the nine months ended September 30, 2016 and 2017.

17. Subsequent Events

For its consolidated financial statements as of December 31, 2016 and for the year then ended, the Company evaluated subsequent events through August 4, 2017, the date on which those financial statements were available to be issued.

On May 31, 2017, the Company granted 394,336 restricted Series D Common units to employees and consultants, with restrictions which generally lapse over four years.

18. Subsequent Events (unaudited)

For its unaudited consolidated financial statements as of September 30, 2017 and for the nine months then ended, the Company evaluated subsequent events through December 29, 2017, the date on which those financial statements were available to be issued.

SOLID BIOSCIENCES, LLC
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(Information as of September 30, 2017 and for the nine months ended September 30, 2016 and 2017 is unaudited)
(Amounts in thousands, except unit and per unit data)

18. Subsequent Events (unaudited)—(Continued)

On October 26, 2017, the LLC Agreement was amended to increase the number of authorized Series 2 Senior Preferred Units to 4,886,000 and the Company sold 4,886,000 Series 2 Senior Preferred Units at a price of $11.26 per unit in exchange for net proceeds of $55,000. The Series 1 Tranche Right was settled in connection with the closing of the Series 2 Senior Preferred Unit financing.

Events Subsequent to the Original Issuance of Financial Statements (Unaudited)

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 $3.8 million. In addition to rent, the lease requires us to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

Until February 19, 2018 (the 25th day after the date of this prospectus), all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the obligations of dealers to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.