

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549**

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2020

OR

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

For the transition period from _____ to _____

Commission File Number 001-38360

Solid Biosciences Inc.

(Exact name of Registrant as specified in its Charter)

Delaware
(State or other jurisdiction of
incorporation or organization)
141 Portland Street, Fifth Floor
Cambridge, MA
(Address of principal executive offices)

90-0943402
(I.R.S. Employer
Identification No.)

02139
(Zip Code)

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

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

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

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

None
(Title of class)

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. YES NO

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. YES NO

Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the Registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. YES NO

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the Registrant was required to submit such files). YES NO

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

| | | | |
|-------------------------|-------------------------------------|---------------------------|-------------------------------------|
| Large accelerated filer | <input type="checkbox"/> | Accelerated filer | <input type="checkbox"/> |
| Non-accelerated filer | <input checked="" type="checkbox"/> | Smaller reporting company | <input checked="" type="checkbox"/> |
| Emerging growth company | <input checked="" type="checkbox"/> | | |

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report.

Indicate by check mark whether the Registrant is a shell company (as defined in Rule 12b-2 of the Act). YES NO

As of June 30, 2020, the last business day of the Registrant's most recently completed second fiscal quarter, the aggregate market value of the Registrant's common stock held by non-affiliates was \$72.8 million, based on the last reported sale price of such stock on the Nasdaq Global Select Market as of such date.

The number of shares of Registrant's common stock outstanding as of February 16, 2021 was 85,130,417.

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CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K includes forward-looking statements, which involve risks and uncertainties. These forward-looking statements can be identified by the use of forward-looking terminology, including the terms “believe,” “estimate,” “project,” “anticipate,” “expect,” “seek,” “predict,” “aim,” “continue,” “possible,” “intend,” “may,” “might,” “will,” “could,” “would” or “should” or, in each case, their negative, or other variations or comparable terminology. These forward-looking statements include all matters that are not historical facts. They appear in a number of places throughout this Annual Report on Form 10-K. We derive many of our forward-looking statements from our operating budgets and forecasts, which are based upon many detailed assumptions. While we believe that our assumptions are reasonable, we caution that it is very difficult to predict the impact of known factors, and, of course, it is impossible for us to anticipate all factors that could affect our actual results. All forward-looking statements are based upon information available to us on the date of this Annual Report on Form 10-K.

The forward-looking statements in this Annual Report on Form 10-K include, among other things, statements about:

- the timing, progress and results of preclinical studies and clinical trials for SGT-001 and our other product candidates;
- our ability to establish or maintain collaborations or strategic relationships, including our collaboration with Ultragenyx Pharmaceutical Inc.;
- our ability to obtain and maintain U.S. regulatory approval of SGT-001, and the timing and scope thereof;
- our ability to obtain and maintain foreign regulatory approvals, and the timing and the scope thereof;
- the size of the patient populations and potential market opportunity 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 plans to develop and 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 expectations related to our use of capital resources;
- our estimates regarding expenses, ongoing losses, future revenue, capital requirements and need for and ability to obtain additional financing;
- our intellectual property position;
- our competitive and market position;
- developments relating to our competitors and our industry;
- the impact of the COVID-19 pandemic on our business and operations and our future financial results;
- our ability to continue as a going concern; 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 Annual Report on Form 10-K. In addition, even if our results of operations, financial condition, business and prospects are consistent with the forward-looking statements contained in this Annual Report on Form 10-K, those results may not be indicative of results in subsequent periods.

You should read this Annual Report on Form 10-K 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.

As used in this Annual Report on Form 10-K, the terms “Solid,” “the Company,” “we,” “us” and “our” refer to Solid Biosciences Inc. unless the context indicates otherwise.

RISK FACTOR SUMMARY

Our business is subject to a number of risks that if realized could materially affect our business, operating results and financial condition and the trading price of our common stock could decline. These risks are discussed more fully in the “Risk Factors” section of this Annual Report on Form 10-K. These risks include the following:

- We have incurred significant net losses since inception and anticipate that we will continue to incur net losses for the foreseeable future and may never achieve or maintain profitability.
- We will need additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.
- Our independent registered public accounting firm has included an explanatory paragraph relating to our ability to continue as a going concern in its report on our audited financial statements included in this Annual Report on Form 10-K.
- We have never generated revenue from product sales and do not expect to do so for the next several years, if ever.
- The ongoing COVID-19 pandemic may affect our ability to initiate, resume and complete current or future preclinical studies or clinical trials, disrupt regulatory activities or have other adverse effects on our business and operations.
- In November 2019, the FDA placed IGNITE DMD, our Phase I/II clinical trial of SGT-001, on clinical hold after we reported a serious adverse event in the clinical trial. Even though the clinical hold was lifted in October 2020, we cannot guarantee that similar events will not happen in the future.
- SGT-001 is a gene transfer candidate based on a novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval.
- Our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.
- Success in preclinical studies may not be indicative of results obtained in later trials.
- Preliminary or interim data that we announce or publish from time to time may change as more data becomes available and are subject to audit and verification procedures that could result in material changes in final data.
- We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.
- 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 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 may establish our own SGT-001 manufacturing facility, we expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.
- 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.

PART I

Item 1. Business.

Overview

Our mission is to cure Duchenne muscular dystrophy (“Duchenne”), a genetic muscle-wasting disease predominantly affecting boys, with symptoms that usually manifest between three and five years of age. Duchenne is a progressive, irreversible and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated prevalence of 10,000 to 15,000 cases in the United States alone. Duchenne is caused by mutations in the dystrophin gene, which result in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Without functioning dystrophin and certain associated proteins, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. There is no cure for Duchenne and, for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments. Our efforts are focused on our lead product candidate, SGT-001, a gene transfer candidate under investigation for its ability to drive functional dystrophin protein expression in patients’ muscles and improve the course of the disease. Based on our preclinical program that included multiple animal species of different phenotypes and genetic variations, as well as preliminary clinical trial biomarker results, we believe that SGT-001, has the potential to slow or even halt the progression of Duchenne, regardless of the type of genetic mutation or stage of the disease.

For patients suffering from Duchenne, 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 Duchenne 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, Duchenne 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 Duchenne 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 Duchenne. Through this disease-focused business model, our research team, led by experts in Duchenne biology and drug development, along with key opinion leaders in Duchenne, 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 Duchenne, and thus initiated development of our lead product candidate SGT-001.

Our product candidates

SGT-001 is our lead gene transfer candidate and the focus of our research and development efforts. 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 Duchenne by delivering a synthetic transgene that produces dystrophin-like protein that is only expressed in muscles of the body, including cardiac and respiratory muscles. Our SGT-001 vector is derived from a naturally occurring, non-pathogenic virus called adeno-associated virus, or AAV, which was selected for its ability to efficiently enter skeletal, diaphragm and cardiac muscle tissues. The vector is designed to carry a synthetic dystrophin transgene construct, called microdystrophin, that retains the most critical components of the full-size dystrophin gene yet is small enough to fit within AAV packaging constraints. These components not only include the domains necessary to confer protection against muscle damage, but also to drive the expression of key dystrophin associated proteins, such as neuronal Nitric Oxide Synthase, or nNOS. Inclusion of this nNOS coding region of the dystrophin protein may result in microdystrophin protein that has unique activity, restore nitric oxide production in the muscle and potentially provide important functional benefits such as diminished muscle fatigue and protection against ischemic muscle damage. In our Investigational New Drug Application-, or IND, enabling preclinical program, we have studied the efficacy, safety and durability of SGT-001 in multiple preclinical models and its functional benefits in Duchenne 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.

SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, and Fast Track Designation, in the United States and Orphan Drug Designations in both the United States and European Union. The safety and efficacy of SGT-001 are under evaluation in a randomized, controlled, open-label, single-ascending dose Phase I/II clinical trial called IGNITE DMD.

In the fourth quarter of 2017, we initiated IGNITE DMD to evaluate SGT-001 in ambulatory and non-ambulatory males with Duchenne aged four to 17 years. The primary objectives of IGNITE DMD are to assess the safety and tolerability of SGT-001, as well as efficacy as defined by SGT-001 microdystrophin protein expression. The clinical trial is also designed to assess other parameters of muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with SGT-001 microdystrophin production, SGT-001 microdystrophin associated biochemical properties (e.g. nNOS binding) and patient and parent reported outcomes and quality of life measures, among other endpoints. In February 2019, we announced preliminary findings based on three-month biopsy data from the first three patients dosed with 5E13 vg/kg of SGT-001, the lowest dose outlined in the trial protocol. In one patient, SGT-001 microdystrophin was detected via western blot below the five percent level of quantification of the assay and in approximately 10 percent of fibers via immunofluorescence. Due to these findings, and in consultation with the Data Safety Monitoring Board, or DSMB, in May 2019 we announced that we had initiated dosing of the next cohort of patients at 2E14 vg/kg. In August 2019, we amended our protocol to remove the matched patient control arm for the rest of the 2E14 vg/kg cohort in the IGNITE DMD trial and to provide measures to potentially improve safety, such as initially proceeding to dose patients weighing 25 kg or less.

In November 2019, IGNITE DMD was placed on clinical hold by the U.S. Food and Drug Administration, or FDA, as a result of a serious adverse event, or SAE, experienced by the third patient dosed in the 2E14 vg/kg cohort. The SAE, deemed related to the study drug, was characterized by complement activation, thrombocytopenia, a decrease in red blood cell count, acute kidney injury, and cardio-pulmonary insufficiency. Neither cytokine- nor coagulopathy-related abnormalities were observed. In December 2019, we reported that the SAE was fully resolved and the patient had resumed his normal activities.

In December 2019, we announced preliminary findings based on three-month biopsy data from the first two patients dosed with 2E14 vg/kg of SGT-001. Using two independent immunofluorescence assays, 10% to 20% of microdystrophin positive muscle fibers were determined to express SGT-001 microdystrophin in the fourth patient and 50% to 70% of microdystrophin positive muscle fibers in the fifth patient. Immunofluorescence also showed clear stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin in both patients. Using western blot, the expression levels for the fourth patient were detectable and estimated to be near the assay's level of quantification which is 5% of non-dystrophic control samples, with one assay replicate at 5.5%. Expression for the fifth patient was 17.5% of normal control samples. The levels of serum creatine kinase, a highly variable biochemical marker of muscle damage, declined from baseline in both patients.

In March 2020, we announced data from the third patient dosed in the 2E14 vg/kg dose cohort of IGNITE DMD, including three-month biopsy data. Using immunofluorescence assays, 50% to 70% of the muscle fibers were determined to express SGT-001 microdystrophin. Immunofluorescence also showed stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin. Using western blot, microdystrophin expression was 8% of normal control samples. In addition, the level of serum creatine kinase decreased from baseline.

In April 2020, we submitted a response to the FDA that included changes to the clinical protocol designed to enhance patient safety, as well as information related to improvements to our manufacturing process. The FDA responded by maintaining the clinical hold and requesting further data and analyses relating to this manufacturing process. In June 2020, we submitted a response to the FDA that provided data related to manufacturing process improvements. In July 2020, we announced that the FDA responded by maintaining the clinical hold and requesting further manufacturing information and updated safety and efficacy data for all patients dosed in the trial, as well as providing direction on the total viral load to be administered per patient. In October 2020, we announced that the FDA lifted the clinical hold placed on our IGNITE DMD Phase I/II clinical trial. In connection with the lifting of the clinical hold, we are reducing the maximum weight of the next two patients dosed in IGNITE DMD to 18 kg per patient, with safety outcomes from these two patients driving potential weight increase of patients dosed subsequently. This reduction, in conjunction with the delivery of fewer viral particles as a result of our manufacturing process improvements, will reduce patients' total viral load while continuing dosing at the 2E14 vg/kg dose. Additionally, to mitigate the risk of serious drug-related adverse events, we have amended the IGNITE DMD clinical protocol to include the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and increasing the prednisone dose in the first month post dosing.

In March 2021, we announced interim data collected from the first six patients dosed in IGNITE DMD twelve to twenty-four months after treatment, including data from three patients dosed at the low dose (5E13vg/kg) and three patients dosed at the high dose (2E14 vg/kg). Data from the delayed treatment cohort, analyzed as an untreated control cohort, was evaluated as was representative natural history data. The six patients dosed in IGNITE DMD ranged in age from five to fourteen at baseline.

The data announced in March 2021 included functional data from the IGNITE DMD clinical trials, including:

- 6-Minute Walk tests (6MWT), North Star Ambulatory Assessments (NSAA) and pulmonary function tests (PFT), which provided evidence of potential benefit in functional endpoints one year after a single infusion of SGT-001 at a dose of 2E14 vg/kg, and to a lesser degree in the lower dose of 5E13 vg/kg;
- Mean increase in 6MWT distances was above the generally accepted minimally clinically important difference (MCID) of 30 meters in both the low and high-dose patients one year after treatment. While patients in the untreated control cohort exhibited a decline of 8.5 meters from baseline to one year, patients in the low dose cohort exhibited a mean improvement of 37 meters and patients in the high dose cohort exhibited an improvement of 49.7 meters over the same twelve-month period;
- Among patients in the low and high dose cohorts, NSAA scores at 1 year suggest benefit after treatment as compared to trajectories typically observed in natural history data. Natural history analyses suggest that patients similarly aged to those enrolled in IGNITE DMD would normally be expected to exhibit year over year disease progression ranging from a plateau in gains to a 3 to 3.7-point decline. Patients in the untreated control cohort exhibited a mean decline of 4.0 points from baseline to 1 year, while patients in the low dose cohort exhibited a mean improvement of 1.0 point over the same period of time. Patients in the high dose exhibited a mean improvement of 0.3 points as compared to their baseline values;
- With respect to spirometry testing of pulmonary function, the majority of patients in both the high dose and low dose groups exhibited improved forced vital capacity (% predicted FVC) at one year when declines in pulmonary function would otherwise be typically observed in patients with Duchenne. From baseline to one year, patients in the untreated control cohort exhibited a mean decline of 10.7% on an absolute basis, while patients in the low dose and high dose cohorts exhibited a mean improvement of 3.9% and 16.7%, respectively, over the same period.

The data also included the following biomarker data:

- Biopsies of skeletal muscle three months after a single infusion of SGT-001 at a dose of 2E14vg/kg, demonstrated widespread distribution of microdystrophin-positive muscle fibers with co-localization of nNOS and β -sarcoglycan in the muscles of these patients;
- Creatine kinase (CK) assessments of the six patients provide physiological evidence of a positive or stabilizing effect after one year of treatment with a single high dose infusion of SGT-001. An average sustained CK decline of approximately 50% in patients in the high dose was observed. In the low dose cohort, an average CK increase of approximately 166% was observed, and in the control group an average CK increase of approximately 17% was observed.

The Patient Reported Outcome Measure (PROM) assessments revealed a trend towards dose-response improvements in motor function subscales and fatigability assessments, providing real-world evidence to support the clinical and biomarker findings of varying degrees of benefit in low and high dose patients. The following PROM Data was highlighted:

- Meaningful improvements in the Pediatric Outcomes Data Collection Instrument (PODCI), a PROM that contains questions to assess how caregivers and children evaluate the child's ability to walk, stand, and perform activities of daily living, as well as recreational activities. Motor function scores in dosed patients reflect the gains seen in 6MWT and stability of NSAA observed in these patients;
- MODUS semi-structured interviews with patients and caregivers about the impact of Duchenne on functioning demonstrated overall improvement in functional activity and school-related impacts (e.g. lower limb mobility, keeping up with peers, climbing stairs, sports) in low and high-dose cohorts, with subjective decreased fatigability in patients of both treatment cohorts.

In March 2021, we announced that the seventh patient was treated with SGT-001 under the amended clinical protocol. This patient exhibited significantly reduced complement activation, without clinical manifestations, supporting continued enrollment in IGNITE DMD utilizing clinical supply produced from second generation manufacturing process; no new drug-related safety findings were identified seventeen to thirty-seven months post-infusion in the six patients previously dosed. We believe the unique functionality of our SGT-001 microdystrophin may result in functional benefits including diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle. Collectively, we believe these data provide evidence supporting the biological activity of SGT-001 and provide support for continued development.

The most common drug related clinical adverse reactions at the time of initial dosing were nausea, experienced by the six patients previously dosed; fever experienced by four of the six patients previously dosed; and vomiting, experienced by five of the six patients previously dosed. The most common drug related laboratory abnormalities were thrombocytopenia, increased fibrin D-dimer, increased soluble C5b9, increased LDH, proteinuria. Activation of the terminal pathway (sC5b9) of the classical complement system occurred in all six patients resulting in two serious adverse events, which were previously resolved. Two other significant adverse events included an episode of immune hepatitis four weeks post dosing which resolved rapidly after a transient increase of corticosteroids and one serious adverse event unrelated to SGT-001.

The following table summarizes the interim efficacy results of IGNITE-DMD:

Summary of Interim Efficacy Results of IGNITE-DMD
Absolute Change From Baseline to One Year

| | | CK (U/L) | NSAA | 6MWT (m) | FVC% | PODCI – Sports | PODCI – Global |
|-----------------------|------------------|-----------------|-------------|-----------------|-------------|-----------------------|-----------------------|
| Control | CT 1 15.3 yrs | 2,831 | n/a | n/a | -9.6% | -16 | -18 |
| | CT 2 9.5 yrs | -3,428 | -1 | -8 | -7.6% | -11 | -10 |
| | CT 3 6.2 yrs | -3,810 | -7 | -9 | -15.0% | n/a | n/a |
| 2E14 vg/kg | Pt 1 14.4 yrs | -1,507 | n/a | n/a | +8.9% | -6 | 18 |
| | PT 2 5.2 yrs | 14,300 | -3 | 12 | +5.3% | 5 | 6 |
| | PT 3 6.9 yrs | 13,846 | +5 | 62 | -2.4% | 21 | 9 |
| 2E14 vg/kg | PT 4 10.7 yrs | -8,455 | +1 | 12 | +3.1% | 22 | 13 |
| | PT 5 6.8 yrs | -8,381 | -1 | 85 | +36.7% | 28 | 11 |
| | PT 6 7.7 yrs | -5,305 | +1 | 52 | +10.2% | 39 | 27 |

Taking into account the prevalence and incidence of Duchenne 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 have developed a manufacturing process that we believe can scale to adequately support our needs for clinical trials, commercial launch and beyond. Our in-house scientists are continuing to work to increase the productivity, efficiency and purity of our manufacturing process.

In conjunction with our development of SGT-001, we believe it is critical to continue to invest time and resources in tools and technologies designed to help us more effectively understand Duchenne, 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.

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. For our gene transfer programs, we have exclusively licensed four issued U.S. patents and two pending U.S. patent applications, and nine issued patents and fourteen pending patent applications in foreign jurisdictions. We have filed five pending PCT international patent applications, and one pending patent application in Taiwan. We intend to continue building out our intellectual property protection to further strengthen our position in the Duchenne field.

Who we are

Solid Biosciences was founded in 2013 by our Chief Executive Officer, Ilan Ganot, our former Chairman of the Board, Andrey Zarur, our former President, Gilad Hayeem, our board member Matthew Arnold and our Head of Patient Advocacy Annie Ganot, with the goal of developing meaningful therapies for patients with Duchenne. 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 Duchenne, 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 Duchenne patients.

Since our inception, we have raised private capital from a group of top-tier corporate and private investors. In addition, three leading U.K.-based Duchenne charities provided initial seed funding for our gene transfer program in return for equity in our company, and we have accepted additional contributions from several Duchenne charities to fund our early-stage research programs. In January 2018, we completed our initial public offering resulting in net proceeds of \$129.1 million, after deducting underwriting discounts and commissions and offering expenses. In July 2019, we completed a private placement of shares of our common stock and pre-funded warrants to purchase shares of our common stock resulting in net proceeds to us of \$57.9 million, after deducting offering costs.

In October 2020, we entered into a collaboration and license agreement, or the "Collaboration Agreement," with Ultragenyx Pharmaceuticals, Inc. or "Ultragenyx" to focus on the development and commercialization of new gene therapies for Duchenne. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement with Ultragenyx, pursuant to which the Company issued and sold 7,825,797 shares of our common stock to Ultragenyx at a price of \$5.1113 per share.

In October 2020, the Company sold 6,309,632 shares of common stock pursuant to the ATM Sales Agreement resulting in net proceeds to the Company of \$23.2 million.

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

Mission

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

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

About Duchenne muscular dystrophy

Duchenne is an X-chromosome-linked, muscle-wasting disease, predominantly affecting boys. Progressive, irreversible and ultimately fatal, Duchenne 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 Duchenne, 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 Duchenne.

For patients suffering from Duchenne, 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 Duchenne 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, Duchenne also commonly involves cognitive difficulties and behavioral challenges.

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

Need for effective therapies

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

Glucocorticoid treatment, the current standard-of-care, has been shown to temporarily improve muscle strength, prolong the period of ambulation and slow the progression of Duchenne. However, glucocorticoid use is associated with well-known adverse 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).

In recent years, certain regulators have conditionally approved therapies which target specific mutations in the dystrophin gene. These therapies are indicated for only a small portion of the Duchenne patient population, and their respective efficacy profiles still need to be fully understood.

Current best practices for treating Duchenne 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, Duchenne represents a significant societal and economic burden. The economic burden, estimated at \$1.2 billion annually in the United States (excluding costly mortality and end-of-life care expenses), includes costs associated with hospital admissions, medication, frequent doctor visits and investment in assistive devices, as well as indirect costs related to productivity losses for the caregivers and costs due to pain, anxiety and social handicap. Of this amount, approximately 45% is represented by indirect costs. Only a small proportion of Duchenne patients are employed and many caregivers reduce their hours or stop working altogether to care for their children, who progressively require more help with everyday tasks, such as eating, dressing and using the bathroom. In some cases, patients also experience serious mental health issues that require additional support and treatment.

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 deoxyribonucleic acid, or 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 Duchenne.

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.

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

Our gene transfer candidate, or vector, includes three components:

- a viral capsid—a protein shell utilized as a vehicle to deliver 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.

SGT-001

SGT-001, our lead gene transfer candidate, is designed to preserve muscle function in Duchenne patients after a single administration and is based on some of the most recent understanding of dystrophin biology in the field. The SGT-001 vector is comprised of a functional transgene and a muscle-specific promoter, which are delivered via an AAV capsid. We believe that the SGT-001 construct is differentiated from other gene transfer candidates and may provide unique clinical benefit.

The vector is modified to no longer self-replicate, yet retains its 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.

Capsid: The capsid of the SGT-001 vector is derived from a naturally occurring, non-pathogenic virus called AAV. There are several subtypes of AAV capsids that differ based on the proteins that make up their structure. 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 efficiently enter skeletal, diaphragm and cardiac muscle tissues, as well as its favorable tolerability reported in other gene transfer clinical programs.

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 Duchenne.

Our SGT-001 microdystrophin construct is based on three decades of development and optimization work at the University of Missouri and the University of Washington as well as other academic institutions. In preclinical studies, the laboratories of Jeffrey Chamberlain, Ph.D., from the University of Washington, and Dongsheng Duan, Ph.D., from the University of Missouri, identified a proprietary configuration of genetic components that, when administered systemically, produces functional microdystrophin protein expression that not only stabilizes muscle membranes and protects muscle against injury, but also simultaneously restores the localization of DGC to the muscle membrane, notably increasing neuronal nitric oxide synthase, or nNOS, concentration. In subsequent published studies, Drs. Duan and Chamberlain demonstrated in animal models that, in comparison to earlier configurations, nNOS-restoring microdystrophins were more effective in improving muscle function and resistance to fatigue. We believe the unique functionality of our SGT-001 microdystrophin may result in functional benefits including diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle.

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 was 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 non-human primates, or NHPs. Our preclinical studies were performed by third-party collaborators.

Well-established mouse and dog disease models for Duchenne 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.

Because Duchenne 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.
- 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. Muscle strength assessments in mild and severe mouse models of Duchenne demonstrated functional improvements in animals treated with SGT-001 in comparison to untreated dystrophic controls. 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 immunofluorescence staining in the quadriceps and heart muscle tissues was 50% and 80%, respectively, 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 statistically significant improvements in multiple muscle strength parameters as well as resistance to treadmill-induced fatigue.

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 Duchenne 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). Robust transgene expression was detected by immunofluorescence at all biopsy time points and at all of the dose levels. In animals dosed with 1E14 vg/kg, approximately 70-90% of the muscle fibers were positive for microdystrophin and correlated to the restoration of DGC associated proteins, including nNOS.

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.

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.

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.

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. All doses were well tolerated and there was no observed immune response to the transgene.

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 good laboratory practices, or 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 trials. In the NHP study, test-article-related effects were self-limited, mild chemistry and hematology changes with no microscopic correlates at the end of the study. There was a transient and asymptomatic increase in liver function enzymes observed in NHPs starting on day 9, which returned to normal levels by day 21. We believe there were no other relevant test-article-related adverse events associated with SGT-001 administration in either GLP study. In the NHP toxicology study, a single animal from the high dose cohort was euthanized after it did not recover from an anesthetic procedure. We believe this event was attributed to procedural errors. However, AAV vector cannot be completely ruled out as a contributing factor to the toxicity that gave rise to the event.

Clinical development of SGT-001

We are developing SGT-001 for the treatment of Duchenne 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 trial designed to evaluate SGT-001 in ambulatory and non-ambulatory males with Duchenne aged four to 17 years. 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 clinical trial is also designed to assess muscle function and mass, respiratory and cardiovascular function, serum and muscle biomarkers associated with microdystrophin production, SGT-001 microdystrophin associated biochemical properties (e.g., nNOS binding), functional outcome, patient and parent reported outcomes and quality of life measures, among other endpoints. Key inclusion criteria include: established clinical diagnosis of Duchenne and documented dystrophin gene mutation predictive of Duchenne phenotype; anti-AAV9 antibodies below pre-specified thresholds; stable cardiac and pulmonary function; and a stable daily dose of oral corticosteroids for 12 weeks. There is no enrollment restriction in the clinical trial protocol based on a patient's underlying dystrophin gene mutation.

In the current IGNITE DMD protocol, as amended, participants are all assigned to open label treatment with SGT-001. The selection of the original starting dose in our first cohort, 5E13 vg/kg, was based on safety and efficacy data observed in our preclinical studies. Dose escalation and decisions regarding clinical trial progression occur after review by the DSMB. Efficacy is being assessed by comparing microdystrophin protein expression in muscle biopsy before and 12 months after treatment for each patient. An intermediate biopsy at either 45 days, three months, six months or nine months will inform the time course of microdystrophin expression. Long-term follow up will continue per regulatory guidelines.

In February 2019, we announced preliminary findings based on three-month biopsy data from the first three patients dosed with 5E13 vg/kg of SGT-001, the lowest dose outlined in the trial protocol. In one patient, SGT-001 microdystrophin was detected via western blot below the five percent level of quantification of the assay and in approximately 10 percent of fibers via immunofluorescence. Due to these findings, and in consultation with the DSMB, in May 2019 we announced that we had initiated dosing of the next cohort of patients at 2E14 vg/kg. To date we have dosed 3 patients with the 2E14 vg/kg dose. In August 2019, we amended our protocol to remove the matched patient control arm for the rest of the 2E14 vg/kg cohort in the IGNITE DMD trial and to provide measures to potentially improve safety, such as initially proceeding to dose patients weighing 25 kg or less.

In November 2019, we announced that the third patient in the 2E14 vg/kg cohort of IGNITE DMD, dosed in late October 2019, experienced an SAE deemed related to the study drug and that IGNITE DMD was placed on clinical hold by the FDA as a result of the SAE. The SAE was characterized by complement activation, thrombocytopenia, a decrease in red blood cell count, acute kidney injury, and cardio-pulmonary insufficiency. Neither cytokine- nor coagulopathy-related abnormalities were observed. In December 2019, we reported that the SAE had fully resolved and the patient had resumed his normal activities. In December 2019, we announced preliminary findings based on three-month biopsy data from the first two patients dosed with 2E14 vg/kg of SGT-001. Using two independent immunofluorescence assays, 10% to 20% of microdystrophin positive muscle fibers were determined to express SGT-001 microdystrophin in the fourth patient and 50% to 70% of microdystrophin positive muscle fibers in the fifth patient. Immunofluorescence also showed clear stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin in both patients. Using western blot, the expression levels for the fourth patient were detectable and estimated to be near the assay's level of quantification which is 5% of non-dystrophic control samples, with one assay replicate at 5.5%. Expression for the fifth patient was 17.5% of normal control samples. The levels of serum creatine kinase, a highly variable biochemical marker of muscle damage, declined from baseline in both patients.

In March 2020, we announced data from the third patient dosed in the 2E14 vg/kg dose cohort of IGNITE DMD, including three-month biopsy data. Using immunofluorescence assays, 50% to 70% of the muscle fibers were determined to express SGT-001 microdystrophin. Immunofluorescence also showed stabilization and co-localization of nNOS and beta-sarcoglycan with SGT-001 microdystrophin. Using western blot, microdystrophin expression was 8% of normal control samples. In addition, the level of serum creatine kinase decreased from baseline.

In April 2020, we submitted a response to the FDA that included changes to the clinical protocol designed to enhance patient safety, as well as information related to improvements to our manufacturing process. The FDA responded by maintaining the clinical hold and requesting further data and analyses relating to this manufacturing process. In June 2020, we submitted a response to the FDA that provided data related to manufacturing process improvements. In July 2020, we announced that the FDA responded by maintaining the clinical hold and requesting further manufacturing information and updated safety and efficacy data for all patients dosed in the trial, as well as providing direction on the total viral load to be administered per patient. In October 2020, we announced that the FDA lifted the clinical hold placed on our IGNITE DMD Phase I/II clinical trial. In connection with the lifting of the clinical hold, we are reducing the maximum weight of the next two patients dosed in IGNITE DMD to 18 kg per patient, with safety outcomes from these two patients driving potential weight increase of patients dosed subsequently. This reduction, in conjunction with the delivery of fewer viral particles as a result of our manufacturing process improvements, will reduce patients' total viral load while continuing dosing at the 2E14 vg/kg dose. Additionally, to mitigate the risk of serious drug-related adverse events, we have amended the IGNITE DMD clinical protocol to include the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and increase the prednisone dose in the first month post dosing

In March 2021, we announced interim data collected from the first six patients dosed in IGNITE DMD twelve to twenty-four months after treatment, including data from three patients dosed at the low dose (5E13vg/ kg) and three patients dosed at the high dose (2E14 vg/kg). Data from the delayed treatment cohort, analyzed as an untreated control cohort, was evaluated as was representative natural history data. The six patients dosed in IGNITE DMD ranged in age from five to fourteen at baseline.

The data announced in March 2021 included functional data from the IGNITE DMD clinical trials, including:

- 6MWT, NSAA and PFT, which provided evidence of potential benefit in functional endpoints one year after a single infusion of SGT-001 at a dose of 2E14 vg/kg, and to a lesser degree in the lower dose of 5E13 vg/kg;
- Mean increase in 6MWT distances was above the generally accepted MCID of 30 meters in both the low and high-dose patients one year after treatment. While patients in the untreated control cohort exhibited a decline of 8.5 meters from baseline to one year, patients in the low dose cohort exhibited a mean improvement of 37 meters and patients in the high dose cohort exhibited an improvement of 49.7 meters over the same twelve-month period;
- Among patients in the low and high dose cohorts, NSAA scores at 1 year suggest benefit after treatment as compared to trajectories typically observed in natural history data. Natural history analyses suggest that patients similarly aged to those enrolled in IGNITE DMD would normally be expected to exhibit year over year disease progression ranging from a plateau in gains to a 3 to 3.7-point decline. Patients in the untreated control cohort exhibited a mean decline of 4.0 points from baseline to 1 year, while patients in the low dose cohort exhibited a mean improvement of 1.0 point over the same period of time. Patients in the high dose exhibited a mean improvement of 0.3 points as compared to their baseline values;
- With respect to spirometry testing of pulmonary function, the majority of patients in both the high dose and low dose groups exhibited improved forced vital capacity (% predicted FVC) at one year when declines in pulmonary function would otherwise be typically observed in patients with Duchenne. From baseline to one year, patients in the untreated control cohort exhibited a mean decline of 10.7% on an absolute basis, while patients in the low dose and high dose cohorts exhibited a mean improvement of 3.9% and 16.7%, respectively, over the same period.

The data also included the following biomarker data:

- Biopsies of skeletal muscle three months after a single infusion of SGT-001 at a dose of 2E14vg/kg, demonstrated widespread distribution of microdystrophin-positive muscle fibers with co-localization of nNOS and β -sarcoglycan in the muscles of these patients;
- CK assessments of the six patients provide physiological evidence of a positive or stabilizing effect after one year of treatment with a single high dose infusion of SGT-001. An average sustained CK decline of approximately 50% in patients in the high dose cohort was observed. In the low dose cohort, an average CK increase of approximately 166% was observed, and in the control group an average CK increase of approximately 17% was observed.

PROM assessments revealed a trend towards dose-response improvements in motor function subscales and fatigability assessments, providing real-world evidence to support the clinical and biomarker findings of varying degrees of benefit in low and high dose patients. The following PROM Data was highlighted:

- Meaningful improvements in the PODCI, a PROM that contains questions to assess how caregivers and children evaluate the child's ability to walk, stand, and perform activities of daily living, as well as recreational activities. Motor function scores in dosed patients reflect the gains seen in 6MWT and benefit of NSAA observed in these patients.
- MODUS semi-structured interviews with patients and caregivers about the impact of Duchenne on functioning demonstrated overall improvement in functional activity and school-related impacts (e.g., lower limb mobility, keeping up with peers, climbing stairs, sports) in low- and high-dose cohorts, with subjective decreased fatigability in patients of both treatment cohorts.

In March 2021, we announced that the seventh patient was treated with SGT-001 under the amended clinical protocol. This patient exhibited significantly reduced complement activation, without clinical manifestations, supporting continued enrollment in IGNITE DMD utilizing clinical supply produced from second generation manufacturing process; no new drug-related safety findings were identified seventeen to thirty-seven months post-infusion in the six patients previously dosed.

We believe the unique functionality of our SGT-001 microdystrophin may result in functional benefits including diminished muscle fatigue and protection against ischemic muscle damage, which can lead to loss of functional muscle. Collectively, we believe these data provide evidence supporting the biological activity of SGT-001 and provide support for continued development. Based on data from the clinical trial, we will determine next steps for SGT-001 clinical development, including additional clinical trials that may include other patient populations, as well as the need for larger confirmatory clinical trials.

The most common drug related clinical adverse reactions at the time of initial dosing were nausea, experienced by the six patients previously dosed; fever experienced by four of the six patients previously dosed; and vomiting, experienced by five of the six patients previously dosed. The most common drug related laboratory abnormalities were thrombocytopenia, increased fibrin D-dimer, increased soluble C5b9, increased LDH, proteinuria. Activation of the terminal pathway (sC5b9) of the classical complement system occurred in all six patients resulting in two serious adverse events, which were previously resolved. Two other significant adverse events included an episode of immune hepatitis four weeks post dosing which resolved rapidly after a transient increase of corticosteroids and one serious adverse event unrelated to SGT-001.

Manufacturing SGT-001

The prevalence and incidence of Duchenne, 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 support 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 our Contract Development Manufacturing Organization, or CDMO, partners. The process consists of three steps. First, we produce two replication-incompetent Herpes Simplex Virus, or HSV, stocks, one containing our microdystrophin construct and the other containing the critical elements of the AAV9. Second, we then use these two HSV stocks to coinfect suspension-adapted human embryonic kidney cells (HEK-293). Third, these cells 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.

In 2020, we implemented our second generation manufacturing process which collectively includes improvements to our AAV manufacturing methods as well as the introduction of a refined set of analytical methods for product release. Our second generation AAV manufacturing process includes a scalable purification step which results in a significant enrichment of microdystrophin-containing “full” capsids and elimination of empty capsids. On average, drug product produced by the second generation manufacturing method contains 90% full capsids, whereas drug product produced by the first generation process is on average 50% full. This improvement in purification methods significantly reduces the total capsid load administered at any given dose level, allowing us to achieve the same effective dose of drug product with fewer total capsids. As of Q1 2021, and following lift of the clinical hold, all patients dosed in IGNITE-DMD will be dosed with material produced using our second generation manufacturing process.

We believe that our decision to invest early in our manufacturing process was key to developing a scalable process designed to support clinical development and potential commercialization. We are supplying our clinical development program for SGT-001 with drug product produced at current good manufacturing practices, or cGMP, - compliant facilities located at partner CDMOs. We have operated at 250-liter scale and have successfully produced multiple drug product batches at this scale. Our in-house scientists are continuing to work to increase the productivity, efficiency and purity of our manufacturing process.

Complementary disease-modifying therapy: Anti-LTBP4

While we believe gene transfer may be able to slow or halt Duchenne disease progression, many patients would still suffer from the manifestations of the disease, such as tissue damage to their muscles, impaired muscle strength, inflammation, cardiac dysfunction and fibrosis.

Anti-LTBP4 is our complementary disease modifying program with the goal of identifying and developing a monoclonal antibody intended to reduce fibrosis and inflammation by targeting and stabilizing 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.

The rationale for targeting LTBP4 originated from observations in Duchenne 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 Duchenne 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 Duchenne 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 order to focus our efforts on addressing the underlying cause of Duchenne through our SGT-001 program, we have significantly curtailed investment in our other programs, including anti-LTBP4 which has been discontinued.

Tools to accelerate discovery and development

In conjunction with our development of SGT-001, we believe it is critical to continue 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 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.

Non-invasive biochemical and imaging biomarkers

We are working to identify non-invasive biochemical 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 Duchenne patients by using established imaging techniques. We are also currently using leading, robust platforms to perform extensive analysis on patient samples to establish molecular signatures based on various stages of Duchenne disease progression.

Assistive devices

Solid Suit

In partnership with Silicon Valley's Seismic and Parent Project Muscular Dystrophy (PPMD), we have advanced development of 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 was done with the insight and oversight of technology innovators and engineering and disease experts, and each step 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. The Solid Suit is comprised of three separate components, all of which are currently at prototype development.

In order to focus our efforts on addressing the underlying cause of Duchenne through our SGT-001 program, we have significantly curtailed investment in our other programs, including the Solid Suit.

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 February 16, 2021, we have filed five pending PCT international patent applications, and one pending patent application in Taiwan. For our gene transfer programs, we have exclusively licensed four issued U.S. patents, three pending U.S. non-provisional patent applications, and nine granted patents and thirteen pending patent applications in foreign jurisdictions. The issued U.S. patents are projected to expire between 2021 and 2036, excluding any patent term adjustments and any patent term extensions, and any U.S. patents that may issue from the pending U.S. non-provisional patent applications would be projected to expire between 2036 and 2039, 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 two issued U.S. patents and two pending U.S. non-provisional patent applications, which generally claim the structural elements of SGT-001 and the promoter sequence used in SGT-001. These issued U.S. patents are projected to expire in 2028 and 2036, excluding any patent term adjustments and any patent term extensions.

Relating to SGT-001, we also own five pending PCT international patent applications. Any patents that may be issued from the pending PCTs would be projected to expire between 2036 and 2041, excluding any patent term adjustments and any patent term extensions. Substantive prosecution of our patent applications has not yet commenced at the U.S. Patent and Trademark Office, or USPTO. We cannot predict whether such pending patent applications will result in the issuance of 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 patent offices in various jurisdictions are often significantly narrowed by the time they issue, if they issue at all.

With respect to our Anti-LTBP4 program, we do not currently own or in-license any issued patents or patent applications relating to such product candidate. We have exercised an exclusive option to negotiate for licenses of certain patents and patent applications relating to Anti-LTBP4 from Ikaika Therapeutics, LLC. 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. In order to focus our efforts on addressing the underlying cause of Duchenne through our SGT-001 program, we have significantly curtailed investment in our other programs, including anti-LTBP4.

The term of individual patents depends upon the legal term for patents in the countries in which they are obtained. In most countries, including the United States, the patent term is 20 years from the earliest filing date of a non-provisional patent application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the USPTO in examining and granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier filed patent. The term of a patent that covers a drug or biological product may also be eligible for patent term extension when FDA approval is granted, subject to certain limitations and provided statutory and regulatory requirements are met (for more information, please see "Business— Government regulation and product approval —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 Duchenne and related disease indications caused by a lack of functional dystrophin. We have the right to grant sublicenses to third parties contingent upon written approval by the University of Washington prior to executing such sublicense, which approval may not be unreasonably withheld.

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We are required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately \$1 million upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2020, 2019 and 2018. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017. In October 2020, the license agreement was amended such that we were required to pay the University of Washington \$375 thousand in connection with the execution of the Collaboration Agreement. This payment was recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that we are required to pay an aggregate of approximately \$3.4 million upon the achievement of certain milestones. We must also pay royalties of a low single digit percentage of future sales by us and our sublicensees of products developed under the licensed patent rights. In addition, we must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to us and our sublicensees.

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

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

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

The University of Missouri License Agreement

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

In consideration for the rights granted by the agreement, we paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. We 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.

Under the agreement, in the event we grant a sublicense to another party, we are required to pay the University of Missouri a percentage of the consideration received. The license agreement was amended such that we are required to pay the University of Missouri \$0.8 million in 2021 and \$1.3 million in 2022 as a result of the execution of the Collaboration Agreement with Ultragenyx in October 2020. These amounts were recorded as a research and development expense in the fourth quarter of 2020. We paid \$0.8 million in February 2021. The license agreement was also amended such that we are required to make aggregate milestone payments of approximately \$1.9 million upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2020, 2019, and 2018. We must pay a royalty of a low single digit percentage of future sales or by its sublicensees of products developed using the licensed patents. In addition, we must pay an annual maintenance fee until certain milestones are achieved, after which time a minimum annual royalty will replace such maintenance fee.

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

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

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

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

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 during the years ended December 31, 2020, 2019 and 2018. 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 Duchenne.

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 during the years ended December 31, 2020, 2019 and 2018 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.

Ultragenyx Collaboration Agreement

On October 22, 2020, or the Effective Date, we entered into a collaboration and license agreement with Ultragenyx, to focus on the development and commercialization of new gene therapies for Duchenne. We granted Ultragenyx an exclusive worldwide license for any pharmaceutical product that expresses our proprietary microdystrophin construct from AAV8 and variants thereof in clade E for the treatment of Duchenne and other diseases resulting from the lack of functional dystrophin. We are conducting certain activities agreed to by the parties with respect to the development of licensed products. Ultragenyx will reimburse us for personnel and out-of-pocket costs that we incur in conducting such development activities. Otherwise, Ultragenyx has decision-making authority with respect to the development, manufacturing and commercialization of licensed products. We retain exclusive rights to all other uses of our microdystrophin proteins, including under our existing SGT-001 program.

We are conducting certain activities agreed to by the parties with respect to the development of licensed products. Ultragenyx is obligated to reimburse us for personnel and out-of-pocket costs that we incur in conducting such development activities. Otherwise, Ultragenyx has decision-making authority with respect to the development, manufacturing and commercialization of licensed products. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement and an investor agreement with Ultragenyx, pursuant to which we issued and sold 7,825,797 shares of our common stock to Ultragenyx at a price of \$5.1113 per share for an aggregate purchase price of approximately \$40.0 million. The shares purchased by Ultragenyx are subject to a lock-up period until the earliest to occur of (i) 18 months

from the closing date, (ii) the termination of the Collaboration Agreement or (iii) other specified events. Pursuant to the terms of the investor agreement, Ultragenyx agreed that, so long as it holds at least 10% of our outstanding common stock, the shares will be subject to a voting agreement, such that until the earliest to occur of certain specified events, and subject to specified conditions, Ultragenyx will, and will cause its permitted transferees to, vote in accordance with the recommendation of our board of directors with respect to specified matters.

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

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

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

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

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

As described in Note 3, in October 2020, the Company entered into the Collaboration Agreement with Ultragenyx for the research, development and commercialization of other pharmaceutical products that express the Company's MD5 nNOS binding domain form of microdystrophin protein. Ultragenyx is a related party since Ultragenyx is one of the Company's significant stockholders. During the year ended December 31, 2020, the Company recognized no related party revenue associated with the Collaboration Agreement. As of December 31, 2020, there was \$20,717 of deferred revenue related to the Collaboration Agreement, which is classified as current or non-current in the consolidated balance sheets. The Company has made no payments to Ultragenyx during the year ended December 31, 2020. There is no amount due from Ultragenyx as of December 31, 2020.

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 Duchenne, 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 with product candidates in clinical development focused on developing systemic gene transfers for Duchenne, including Pfizer Inc., with a product candidate currently in Phase III clinical development, and Sarepta Therapeutics, Inc., with a product candidate currently in Phase I/II clinical trial development. In addition, there are four therapies, which are intended to be disease modifying, that are currently approved for Duchenne by certain regulators. These products are eteplirsen (EXONDYS 51) and ataluren (Translarna), each of which is indicated for approximately 13% of Duchenne patients, golodirsen (VYONDYS 53), which is indicated for approximately 8% of Duchenne patients and casimersen (AMONDYS 45), which is indicated for approximately 8% of Duchenne patients. Any advances in gene transfer technology made by a competitor may be used to develop therapies that could compete with our lead product candidate or other product candidates we may develop

Government regulation and product licensure

U.S. government regulation and product licensure

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

Within the FDA, the Center for Biologics Evaluation and Research, or the CBER, regulates gene therapy products. Within CBER, the review of gene therapy and related products is consolidated in the Office of Tissues and Advanced Therapies, or the OTAT, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. CBER, which works closely with the NIH and the RAC, 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. The FDA has licensed human gene therapies products for sale in the United States, and the agency has provided guidance for the development of other 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 GLP requirements 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 institutional review board, or IRB, reviewing each clinical site before each clinical trial may be initiated;
- approval by an institutional biosafety committee, or IBC, assessing the safety of the clinical research and identifying any potential risk to public health or the environment;

- performance of adequate and well controlled human clinical trials according to the FDA’s regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection of human research subjects and their health information, to establish the safety, potency and purity of the proposed biologic product for its intended use;
- preparation and submission to the FDA of a biologics license application, or 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;
- FDA review and licensure of the BLA; and
- compliance with any post-approval requirements, including the potential requirement to implement a Risk Evaluation and Mitigation Strategy, or REMS, and the potential requirement to conduct post-approval studies.

Preclinical Studies and Investigational New Drug Application

Before testing any biologic product candidate in humans, including a gene therapy product candidate, the product candidate must undergo preclinical testing. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as *in vivo* studies to assess the potential safety and activity of the product candidate and to establish a rationale for therapeutic use. 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. An IND is an exemption from the FD&C Act that allows an unapproved product to be shipped in interstate commerce for use in an investigational clinical trial and a request for FDA authorization to administer an investigational product to humans. 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 full clinical hold or partial clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. 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 delay 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.

Human clinical trials under an IND

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

Further, each clinical trial must be reviewed and approved by an IRB at or servicing each institution at which the clinical trial will be conducted. An IRB is charged with protecting the welfare and rights of trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative, reviews and approves the study protocol and must monitor the clinical trial until completed. Additionally, some trials are overseen by an independent group of qualified experts organized by the trial sponsor, known as a data safety monitoring board or committee, or DSMB. This group provides authorization as to whether or not a trial may move forward at designated check points based on access that only the group maintains to available data from the study. Clinical trials involving recombinant DNA also must be reviewed by an IBC a local institutional committee that reviews and oversees basic and clinical research and utilizes recombinant DNA at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

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

- *Phase I.* The investigational biologic product is initially introduced into a small group of healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early understanding of its effectiveness. In the case of some product candidates for severe or life-threatening diseases, especially when the product candidate may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients. Phase 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 clinical trials, the investigational biologic product is administered to an expanded patient population, generally at multiple geographically dispersed clinical trial sites in adequate and well controlled clinical trials to generate sufficient data to statistically confirm the potency and safety of the product for approval. These clinical trials are intended to establish the overall risk/benefit ratio of the product candidate and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes 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.

Information about certain clinical trials must be submitted within specific timeframes to the NIH for public dissemination on its ClinicalTrials.gov website.

Expanded Access to an Investigational Drug for Treatment Use

Expanded access, sometimes called "compassionate use," is the use of investigational new drug products outside of clinical trials to treat patients with serious or immediately life-threatening diseases or conditions when there are no comparable or satisfactory alternative treatment options. The rules and regulations related to expanded access are intended to improve access to investigational drugs for patients who may benefit from investigational therapies. FDA regulations allow access to investigational drugs under an IND by the company or the treating physician for treatment purposes on a case-by-case basis for: individual patients (single-patient IND applications for treatment in emergency settings and non-emergency settings); intermediate-size patient populations; and larger populations for use of the drug under a treatment protocol or Treatment IND Application.

When considering an IND application for expanded access to an investigational product with the purpose of treating a patient or a group of patients, the sponsor and treating physicians or investigators will determine suitability when all of the following criteria apply: patient(s) have a serious or immediately life-threatening disease or condition, and there is no comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition; the potential patient benefit justifies the potential risks of the treatment and the potential risks are not unreasonable in the context or condition to be treated; and the expanded use of the investigational drug for the requested treatment will not interfere with initiation, conduct, or completion of clinical investigations that could support marketing approval of the product or otherwise compromise the potential development of the product.

There is no obligation for a sponsor to make its investigational products available for expanded access; however, as required by the 21st Century Cures Act, or the Cures Act, passed in 2016, if a sponsor has a policy regarding how it responds to expanded access requests, it must make that policy publicly available. Although these requirements were rolled out over time, they have now come into full effect. Although these requirements were rolled out over time, they have now come into full effect. This provision requires drug and biologic companies to make publicly available their policies for expanded access for individual patient access to products intended for serious diseases. Sponsors are required to make such policies publicly available upon the earlier of initiation of a Phase II or Phase III study; or 15 days after the drug or biologic receives designation as a breakthrough therapy, fast track product, or regenerative medicine advanced therapy.

In addition, on May 30, 2018, the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a drug manufacturer to make its drug products available to eligible patients as a result of the Right to Try Act, but the manufacturer must develop an internal policy and respond to patient requests according to that policy.

Special Regulations and Guidance Governing Gene Therapy Products

The FDA has defined a gene therapy product as one that mediates its effects by transcription and/or translation of transferred genetic material and/or by integrating into the host genome and which is administered as nucleic acids, viruses, or genetically engineered microorganisms. The products may be used to modify cells in vivo or transferred to cells ex vivo prior to administration to the recipient. Within the FDA, the Center for Biologics Evaluation and Research, or CBER, regulates gene therapy products. 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 Local Biosafety Board, a federal advisory committee, in reviewing proposed and ongoing gene therapy protocols and engaging in a public discussion of scientific, safety, ethical, and societal issues related to those protocols. The NIH and the Recombinant DNA Advisory Committee, or RAC, a federal advisory committee, also advise the FDA on gene therapy issues and other issues related to emerging technologies. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols.

The FDA has issued various guidance documents regarding gene therapies, including recent final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, gene therapies for rare diseases and gene therapies for retinal disorders, as well as draft guidance in January 2021 for Human Gene Therapy for Neurodegenerative Diseases. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy product candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire.

If a gene therapy trial is conducted at, or sponsored by, institutions receiving the NIH funding for recombinant DNA research, a protocol and related documentation 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 prior to the submission of an IND to the FDA. In addition, many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH will convene the RAC to discuss 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.

Further, to facilitate adverse event reporting and dissemination of additional information about gene therapy trials, the FDA and the NIH established the Genetic Modification Clinical Research Information System, or GeMCRIS. Investigators and sponsors of human gene transfer trials can utilize this web-based system to report serious adverse events and to provide annual reports.

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

Pediatric Studies

Under the Pediatric Research Equity Act of 2003, or PREA, a BLA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. Sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

For products intended to treat a serious or life-threatening disease or condition, the FDA must, upon the request of an applicant, meet to discuss preparation of the initial pediatric study plan or to discuss deferral or waiver of pediatric assessments. In addition, the FDA will meet early in the development process to discuss pediatric study plans with sponsors and FDA must meet with sponsors by no later than the end-of-phase 1 meeting for serious or life-threatening diseases and by no later than 90 days after the FDA's receipt of the study plan.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in the Food and Drug Administration Safety and Innovation Act, or FDASIA. The FDA also maintains a list of diseases that are exempt from the requirements PREA, due to low prevalence of disease in the pediatric population.

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 licensure of a BLA must be obtained before commercial marketing of the biologic product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. In addition, under the 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. Under federal law, the submission of most NDAs is subject to an application user fee, which for federal fiscal year 2021 is \$2.9 million for an application requiring clinical data. The sponsor of an approved NDA is also subject to an annual program fee, which for fiscal year 2021 is \$0.3 million. 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.

Biosimilars and exclusivity

The Patient Protection and Affordable Care Act and the companion Health Care and Education Reconciliation Act, or the Health Care Reform Law, which was signed into law on March 23, 2010, included a subtitle called the Biologics Price Competition and Innovation Act of 2009, or BPCIA. That Act established a regulatory scheme authorizing the FDA to approve biosimilars and interchangeable biosimilars.

Under the BPCIA, a manufacturer may submit an application for licensure of a biologic product that is "biosimilar to" or "interchangeable with" a previously approved biological product or "reference product." In order for the FDA to approve a biosimilar product, it must find that there are no clinically meaningful differences between the reference product and proposed biosimilar product in terms of safety, purity and potency. For the FDA to approve a biosimilar product as interchangeable with a reference product, the agency must find that the biosimilar product can be expected to produce the same clinical results as the reference product, and (for products administered multiple times) that the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic.

Under the BPCIA, an application for a biosimilar product may not be submitted to the FDA until four years following the date of approval of the reference product. The FDA may not approve a biosimilar product until 12 years from the date on which the reference product was approved. Even if a product is considered to be a reference product eligible for exclusivity, another company could market a competing version of that product if the FDA approves a full BLA for such product containing the sponsor's own preclinical data and data from adequate and well-controlled clinical trials to demonstrate the safety, purity and potency of their product. The BPCIA also created certain exclusivity periods for biosimilars approved as interchangeable products. At this juncture, it is unclear whether products deemed "interchangeable" by the FDA will, in fact, be readily substituted by pharmacies, which are governed by state pharmacy law.

As of December 27, 2020 (enacted as part of the Consolidated Appropriations Act, 2021), the “patent dance” lists will now become public information as listed in the Purple Book (FDA’s “Database of Licensed Biological Products”). In particular, reference product BLA holders must submit to the FDA within 30 days of exchanging a patent list (patents with expiry dates) with a biosimilar applicant, as well as any supplemental lists. This information was previously maintained a confidential as between the BLA holder and biosimilar applicant. Despite publication of these lists, a BLA holder may assert other patents against future filers, and does not exclude enforcement of newly granted patents.

Additionally, under the Act, the FDA must now publish in the Purple Book the following information about patented biological products:

- a list of each biological product, by nonproprietary name, for which a biologics license is in effect;
- the date of licensure and the application number;
- the licensure status and, as available, the marketing status; and
- exclusivity periods.

The FDA must publish in the Purple Book all of the above information in the first instance within 180 days of enactment and update every 30 days.

As of January 1, 2021, the FDA has approved 29 biosimilar products for use in the United States. No interchangeable biosimilars, as described below, have been approved. The FDA has issued several guidance documents outlining an approach to review and approval of biosimilars. Additional guidances are expected to be finalized by the FDA.

Pediatric exclusivity

Pediatric exclusivity is another type of non-patent exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including reference product and orphan exclusivity. This six-month exclusivity may be granted if an application sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA’s request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity cover the product are extended by six months. Thus, pediatric exclusivity adds six months to existing exclusivity periods applicable to biological products under the BPCIA—namely, the four-year period during which the FDA will not consider an application for a biosimilar product, and the 12-year period during which the FDA will not approve a biosimilar application.

Orphan drug designation and exclusivity

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

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, meaning that the FDA may not approve any other applications to market the same drug or biologic product for the same indication for seven years, except in limited circumstances, such as if the party holding the exclusivity fails to assure the availability of sufficient quantities of the drug to meet the needs of patients with the disease or condition for which the drug was designated. In addition, the FDA may not approve other applications to market the same drug or biologic product for the same indication for seven years unless the sponsor of the other product demonstrates that its product is clinically superior to the product with orphan drug exclusivity. Under Omnibus legislation signed by President Trump on December 27, 2020, this clinical superiority requirement applies to drugs and biologics that received orphan drug designation before enactment of FDARA in 2017, but have not yet been approved or licensed by FDA.

Orphan exclusivity does not block the approval of a different product for the same rare disease or condition, nor does it block the approval of the same product for different indications. In particular, the concept of what constitutes the "same drug" for purposes of orphan drug exclusivity remains in flux in the context of gene therapies, and the FDA has issued recent draft guidance suggesting that it would not consider two gene therapy products to be different drugs solely based on minor differences in the transgenes or vectors. If a product designated as an orphan drug ultimately receives marketing approval for an indication broader than what was designated in its orphan drug application, it may not be entitled to exclusivity. Orphan medicinal product status in the European Union has similar, but not identical, benefits.

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.
- *Regenerative advanced therapy.* With passage of the Cures Act in December 2016, Congress authorized the FDA to accelerate review and approval of products designated as regenerative advanced therapies. A product is eligible for this designation if it is a regenerative medicine therapy that is intended to treat, modify, reverse or cure a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product candidate has the potential to address unmet medical needs for such disease or condition. The benefits of a regenerative advanced therapy designation include early interactions with the FDA to expedite development and review, benefits available to breakthrough therapies, potential eligibility for priority review and accelerated approval based on surrogate or intermediate endpoints.

None of these expedited programs change the standards for approval but they may help expedite the development or approval process of product candidates.

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 requirements, which impose certain procedural, substantive and recordkeeping requirements. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain compliance with cGMP and other aspects of regulatory compliance.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Products may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability. If a company is found to have promoted off-label uses, it may become subject to adverse public relations and administrative and judicial enforcement by the FDA, the Department of Justice, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

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 Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent terms lost during product development and FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period generally is one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biologic product is eligible for the extension, the application for the extension must be submitted prior to the expiration of the patent, and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. Moreover, a given patent may only be extended once based on a single product. The USPTO in consultation with the FDA, reviews and approves the application for any patent term extension or restoration.

Government regulation outside of the U.S.

In addition to regulations in the United States, a manufacturer is subject to a variety of regulations in foreign jurisdictions to the extent it chooses to sell any products in those foreign countries. Even if a manufacturer obtains FDA approval of a product, it must still obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Because biologically sourced materials are subject to unique contamination risks, their use may also be restricted in some countries.

Clinical trial approval in the European Union

Pursuant to the currently applicable Clinical Trials Directive 2001/20/EC and the Directive 2005/28/EC on Good Clinical Practice, an applicant must obtain approval from the competent national authority of the European Union Member State, or the EU Member State, in which the clinical trial is to be conducted. If the clinical trial is conducted in different EU Member States, the competent authorities in each of these EU Member States must provide their approval for the conduct of the clinical trial. Furthermore, the applicant may only start a clinical trial at a specific study site after the competent ethics committee has issued a favorable opinion. In April 2014, the European Union adopted a new Clinical Trials Regulation (EU) No 536/2014, which is set to replace the current Clinical Trials Directive 2001/20/EC. The new Clinical Trials Regulation will be directly applicable to and binding in all EU Member States without the need for any national implementing legislation.

Specifically, the new Regulation aims at simplifying and streamlining the approval of clinical trials in the European Union. Under the new coordinated procedure for the approval of clinical trials, the sponsor of a clinical trial to be conducted in more than one EU Member State will only be required to submit a single application for approval of a clinical trial to a reporting EU Member State. The Clinical Trials Regulation also aims to streamline and simplify the rules on safety reporting for clinical trials. As of January 1, 2021, the website of the European Commission reported that the implementation of the Clinical Trials Regulation was dependent on the development of a fully functional clinical trials portal and database, which would be confirmed by an independent audit, and that the new legislation would come into effect six months after the European Commission publishes a notice of this confirmation. The website indicated that the audit was expected to commence in December 2021.

Parties conducting certain clinical studies must, as in the U.S., post clinical trial information in the European Union at the EudraCT website: <https://eudract.ema.europa.eu>.

PRIME designation

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

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

Pediatric Studies

Applicants developing a new medicinal product must agree upon a Pediatric Investigation Plan, or PIP, with the EMA's pediatric committee, or PDCO, and must conduct pediatric clinical trials in accordance with that PIP, unless a waiver applies (e.g., because the relevant disease or condition occurs only in adults). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which marketing authorization is being sought. The marketing authorization application for the product must include the results of pediatric clinical trials conducted in accordance with the PIP, unless a waiver applies, or a deferral has been granted by the PDCO of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults, in which case the pediatric clinical trials must be completed at a later date.

Marketing authorization

In the European Union, marketing authorizations for medicinal products may be obtained through several different procedures founded on the same basic regulatory process.

The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid across the European Economic Area (i.e. the EU as well as Iceland, Liechtenstein and Norway). The centralized procedure is compulsory for medicinal products produced by certain biotechnological processes, products designated as orphan medicinal products, and products with a new active substance indicated for the treatment of certain diseases. It is optional for those products that are highly innovative or for which a centralized process is in the interest of patients. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the CHMP. Accelerated evaluation may be granted by the CHMP in exceptional cases. These are defined as circumstances in which a medicinal product is expected to be of a "major public health interest." Three cumulative criteria must be fulfilled in such circumstances: the seriousness of the disease, such as severely disabling or life-threatening diseases, to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In these circumstances, the EMA ensures that the opinion of the CHMP is given within 150 days.

Specifically, the grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision, and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products, and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety, and efficacy of their products to EMA which provides an opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

The decentralized procedure provides for approval by one or more other concerned EU Member States of an assessment of an application for marketing authorization conducted by one EU Member State, known as the reference EU Member State. In accordance with this procedure, an applicant submits an application for marketing authorization to the reference EU Member State and the concerned EU Member States. This application is identical to the application that would be submitted to the EMA for authorization through the centralized procedure. The reference EU Member State prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. The resulting assessment report is submitted to the concerned EU Member States which, within 90 days of receipt, must decide whether to approve the assessment report and related materials.

If a concerned EU Member State cannot approve the assessment report and related materials due to concerns relating to a potential serious risk to public health, disputed elements may be referred to the European Commission, whose decision is binding on all EU Member States. In accordance with the mutual recognition procedure, the sponsor applies for national marketing authorization in one EU Member State. Upon receipt of this authorization the sponsor can then seek the recognition of this authorization by other EU Member States. Authorization in accordance with either of these procedures will result in authorization of the medicinal product only in the reference EU Member State and in the other concerned EU Member States.

A marketing authorization may be granted only to an applicant established in the European Union. Regulation No. 1901/2006 provides that, prior to obtaining a marketing authorization in the European Union, an applicant must demonstrate compliance with all measures included in a Pediatric Investigation Plan, or PIP, approved by the Pediatric Committee of the EMA, covering all subsets of the pediatric population, unless the EMA has granted a product-specific waiver, class waiver, or a deferral for one or more of the measures included in the PIP.

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 authorization

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).

Specialized Procedures for Gene Therapies

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

General Data Protection Regulation

The collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the EU, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, which became effective on May 25, 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR also imposes strict rules on the transfer of personal data to countries outside the EU, including the U.S., and permits data protection authorities to impose large penalties for violations of the GDPR, including potential fines of up to €20 million or 4% of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. Compliance with the GDPR will be a rigorous and time-intensive process that may increase the cost of doing business or require companies to change their business practices to ensure full compliance.

Pediatric Exclusivity

Products that are granted a marketing authorization with the results of the pediatric clinical trials conducted in accordance with the PIP are eligible for a six month extension of the protection under a supplementary protection certificate (if any is in effect at the time of approval) even where the trial results are negative. In the case of orphan medicinal products, a two-year extension of the orphan market exclusivity may be available. This pediatric reward is subject to specific conditions and is not automatically available when data in compliance with the PIP are developed and submitted.

Brexit and the Regulatory Framework in the United Kingdom

On June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the EU, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the EU on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020 (extendable by up to two years). On December 24, 2020, the United Kingdom and the European Union entered into a Trade and Cooperation Agreement. The agreement sets out certain procedures for approval and recognition of medical products in each jurisdiction. Since the regulatory framework for pharmaceutical products in the United Kingdom covering quality, safety and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales and distribution of pharmaceutical products is derived from EU directives and regulations, Brexit could materially impact the future regulatory regime which applies to products and the approval of product candidates in the United Kingdom, as the United Kingdom legislation now has the potential to diverge from EU legislation. It remains to be seen how Brexit will impact regulatory requirements for product candidates and products in the United Kingdom in the long-term. The United Kingdom Medicines and Healthcare products Regulatory Agency has recently published detailed guidance for industry and organizations to follow from January 1, 2021 now the transition period is over, which will be updated as the United Kingdom's regulatory position on medicinal products evolves over time.

Furthermore, while the Data Protection Act of 2018 in the United Kingdom that "implements" and complements the GDPR, achieved Royal Assent on May 23, 2018 and is now effective in the United Kingdom, it is still unclear whether transfer of data from the European Economic Area, or EEA, to the United Kingdom will remain lawful under the GDPR. The Trade and Cooperation Agreement provides for a transitional period during which the United Kingdom will be treated like a European Union member state in relation to processing and transfers of personal data for four months from January 1, 2021. This may be extended by two further months. After such period, the United Kingdom will be a "third country" under the GDPR unless the European Commission adopts an adequacy decision in respect of transfers of personal data to the United Kingdom. The United Kingdom has already determined that it considers all of the 27 EU member states and EEA member states to be adequate for the purposes of data protection, ensuring that data flows from the United Kingdom to the EU/EEA remain unaffected.

Healthcare law and regulation

Healthcare providers and third-party payors play a primary role in the recommendation and prescription of products that are granted marketing approval. Arrangements with providers, consultants, third-party payors and customers are subject to broadly applicable fraud and abuse, anti-kickback, false claims laws, reporting of payments to physicians and teaching physicians and patient privacy laws and regulations and other healthcare laws and regulations that may constrain business and/or financial arrangements. Restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, paying, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;
- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false, fictitious or fraudulent or knowingly making, using or causing to be made or used a false record or statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal laws that prohibit, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, and their respective implementing regulations, including the Final Omnibus Rule published in January 2013, which impose obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;
- the federal transparency requirements known as the federal Physician Payments Sunshine Act, under the Health Care Reform Law, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to the Centers for Medicare & Medicaid Services, or CMS, within the United States Department of Health and Human Services, information related to payments and other transfers of value made by that entity to physicians and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

Amendments to the Sunshine Act, which went into effect as of January 1, 2021 now includes as “covered recipients” physician assistants and advance practice registered nurses (nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and certified nurse midwives). Some state laws require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government in addition to requiring manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Pharmaceutical insurance coverage and health care reform

In the United States and markets in other countries, patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated health care costs. Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Thus, even if a product candidate is approved, sales of the product will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage and establish adequate reimbursement levels for, the product. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged, examining the medical necessity and reviewing the cost-effectiveness of medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the approved products for a particular indication.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the Health Care Reform Law, which, among other things, includes changes to the coverage and payment for products under government health care programs. In addition, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2030 under the Coronavirus Aid, Relief, and Economic Security Act. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Additionally, the 2020 federal spending package permanently eliminated, effective January 1, 2020, the Health Care Reform Law-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and, effective January 1, 2021, also eliminates the health insurer tax. Further, the Bipartisan Budget Act of 2018, among other things, amended the Health Care Reform Law, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole." These laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, which was signed by President Trump on December 22, 2017, Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the Health Care Reform Law is an essential and inseparable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the Health Care Reform Law are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit court affirmed the lower court's ruling that the individual mandate portion of the Health Care Reform Law is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the Health Care Reform Law. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On February 10, 2021, the Biden Administration withdrew the United States Department of Justice's support for this lawsuit. A ruling by the Court is expected sometime this year. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

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

The costs of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. To those ends, President Trump issued five executive orders intended to lower the costs of prescription drug products but it is unclear whether, and to what extent, these orders will remain in force under the Biden Administration. Further, on September 24, 2020, the Trump Administration finalized a rulemaking allowing states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants are required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. The FDA has issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

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

Environmental regulations

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

Human Capital

In January 2020, we announced a reduction in workforce by approximately one third as part of a strategic plan designed to create a leaner company focused on advancing SGT-001. As of December 31, 2020, we employed 70 full-time employees, including 55 in research and development and 15 in general and administrative positions. Twenty of our employees hold Ph.D. or M.D. degrees.

We recognize that attracting, motivating and retaining talented employees is vital to our success. We value the health and wellness of our employees and their families. It is our goal to deliver innovative programs that provide choice, quality and value. We aim to create an equitable, inclusive and empowering environment in which our employees can grow and advance their careers, with the overall goal of developing, expanding and retaining our workforce to support our current pipeline and future business goals. Our success also depends on our ability to attract, engage and retain a diverse group of employees. Our efforts to recruit and retain a diverse and passionate workforce include providing competitive compensation and benefits packages.

Our human capital resources objectives include, as applicable, identifying, recruiting, retaining, incentivizing and integrating our existing and additional employees. The principal purposes of our equity incentive plans are to attract, retain and motivate selected employees, consultants and directors through the granting of stock-based compensation awards. We offer a comprehensive benefits program that provides resources to help employees manage their health, finances, and life outside of work.

Corporate Information

Our principal executive offices are located at 141 Portland Street, Fifth Floor, Cambridge, Massachusetts 02139 and our telephone number is (617) 337-4680. Our website address is www.solidbio.com. The information contained in, or accessible through, our website does not constitute a part of this Annual Report on Form 10-K.

We make available free of charge on our website our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, or the Exchange Act, as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC.

Item 1A Risk Factors.

You should carefully consider the following risk factors, in addition to the other information contained in this annual report on Form 10-K, including the section of this report titled “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and our financial statements and related notes. If any of the events described in the following risk factors and the risks described elsewhere in this annual report on Form 10-K occurs, our business, operating results and financial condition could be seriously harmed and the trading price of our common stock could decline. This annual report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements as a result of factors that are described below and elsewhere in this annual report on Form 10-K.

Risks related to our financial position and need for capital requirements

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

Since inception, we have incurred significant net losses. Our net loss was \$88.3 million for the year ended December 31, 2020. Our net losses were \$117.2 million, \$74.8 million and \$53.2 million for the years ended December 31, 2019, 2018 and 2017, respectively. As of December 31, 2020, we had an accumulated deficit of \$404.6 million. As a result of our historical operating losses and expected future negative cash flows from operations, we have concluded that there is substantial doubt about our ability to continue as a going concern. To date, we have devoted substantially all of our efforts to research and development, including clinical development of our gene transfer product candidate, SGT-001, as well as to building out our management team and infrastructure. We expect that it could be several years, if ever, before we have a commercialized product. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if, and as, we:

- continue to enroll patients in IGNITE DMD and continue clinical development of SGT-001;
- move other product candidates into clinical trials;
- 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 activities;
- acquire or in-license other drugs, technologies and intellectual property;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to our collaboration and license agreement with Ultragenyx; and
- add operational, financial and management information systems and personnel.

To become and remain profitable, we must develop and eventually commercialize one or more product candidates with significant market potential. This will require us to be successful in a range of challenging activities, and our expenses will increase substantially as we seek to resume IGNITE DMD and 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.

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

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, conduct clinical trials of, and seek marketing approval for, SGT-001 and our other product candidates. In addition, if we obtain marketing approval for SGT-001 and our other product candidates, we expect to incur significant expenses related to product sales, marketing, manufacturing and distribution. We also incur additional costs associated with operating as a public company. While we believe that our cash and cash equivalents as of December 31, 2020 will be sufficient to fund our operating expenses and capital requirements into the first quarter of 2022, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate. In order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we anticipate that we will need additional funding to complete the development of SGT-001 and our other product candidates.

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

- the progress and results of IGNITE DMD and future clinical trials of SGT-001 and our other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001 and our other product candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for other product candidates that we may pursue in the future, if any;
- the costs associated with our manufacturing process development and evaluation of third-party manufacturers;
- whether we decide to construct and validate our own manufacturing facility and the associated costs;
- revenue, if any, received from commercial sale of SGT-001 or our other product candidates, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining, defending and enforcing our intellectual property rights and defending intellectual property-related claims;
- the outcome of any lawsuits filed against us;
- the terms of our current and any future license agreements and collaborations;
- the success of our collaboration with Ultragenyx;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other product candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to the COVID-19 pandemic and its collateral consequences.

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

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

The report from our independent registered public accounting firm for the year ended December 31, 2020 includes an explanatory paragraph stating that our losses from operations and required additional funding to finance our operations raise substantial doubt about our ability to continue as a going concern. If we are unable to obtain sufficient funding, our business, prospects, financial condition and results of operations will be materially and adversely affected and we may be unable to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited financial statements, and it is likely that investors will lose all or a part of their investment. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding to us on commercially reasonable terms or at all.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies, SGT-001 or our other product candidates.

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, ownership of our common stock will be diluted and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and could involve restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, SGT-001 or our other product candidates, or grant licenses on terms unfavorable to us.

We have never generated revenue from product sales and do not expect to do so for the next several years, if ever.

Our ability to generate revenue from product sales and achieve profitability depends on our ability, alone or with collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, SGT-001 and any other product candidates that we may pursue in the future. We do not anticipate generating revenue from product sales for the next several years, if ever. Our ability to generate future revenue from product sales depends heavily on our success in:

- completing research and development of SGT-001 and our other product candidates in a timely and successful manner;
- seeking and obtaining regulatory and marketing approvals for any product candidates for which we complete clinical trials;
- launching and commercializing SGT-001 and any other product candidates for which we obtain regulatory and marketing approval by establishing a sales force and marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- maintaining and enhancing a commercially viable, sustainable, scalable, reproducible and transferable manufacturing process for SGT-001 and our other product candidates that is compliant with cGMPs;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the commercial demand for SGT-001 and our other product candidates, if approved;

- obtaining market acceptance, if and when approved, of SGT-001 or any other product candidate as a viable treatment option by patients, the medical community and third-party payors;
- qualifying for coverage and adequate reimbursement by government and third-party payors for SGT-001 and our other product candidates both in the U.S. and internationally;
- effectively addressing any competing technological and market developments;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations under such arrangements;
- maintaining, protecting, enforcing and expanding our portfolio of intellectual property rights, including patents, trademarks, trade secrets and know-how;
- avoiding and defending against intellectual property infringement, misappropriation and other claims;
- implementing additional internal systems and infrastructure, as needed; and
- attracting, hiring and retaining qualified personnel.

Our limited operating history may make it difficult for our stockholders to evaluate the success of our business to date and to assess our future viability.

We are a development-stage company founded in 2013. Our operations to date, with respect to the development of SGT-001 and other potential product candidates, have been limited to organizing and staffing our company, business planning, raising capital, acquiring rights to our technology, identifying SGT-001 as a potential gene transfer product candidate and undertaking preclinical studies and a clinical trial of that product candidate and establishing research and development and manufacturing collaborations. We have not yet demonstrated the ability to complete clinical trials of SGT-001 or any other product candidate, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions our stockholders make about our prospects may not be as accurate as they could be if we had a longer operating history.

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

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

We and our third-party manufacturers for our SGT-001 supply and prospective contract research organizations, or CROs, may face disruptions that may affect our ability to initiate, resume and complete preclinical studies or clinical trials, including disruptions in procuring items that are essential for our research and development activities, including, for example, raw materials used in the manufacturing of our product candidates, and laboratory supplies for our current and future preclinical studies and clinical trials, in each case, for which there may be shortages because of ongoing efforts to address the outbreak. We and our third-party manufacturers and prospective CROs, may face disruptions related to IGNITE DMD or future clinical trials arising from delays in IND-enabling studies, manufacturing disruptions, and the ability to obtain necessary institutional review board or other necessary site approvals, as well as other delays at clinical trial sites.

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

The response to the COVID-19 pandemic may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. For example, the FDA has announced that in order to bring new therapies to patients sick with COVID-19 as quickly as possible, it has redeployed medical and regulatory staff from other areas to work on COVID-19 therapies. In addition, we may face impediments to regulatory meetings and approvals due to measures intended to limit in-person interactions.

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

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

Risks related to the development of our product candidates

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

In November 2019, the FDA placed a clinical hold on SGT-001 following a serious adverse event in IGNITE DMD. The third patient in the 2E14 vg/kg cohort of IGNITE DMD, dosed in late October 2019, experienced a serious adverse event deemed related to the study drug that was characterized by complement activation, thrombocytopenia, decrease in red blood cell count, acute kidney injury, and cardio-pulmonary insufficiency. In October 2020, the FDA lifted the clinical hold placed on IGNITE DMD. In connection with the lifting of the clinical hold, we are reducing the maximum weight of the next two patients dosed in IGNITE DMD to 18 kg per patient. Additionally, to mitigate the risk of serious drug-related adverse events, we have amended the IGNITE DMD clinical protocol to include the prophylactic use of both anti-complement inhibitor eculizumab and C1 esterase inhibitor, and increase the prednisone dose in the first month post dosing; however, we cannot guarantee that similar events will not happen in the future. In addition, the use of dual complement inhibition as part of the risk mitigation strategy is untested in this disease and may pose additional, unknown risk. Even though the FDA lifted the clinical hold, additional preclinical studies or clinical trials involving SGT-001, further amendments to the SGT-001 enrollment criteria and/or clinical trial protocol or changes to our manufacturing process may be needed, which may prove difficult to implement and/or complete. In such instance, our progress in the development of SGT-001 may be significantly slowed or stopped and the associated costs may be significantly increased, adversely affecting our business, and our stock price would likely decline. Furthermore, even though the FDA lifted the clinical hold, we may nonetheless face difficulties in recruiting patients to enroll in or retaining patients in IGNITE DMD if patients or their caregivers are affected by the COVID-19 virus or are fearful of traveling to, or are unable to travel to, our clinical trial sites because of the COVID-19 pandemic.

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

SGT-001 is a gene transfer candidate based on a novel technology, which makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union.

We have concentrated our research and development efforts on SGT-001 for the treatment of Duchenne and our future success depends on our successful development of that product candidate. Our risk of failure is high. We have experienced, and may in the future experience, problems or delays in developing SGT-001. Any such problems or delays would cause unanticipated costs, and any development problems may not be solved. For example, we or another party may uncover a previously unknown risk associated with SGT-001, the adeno-associated virus, or AAV, vector, toxicity or other issues that may be more problematic than we currently believe and this may prolong the period of observation required for obtaining, or result in the failure to obtain, regulatory approval or may necessitate additional clinical testing.

In addition, the product specifications and the clinical trial requirements of the FDA, the European Commission, the European Medicines Agency, or the EMA, and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidate. The regulatory approval process for novel product candidates such as ours is unclear and can be more expensive and take longer than for other, better known or more extensively studied product candidates. To our knowledge, only a limited number of gene transfer products have been approved for commercialization in the United States and the European Union. As a result, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for SGT-001 in either the United States or the European Union. Approvals by the European Commission may not be indicative of what the FDA may require for approval and vice versa.

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

During the conduct of clinical trials, patients may experience changes in their health, including illnesses, injuries, discomforts or a fatal outcome. Often, it is not possible to determine whether the product candidate being studied caused these conditions. For instance, we reported a serious adverse event in IGNITE DMD, which resulted in a clinical hold in November 2019, which has since been resolved, and previously the FDA had placed IGNITE DMD on clinical hold after we reported another serious adverse event. In addition, it is possible that as we test SGT-001 or our other product candidates in larger, longer and more extensive clinical programs, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in earlier clinical trials, as well as conditions that did not occur or went undetected in previous clinical trials, will be reported by subjects. Many times, side effects are only detectable after investigational products are tested in large-scale, Phase III clinical trials or, in some cases, after they are made available to patients on a commercial scale after approval. If additional clinical experience indicates that SGT-001 or any other product candidate has side effects or causes serious or life-threatening side effects, the development of the product candidate may fail or be delayed, or, if the product candidate has received regulatory approval, such approval may be revoked.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia and death seen in other clinical trials using other vectors. While new recombinant vectors have been developed with the intent to reduce these side effects, gene therapy is still a relatively new approach to disease treatment and additional adverse side effects could develop. Patients will create antibodies to the AAV vector and a second administration of gene transfer might not be successful. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that may occur with treatment with gene therapy products include an immunologic reaction early after administration that could substantially limit the effectiveness of the treatment or represent safety risks for patients. Additionally, in previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a positive ELISPOT test associated with T-cell responses, which is of unclear clinical translatability. If T-cells are activated, the cellular immune response system may trigger the removal of transduced cells. If our gene transfer candidate demonstrates a similar effect, we may decide or be required to halt or delay further clinical development of SGT-001.

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

In addition to side effects caused by SGT-001 and our other product candidates, the administration process or related procedures also can cause adverse side effects. For example, integration of AAV DNA into the host cell's genome has been reported to occur. Further, our AAV delivery system has not been validated in human clinical trials previously, and if such delivery system does not meet the safety criteria or cannot provide the desired efficacy results, then we may be forced to suspend or terminate our development of SGT-001. In addition, the relatively high dosing requirements for SGT-001 may amplify the risk of adverse side effects relating to the AAV vector. When James M. Wilson, M.D., Ph.D., resigned from our Scientific Advisory Board in early 2018 he cited emerging concerns about the possible risks of high systemic dosing of AAV. If any such adverse side effects were to occur in the future and we are unable to demonstrate that they were not caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, SGT-001 or any other product candidate for any or all targeted indications. Even if we are able to demonstrate that any serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the clinical trial.

Additionally, if SGT-001 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 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 limited experience in preparing, submitting and prosecuting regulatory filings, and have not previously submitted a biologics license application, or BLA, for any product candidate. We cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin or to begin as proposed, or that, once begun, issues will not arise that suspend or terminate such clinical trials. Carrying out later-stage clinical trials and the submission of a successful BLA is a complicated process. This may be particularly true for design of a pivotal trial for the treatment of Duchenne as the FDA has not given clear guidance as to the necessary endpoints for approval of a treatment for Duchenne. In addition, we cannot be certain how many clinical trials of SGT-001 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.

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

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

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

Preliminary or interim data that we announce or publish from time to time may change as more data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may announce or publish preliminary or interim data from clinical trials. Positive preliminary or interim data may not be predictive of such trial's subsequent or overall results. Preliminary or interim data are subject to the risk that one or more of the outcomes may materially change as more data become available. Additionally, preliminary or interim data are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available. Therefore, positive preliminary or interim data in any ongoing clinical trial may not be predictive of such results in the completed trial. We also make assumptions, estimations, calculations and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully evaluate all data. As a result, preliminary or interim data that we report may differ from future results from the same clinical trials, or different conclusions or considerations may qualify such results, once additional data have been received and fully evaluated. Preliminary or interim data also remain subject to audit and verification procedures that may result in the final data being materially different from the preliminary or interim data we previously published. As a result, preliminary or interim data should be viewed with caution until the final data are available. Material adverse changes in the final data compared to preliminary or interim data could significantly harm our business prospects.

We may encounter substantial delays in our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of SGT-001 or our other product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidate for its intended indications. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement with the appropriate external parties on dose escalation;
- delays in enrolling patients in IGNITE DMD for SGT-001;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent ethics committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials, including because such trials may be placebo-controlled trials and patients are not guaranteed to receive treatment with our product candidates;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;

- failure to perform in accordance with FDA good clinical practices, or GCPs, or applicable regulatory guidelines in the European Union and other countries;
- delays in the testing, validation, manufacturing and delivery of SGT-001 or our other product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in subjects completing participation in a trial or returning for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations, trial sites or manufacturing facilities;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors;
- delays as a result of the COVID-19 pandemic or from the outbreak of another pandemic or contagious disease or other global instability could delay IGNITE DMD, or the commencement or rate of completion of any other clinical trial; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Additionally, if the results of any clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with SGT-001 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 our products, if approved, are administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified REMS;
- be sued and held liable for harm caused to patients; or
- experience damage to our reputation.

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

If our third-party clinical trial vendors fail to comply with strict regulations, the clinical trials for SGT-001 or our other product candidates may be delayed or unsuccessful.

We do not have the personnel capacity to conduct or manage the clinical trials that will be necessary for the development of SGT-001 or our other product candidates. For IGNITE DMD we are relying, and for any future clinical trials we expect we will rely, on third parties to assist us in managing, monitoring and conducting our clinical trials. If these third parties fail to comply with applicable regulations or do not adequately fulfill their obligations under the terms of our agreements with them, we may not be able to enter into alternative arrangements without undue delay or additional expenditures and, therefore, the clinical trials for SGT-001 or our other product candidates may be delayed or unsuccessful.

Furthermore, the FDA can be expected to inspect some or all of the clinical sites participating in our clinical trials to determine if our clinical trials are being conducted according to GCPs. If the FDA determines that these clinical sites are not in compliance with applicable regulations, we may be required to delay, repeat or terminate the clinical trials.

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent us from proceeding with clinical trials of SGT-001 or our other product candidates.

Identifying and qualifying patients to participate in any clinical trials of SGT-001 and our other product candidates is critical to our success. The timing of any clinical trials depends on our ability to recruit patients to participate as well as complete required follow-up periods. If patients are unwilling or unable to participate in our gene therapy clinical trials, including because of negative publicity from adverse events related to our product candidates, other approved gene therapies or the biotechnology or gene therapy fields, or due to competitive clinical trials for similar patient populations, clinical trials in products employing our vector or our platform or for other reasons, the timeline for recruiting patients, conducting clinical trials and obtaining regulatory approval of SGT-001 may be delayed. We may also experience delays if patients withdraw from the clinical trial or do not complete the required monitoring period. Furthermore, we may face difficulties in recruiting patients to enroll in, or retaining patients in, IGNITE DMD if they or their caretakers are affected by the COVID-19 virus or are fearful of traveling to, or are unable to travel to, our clinical trial sites because of the COVID-19 pandemic. These delays could result in increased costs, delays in advancing SGT-001 or our other product candidates, delays in testing the effectiveness of SGT-001 and our other product candidates or termination of clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete any clinical trials in a timely manner. Patient enrollment and trial completion is affected by many factors, including:

- size of the patient population and the process for identifying subjects;
- design of the trial protocol;
- eligibility and exclusion criteria, including that some patients may have pre-existing antibodies to AAV vectors precluding them from being able to receive AAV-mediated gene transfer;
- restrictions on our ability to conduct clinical trials, including full and partial clinical holds on ongoing or planned clinical trials;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of gene therapy-based approaches to the treatment of diseases;
- release or disclosure of data from our completed or ongoing clinical trials;
- availability of competing therapies and clinical trials;
- severity of the disease;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians;
- ability to monitor subjects adequately during and after treatment; and
- in the case of pivotal trials, the risk that patients may opt not to enroll because they are not assured treatment with our product candidate.

In November 2019, the FDA placed our IGNITE DMD clinical trial of SGT-001 on clinical hold following our report of a serious adverse event in the clinical trial. In April 2020, we submitted a response to the FDA, that included changes to the clinical protocol designed to potentially enhance patient safety, as well as information related to improvements to our manufacturing process. The FDA responded by maintaining the clinical hold and requesting further data and analyses relating to this manufacturing process. In June 2020, we submitted a response to the FDA that provided data and analyses related to improvements to our manufacturing process. In July 2020, we announced that the FDA responded by maintaining the clinical hold and requesting further manufacturing information and updated safety and efficacy data for all patients dosed in the trial, as well as providing direction on the total viral load to be administered per patient. In October 2020, we announced that the FDA lifted the clinical hold placed on the IGNITE DMD clinical trial. In connection with the lifting of the clinical hold, we are reducing the maximum weight of the next two patients dosed in IGNITE DMD to 18 kg per patient, and we may face

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

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

Even if we complete the necessary clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize SGT-001 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.

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

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.

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions and warnings on the labeling or marketing of a product;
- restrictions on product distribution or use;

- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- damage to relationships with any potential collaborators;
- unfavorable press coverage and damage to our reputation;
- refusal to permit the import or export of our products;
- product seizure;
- injunctions or the imposition of civil or criminal penalties; or
- litigation involving patients using our products. Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

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

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

Even if we receive FDA approval of SGT-001 or our other product candidates in the United States, approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Future sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials, manufacturing and marketing approval. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. We intend to submit a marketing authorization application, or MAA, to the EMA for approval of SGT-001 in the European Union, but obtaining such approval from the European Commission following the opinion of the EMA is a lengthy and expensive process. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of SGT-001 or our other product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for SGT-001 or our other product candidates may be withdrawn. If we fail to comply with the regulatory

requirements, our target market will be reduced, and our ability to realize the full market potential of our product candidates will be harmed.

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

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

The FDA has issued various guidance documents regarding gene therapies, including recent final guidance documents released in January 2020 relating to chemistry, manufacturing and controls information for gene therapy INDs, gene therapies for rare diseases and gene therapies for retinal disorders. Although the FDA has indicated that these and other guidance documents it previously issued are not legally binding, we believe that our compliance with them is likely necessary to gain approval for any gene therapy product candidate we may develop. The guidance documents provide additional factors that the FDA will consider at each of the above stages of development and relate to, among other things, the proper preclinical assessment of gene therapies; the chemistry, manufacturing, and control information that should be included in an IND application; the proper design of tests to measure product potency in support of an IND or BLA application; and measures to observe delayed adverse effects in subjects who have been exposed to investigational gene therapies when the risk of such effects is high. Further, the FDA usually recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by 10 years of annual queries, either in person or by questionnaire.

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

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

Finally, ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed their intentions to further regulate biotechnology. More restrictive regulations or claims that our product candidates are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under

development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

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

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

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

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

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

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

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

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

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

We may seek approval of SGT-001 or our other product candidates using the FDA’s accelerated approval pathway. A product may be eligible for accelerated approval if it treats a serious or life-threatening condition and generally provides a meaningful advantage over available therapies. In addition, it must demonstrate an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity

or mortality, or IMM, that is reasonably likely to predict an effect on IMM or other clinical benefit. The FDA or other applicable regulatory agency makes the determination regarding whether a surrogate endpoint is reasonably likely to predict long-term clinical benefit. Given that expression of microdystrophin has not yet been established to predict long-term clinical benefit, it is not currently accepted, and it is possible the FDA and/or other applicable regulatory agencies could decide never to accept it, as a surrogate endpoint for the accelerated approval pathway.

As a condition of approval, the FDA may require that a sponsor of a drug or biologic product candidate receiving accelerated approval perform adequate and well-controlled post-marketing clinical trials. These confirmatory trials must be completed with due diligence. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product.

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

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

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

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

The FDA has granted Rare Pediatric Disease Designation, or RPDD, to SGT-001; however, a BLA for SGT-001 may not meet the eligibility criteria for a priority review voucher upon approval.

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

For the purposes of this program, a “rare pediatric disease” is a (a) serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years, including age groups often called neonates, infants, children, and adolescents; and (b) rare disease or conditions within the meaning of the Orphan Drug Act. The FDA has granted Rare Pediatric Disease designation to SGT-001. The FDA may determine, however, that a BLA

for SGT-001 or our other product candidates does not meet the eligibility criteria for a priority review voucher upon approval.

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

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

If a therapy is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a drug sponsor may apply for FDA fast track designation. The FDA has granted fast track designation to SGT-001; however, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with fast track designation compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

We may seek priority review designation for SGT-001 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 Duchenne. Many of our competitors have significantly greater financial, product candidate development, manufacturing and marketing resources than we do. Large pharmaceutical and biotechnology companies have extensive experience in clinical testing and obtaining regulatory approval for their products, and mergers and acquisitions within these industries may result in even more resources being concentrated among a smaller number of larger competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These third parties compete with us in recruiting and retaining qualified scientific and

management personnel, establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

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

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

We are aware of several companies focused on developing gene therapies in various indications, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against SGT-001 or any future gene therapy product candidates we develop.

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

The success of our business depends upon our ability to develop and commercialize SGT-001 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. For example, in January 2020, in connection with implementing our strategic plan to create a leaner company focused on advancing SGT-001, we curtailed certain activities supporting our other research and development programs.

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

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

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

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

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

While we have retained all rights to and are developing on our own SGT-001, we may in the future enter into development, distribution or marketing arrangements with third parties with respect to SGT-001 or future product candidates. Our likely collaborators for any such sales, marketing, distribution, development, licensing or broader collaboration arrangements include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. If we enter into any such arrangements with any third parties in the future, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities and efforts to successfully perform the functions assigned to them in these arrangements.

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

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

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

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

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

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

We have limited gene transfer manufacturing experience and could experience production problems and delays in obtaining regulatory approval of our manufacturing processes, which could result in delays in the development or commercialization of SGT-001 or our other product candidates.

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

We currently rely on third-party manufacturers for our SGT-001 supply. In order to produce sufficient quantities of SGT-001 for clinical trials and initial U.S. commercial demand, we continue to further optimize and increase the capacity of our manufacturing process at our third-party manufacturer, and potentially through our own commercial-scale manufacturing facility. We may need to change our current manufacturing process. We may not be able to produce sufficient quantities of SGT-001 due to several factors, including equipment malfunctions, facility contamination, material shortages or

contamination, natural disasters, a public health issue (for example, an outbreak of a contagious disease such as the COVID-19 pandemic), disruption in utility services, human error or disruptions in the operations of our suppliers. For example, through our contract manufacturer we have performed and released within specifications manufacturing runs of SGT-001 for clinical supply and have experienced variability with respect to the success and yield of these runs. We continue to engage in process development activities to improve the reproducibility, reliability, quality and consistency of yields of our manufacturing process. While we are able to produce for more than one patient from a single batch, additional manufacturing runs will be required to produce necessary or adequate supply for IGNITE DMD and there is no guarantee that all of those runs will be within specifications or produce adequate supply. If we are not able to produce sufficient supply on the timeline expected, our overall development schedule for SGT-001 could be delayed, and we could incur additional expense.

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

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

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

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

Any problems in our manufacturing process or facilities could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in our manufacturing process or facilities also could restrict our ability to meet market demand for SGT-001, our other product candidates or future product candidates.

Although we may establish our own SGT-001 manufacturing facility, we expect to utilize third parties to conduct our product manufacturing for the foreseeable future. Therefore, we are subject to the risk that these third parties may not perform satisfactorily or meet regulatory requirements.

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

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

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

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

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval or impact our ability to successfully commercialize SGT-001 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 Duchenne. Our understanding of the patient population with this disease is based on estimates in published literature and by Duchenne foundations. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of this disease. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our product candidate or patients may become increasingly difficult to identify and access.

Further, there are several factors that could contribute to making the actual number of patients who receive SGT-001 less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a degenerative disease such as Duchenne up to the time of treatment will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell damage.

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

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

The commercial success of any of our product candidates, including SGT-001, if approved, will depend upon market acceptance by physicians, patients, third-party payors and others in the medical community.

Even with the requisite approvals from the FDA in the United States, the European Commission in the European Union and other regulatory authorities internationally, the commercial success of SGT-001 will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and SGT-001 in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community due to ethical, social, medical and legal concerns. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, SGT-001, if approved for commercial sale, will depend on multiple factors, including:

- the efficacy and safety of SGT-001 as demonstrated in clinical trials;
- the efficacy and potential and perceived advantages of SGT-001 over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which SGT-001 is approved by the FDA or the European Commission;
- the willingness of physicians to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;

- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the availability of products to meet market demand;
- publicity concerning our product candidates or competing products and treatments;
- any restrictions on the use of our products together with other medications; and
- favorable third-party payor coverage and adequate reimbursement.

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

Our efforts to educate the medical community and third-party payors on the benefits of SGT-001 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 Duchenne prescribing treatments that involve the use of SGT-001 in lieu of, or in addition to, other treatments with which they are more familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion may delay or impair the development and commercialization of SGT-001 or demand for any product candidate we may develop. A public backlash developed against gene therapy following the death of a patient in 1999 during a gene therapy clinical trial of research subjects with ornithine transcarbamylase, or OTC, deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene. The death of the clinical trial subject was due to complications of adenovirus vector administration. Dr. James M. Wilson, former chair of our Scientific Advisory Board, was a co-investigator of the 1999 trial while he was Director of the Institute for Human Gene Therapy of the University of Pennsylvania. Serious adverse events in our clinical trials, including the events that led to the previously-lifted clinical holds on IGNITE DMD or other clinical trials involving gene transfer products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of SGT-001, stricter labeling requirements for SGT-001 if approved and a decrease in demand for SGT-001.

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

Given the nature of biologics manufacturing, there is a risk of contamination in our manufacturing processes. Any contamination could materially adversely affect our ability to produce SGT-001 on schedule and could cause reputational damage.

Some of the materials required in our manufacturing process are derived from biologic sources. Such materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of SGT-001 could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially and adversely affect our development timelines.

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

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We expect the cost of a single administration of gene transfer products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of SGT-001, if approved, will depend substantially, both domestically and abroad, on the extent to which the costs of SGT-001 will be paid by health maintenance, managed care, pharmacy benefit and similar health care management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

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

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize SGT-001 and our other product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

To our knowledge, only a limited number of gene transfer products have been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services, or the CMS, the agency responsible for administering the Medicaid program. It is difficult to predict what the CMS will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these types of products either in the United States or the European Union. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union member states and vice versa. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for SGT-001 and our other product candidates.

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

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

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

If we obtain approval to commercialize SGT-001 and our other product candidates outside of the United States, in particular in the European Union, a variety of risks associated with international operations could materially adversely affect our business.

We expect that we will be subject to additional risks in commercializing SGT-001 and our other product candidates outside the United States, including:

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

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

Additionally, we could face heightened risks with respect to seeking marketing approval in the United Kingdom as a result of the recent withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the United Kingdom and the European Union, the United Kingdom withdrew from the European Union, effective December 31, 2020. On December 24, 2020, the United Kingdom and European Union entered into a Trade and Cooperation Agreement. The agreement sets out certain procedures for approval and recognition of medical products in each jurisdiction. Since the regulatory framework for pharmaceutical products in the United Kingdom covering the quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing any product candidates in the United Kingdom or the European Union and would restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom or European Union for any product candidates, which could significantly and materially harm our business.

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

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

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;

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

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

Risks related to our business operations

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

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

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

Our strategic plan and the associated workforce reduction announced in January 2020 may not result in anticipated savings, could result in total costs and expenses that are greater than expected and could disrupt our business.

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

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

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

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

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

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

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

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

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

In addition to legislative changes resulting from the passage of the Health Care Reform Law, other legislative changes have been proposed and adopted since the Health Care Reform Law was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the Health Care Reform Law, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017, or the TCJA, Congress repealed the “individual mandate.” The repeal of this provision of the Health Care Reform Law, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseparable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the Health Care Reform Law are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit court affirmed the lower court’s ruling that the individual mandate portion of the Health Care Reform Law is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the Health Care Reform Law. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On February 10, 2021, the Biden Administration withdrew the federal government’s support for overturning the Health Care Reform Law. A ruling by the Court is expected sometime this year. It is unclear how this decision and any subsequent appeals and other efforts to repeal and replace the Health Care Reform Law will impact the Health Care Reform Law and our business. Litigation and legislation over the Health Care Reform Law are likely to continue, with unpredictable and uncertain results.

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

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

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

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

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

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for products. To those ends, President Trump issued several executive orders intended to lower the costs of prescription drug products. Certain of these orders are reflected in recently promulgated regulations, including an interim final rule implementing President Trump's most favored nation model, but such final rule is currently subject to a nationwide preliminary injunction. It remains to be seen whether these orders and resulting regulations will remain in force during the Biden Administration. Further, on September 24, 2020, the Trump Administration finalized a rulemaking allowing states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants are required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. The FDA has issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

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

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

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

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

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

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

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

If we obtain FDA approval for SGT-001 or our other product candidates and begin commercializing those products in the United States, our operations will be directly or indirectly through our prescribers, customers and purchasers, subject to various federal and state fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal laws and the Physician Payment Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal health care program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The Health Care Reform Law amended the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;
- federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. The Health Care Reform Law provides and recent government cases against pharmaceutical and medical device manufacturers support the view that Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- HIPAA, which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any health care benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act; Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;
- federal transparency laws, including the federal Physician Payment Sunshine Act, that require certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children’s Health Insurance Program, with specific exceptions, to report annually to the CMS information related to: (i) payments or other “transfers of value” made to physicians and teaching hospitals and (ii) ownership and investment interests held by physicians and their immediate family members;
- state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other health care providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states; and

- state and foreign laws that govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

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

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

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

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

Similar actions are either in place or under way in the United States. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act, which went into effect on January 1, 2020, is creating similar risks and obligations as those created by GDPR, though the Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects (the Common Rule). Many other states are considering similar legislation. A broad range of legislative measures also have been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with such requirements is rigorous and time intensive and requires significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations. Similarly, failure to comply with federal and state laws regarding privacy and security of personal information could expose us to fines and penalties under such laws. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

Risks related to our intellectual property

We heavily rely on certain in-licensed patents and other intellectual property rights in connection with our development of SGT-001 and may be required to acquire or license additional patents or other intellectual property rights to continue to develop and commercialize SGT-001.

Our ability to develop and commercialize SGT-001 and other product candidates is heavily dependent on licenses to patent rights and other intellectual property granted to us by third parties. In particular, we have licensed certain patents and patent applications from the University of Michigan, the University of Missouri and the University of Washington that are important or necessary to the development of SGT-001 and other elements of our gene transfer program. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization obligations, milestone payments, royalties and other obligations on us. If we fail to comply with our obligations under these agreements, we may be subject to damages, which may be significant, and the licensor may have the right to terminate the license, in which event we may not be able to develop or market product candidates or technologies covered by the license, including SGT-001. In addition, certain of these license agreements are not assignable by us without the consent of the respective licensor, which may have an adverse effect on our ability to engage in certain transactions.

Under our existing license agreements, we do not have, and under future license agreements we may not have, the right to control the preparation, filing and prosecution of patent applications, or the maintenance, enforcement and defense of the patents and patent applications that we license from third parties. For example, under our inbound license agreements with the University of Michigan, the University of Missouri and the University of Washington, each of the applicable licensors controls the prosecution of patent applications and the maintenance of patents and patent applications. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained, enforced and defended in a manner consistent with the best interests of our business. If our licensors fail to maintain, enforce or defend such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our product candidates that are the subject of such licensed rights, including SGT-001, could be adversely affected. For more information, see Part I, Item 1, "Business—Strategic partnerships and collaborations/licenses" of this Annual Report on Form 10-K.

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

several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third-party intellectual property rights on terms that would allow us to make an appropriate return on our investment. Moreover, even if we are able to obtain such licenses, they may only be non-exclusive, which could permit competitors and other third parties to use the same intellectual property in competition with us.

We may collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. These institutions may provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Regardless of such option, we may be unable to negotiate a license within the required timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights to any third-party intellectual property rights that are required for the development and commercialization of SGT-001 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, although we have pending patent applications in the United States and abroad, we cannot predict whether or in which jurisdictions the pending applications will result in issuance of patents that effectively protect any of our product candidates or will effectively prevent others from commercializing competitive products. Further, each of the provisional patent applications is not eligible to become an issued patent until, among other things, we file a non-provisional patent application within 12 months of the filing date of each provisional patent application. If we do not timely file a non-provisional patent application in respect of a provisional patent application, we may lose our priority date with respect to such provisional patent application and any patent protection on the inventions disclosed in such provisional patent application. While we intend to timely file non-provisional patent applications relating to our provisional patent applications, we cannot predict whether such future patent applications will result in the issuance of patents that effectively protect any of our product candidates or will effectively prevent others from commercializing competitive products.

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

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

It is also currently unknown what claims may, if ever, issue from pending applications included in our patent rights. Additionally, certain of our in-licensed U.S. patent rights lack corresponding foreign patents or patent applications, and therefore we will be unable to obtain patent protection for our product candidates in certain jurisdictions. We or our licensors may not be able to obtain or maintain patent protection with respect to SGT-001 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 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. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such proceedings could result in the revocation or cancellation of or amendment to our licensed patents and any patents we may own in the future in such a way that they no longer cover SGT-001 or our other product candidates.

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

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

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

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

We currently depend, and will continue to depend, on our license, collaboration and other similar agreements. Further development and commercialization of SGT-001 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.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and “gene patents” have been decided by the Supreme Court of the United States, or the Supreme Court. On March 20, 2012, the Supreme Court issued a decision in *Mayo Collaborative Services v. Prometheus Laboratories, Inc.*, or *Prometheus*, a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the Supreme Court, the addition of well understood, routine or conventional activity such as “administering” or “determining” steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On July 3, 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the patent claim amounts to significantly more than the natural principle itself should be rejected as directed to patent-ineligible subject matter. On June 13, 2013, the Supreme Court issued its decision in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, or *Myriad*, a case involving patent claims held by Myriad Genetics, Inc. relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent-eligible subject matter, but that complementary DNA may be patent-eligible.

In 2014, the USPTO issued a guidance to its patent examiners for evaluating claims for patent subject matter eligibility under the relevant statute (35 U.S.C. § 101). This guidance was in response to a series of decisions from the U.S. Supreme Court on patent claims reciting judicial exceptions, including Abstract Ideas, Laws of Nature/Natural Principles, Natural Phenomena and/or Natural Products. Based on judicial decisions and public feedback, several supplements to this guidance and additional memoranda and materials have since been issued and are continually being issued, while the current eligibility guidance has been incorporated into the latest (9th) edition of the MPEP (Manual for Patent Examination Procedure), last revised in January 2018. The current subject matter eligibility guideline instructs USPTO examiners to follow a two-part test, set forth in the U.S. Supreme Court decisions *Alice/Mayo*, as the only test that should be used to evaluate the eligibility of claims under examination, including claims directed to natural products and principles including all naturally occurring nucleic acids. Certain claims of our licensed patents and patent applications contain, and any future patents we may obtain may contain, claims that relate to specific recombinant DNA sequences that are naturally occurring at least in part and, therefore, could be the subject of future challenges made by third parties. In addition, the current USPTO subject matter eligibility guidance and the constantly evolving case law, together with contemplated congressional action, could all impact our ability to pursue similar patent claims in patent applications we may prosecute in the future.

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

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

If we do not obtain patent term extension for patents relating to SGT-001 or our other product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of SGT-001 and our other product candidates, one or more U.S. patents that we license or may own in the future may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process based on the first regulatory approval for a particular drug or biologic. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. In addition, to the extent we wish to pursue patent term extension based on a patent that we in-license from a third party, we would need the cooperation of that third party. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may be able to enter the market sooner.

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

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

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

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

- others may be able to make gene therapy products that are similar to our product candidates but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our current or future license partners or collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent applications that we license or may own in the future;
- we, or our current and future license partners or collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative products or duplicate any of our processes without infringing our owned or licensed intellectual property rights;

- others may circumvent our regulatory exclusivities, such as by pursuing approval of a competitive product candidate via the traditional approval pathway based on their own clinical data, rather than relying on the abbreviated pathway provided for biosimilar applicants;
- it is possible that our pending licensed patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to now or in the future may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- others may have access to the same intellectual property rights licensed to us;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents or other intellectual property rights of others may have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know how, and a third party may subsequently file a patent covering such intellectual property.

Risks related to ownership of our common stock

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

Our executive officers and directors and principal stockholders, in the aggregate, own shares representing approximately 40.9% of our capital stock as of February 16, 2021. As a result, if these stockholders were to choose to act together, they would be able to control or significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

This concentration of voting power may:

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

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

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

In October 2020, in connection with the execution of our collaboration and license agreement with Ultragenyx, we issued and sold 7,825,797 shares of our common stock to Ultragenyx. Following the expiration of an 18-month lock-up period and for the ten year period after date of such sale, subject to specified conditions, we have agreed to file a registration statement in order to register all or a portion of the shares sold to Ultragenyx.

In July 2019 and December 2020, we completed private placements of shares of our common stock and pre-funded warrants to purchase shares of our common stock to several accredited investors. We have filed, or have agreed to file, a registration statement covering the resale of these shares by the purchasers in these private placements and have agreed to keep such registration statements effective until the date the shares covered by the respective registration statement have been sold or can be resold without restriction under Rule 144 of the Securities Act.

In addition, we have filed registration statements registering all shares of common stock that we may issue under our equity compensation plans. These shares can be freely sold in the public market upon issuance, subject to black-out periods and volume limitations applicable to affiliates.

We currently have on file with the SEC a universal shelf registration statement which allows us to offer and sell registered common stock, preferred stock, debt securities, depository shares, warrants and/or units from time to time pursuant to one or more offerings at prices and terms to be determined at the time of sale.

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

Our stock price has been, and in the future is likely to be, volatile. The stock market in general and the market for biopharmaceutical or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price they paid for their shares. The market price for our common stock may be influenced by many factors, including:

- results of or developments in preclinical studies and clinical trials of SGT-001 or our other product candidates or those of our competitors;
- the success of competitive products or technologies;
- the effect of the COVID-19 pandemic on both the healthcare system and the patient population;
- regulatory or legal developments in the United States, the European Union and other countries;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates, or our clinical development programs and our commercialization efforts;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in our development timelines;
- our ability to raise additional capital;
- our inability to obtain or delays in obtaining adequate product supply for any approved product or inability to do so at acceptable prices;
- disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates;
- significant lawsuits, including patent or stockholder litigation;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of health care payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this “Risk Factors” section.

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

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

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

Prior to our initial public offering, which occurred on January 26, 2018, there was no public market for our common stock. Although our common stock is listed on the Nasdaq Global Select Market, given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for our stockholders to sell shares without depressing the market price for the shares, or at all.

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

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

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

We are also a smaller reporting company, and we will remain a smaller reporting company until the fiscal year following the determination that our voting and non-voting common stock held by non-affiliates is more than \$250 million measured on the last business day of our second fiscal quarter, or our annual revenues are less than \$100 million during the most recently completed fiscal year and our voting and non-voting common stock held by non-affiliates is more than \$700 million measured on the last business day of our second fiscal quarter. Similar to emerging growth companies, smaller reporting companies are able to provide simplified executive compensation disclosure and have certain other reduced disclosure obligations, including, among other things, being permitted to provide only two years of audited financial statements, with correspondingly reduced "Management's Discussion and Analysis of Financial Condition and Results of Operations"; not being required to furnish a contractual obligations table in "Management's Discussion and Analysis of Financial Condition and Results of Operations"; and not being required to furnish a stock performance graph in our annual report.

We may choose to take advantage of some, but not all, of the available exemptions. We have taken advantage of reduced reporting burdens in our filings with the Securities and Exchange Commission. In particular, we have not included all of the executive compensation information that would be required if we were not an EGC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

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

As a public company, and particularly after we are no longer an EGC, we incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act and rules subsequently implemented by the SEC and Nasdaq have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance.

Pursuant to Section 404, we are required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC or a smaller reporting company with less than \$100 million in revenue, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Provisions in our certificate of incorporation and our bylaws and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Provisions in our certificate of incorporation and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of our board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, or the DGCL, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, is the only sole source of gain for an investment in our common stock.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be the sole source of gain for an investor for the foreseeable future.

Our certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for such disputes with us or our directors, officers or employees.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware is the exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim for breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the DGCL, our certificate of incorporation or our bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine. We do not intend to have this choice of forum provision apply to, and this choice of forum provision will not apply to, actions arising under the Securities Act or the Exchange Act. The choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. Alternatively, if a court were to find the choice of forum provision contained in our certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

Item 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

We lease our corporate headquarters, which consists of approximately 16,000 square feet in Cambridge, Massachusetts. The lease for our corporate headquarters has an initial term of approximately 4 years that expires in February 2022. In addition, we lease our primary laboratory space, which consists of 9,500 square feet in Cambridge, Massachusetts, under a lease with an initial term of five years that expires in April 2023 and includes an option to extend for one additional two-year term. In addition, we lease smaller laboratory and office space.

Item 3. Legal Proceedings.

None.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

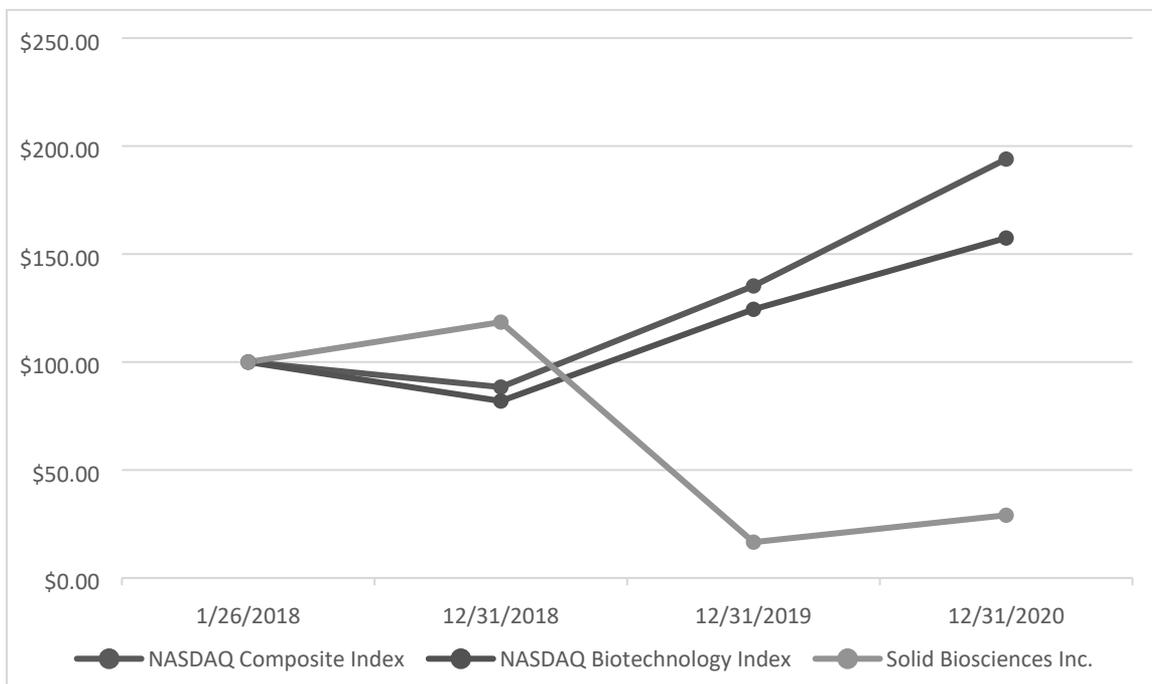
Item 5. Market for Registrant’s Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities.

Market Information

Our common stock has been publicly traded on the Nasdaq Global Select Market under the symbol “SLDB” since January 26, 2018 in connection with our initial public offering. Prior to that date, there was no established public trading market for our common stock.

The following graph compares the performance of our common stock to The Nasdaq Composite Index and to The Nasdaq Biotechnology Index from January 26, 2018 (the first date that shares of our common stock were publicly traded) through December 31, 2020. The comparison assumes \$100 was invested after the market closed on January 26, 2018 in our common stock and in each of the foregoing indices, and it assumes reinvestment of dividends, if any. The stock price performance included in this graph is not necessarily indicative of future stock price performance.

COMPARISON OF CUMULATIVE TOTAL RETURN
Among The Nasdaq Composite Index, The Nasdaq Biotechnology Index and Solid Biosciences Inc.



Holder

As of February 16, 2021, we had approximately 56 holders of record of our common stock. This number does not include beneficial owners whose shares were held in street name. The actual number of holders of our common stock is greater than this number of record holders and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers or held by other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Information about our Equity Compensation Plans

Information about our equity compensation plans is incorporated herein by reference to the section entitled “Securities Authorized for Issuance Under Equity Compensation Plans” in Item 12 of Part III of this Annual Report on Form 10-K.

Purchase of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Recent Sales of Unregistered Securities

On November 20, 2020, we issued a warrant to purchase 30,000 shares of our common stock, or the Warrant, to a consultant in consideration for services provided by the consultant. The Warrant is exercisable at an exercise price of \$3.29 per share and expires on November 19, 2030. The shares issuable upon exercise of the Warrant vest on a monthly basis over six months from the date of grant. We offered and sold the Warrant pursuant to an exemption from registration under Section 4(a)(2) of the Securities Act.

Except as set forth above, we did not sell any securities, during the year ended December 31, 2020 that were not registered under the Securities Act of 1933, as amended, or the Securities Act, and that have not otherwise been described in a Quarterly Report on Form 10-Q or in a Current Report on Form 8-K.

Item 6. Selected Financial Data.

We are a smaller reporting company as defined by Rule 12b-2 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and are not required to provide the information required under this item.

Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described, in or implied, by these forward-looking statements.

Overview

Our mission is to cure Duchenne muscular dystrophy, or Duchenne, a genetic muscle-wasting disease predominantly affecting boys, with symptoms that usually manifest between three and five years of age. Duchenne is a progressive, irreversible and ultimately fatal disease that affects approximately one in every 3,500 to 5,000 live male births and has an estimated prevalence of 10,000 to 15,000 cases in the United States alone. Duchenne is caused by mutations in the dystrophin gene, which result in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Without functioning dystrophin and certain associated proteins, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. There is no cure for Duchenne and, for the vast majority of patients, there are no satisfactory symptomatic or disease-modifying treatments. Our efforts are focused on our lead product candidate, SGT-001, a gene transfer candidate under investigation for its ability to drive functional dystrophin protein expression in patients' muscles and improve the course of the disease. Based on our preclinical program that included multiple animal species of different phenotypes and genetic variations, as well as preliminary clinical trial biomarker results, we believe that SGT-001, has the potential to slow or even halt the progression of Duchenne, regardless of the type of genetic mutation or stage of the disease.

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. If successfully developed and approved, we intend to commercialize SGT-001 in the United States and European Union and may enter into licensing agreements or strategic collaborations in other markets. If we generate product sales or enter into licensing agreements or strategic collaborations, we expect that any revenue we generate will fluctuate from quarter to quarter and year to year as a result of the timing and amount of any product sales, license fees, milestone payments and other payments. If we fail to complete the development of SGT-001 and our other product candidates in a timely manner or obtain regulatory approval of them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

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

Due to our significant research and development expenditure, licensing and patent investment, and general administrative costs associated with our operations, we have generated substantial operating losses in each period since our inception. Our net losses were \$88.3 million, \$117.2 million and \$74.8 million for the years ended December 31, 2020, 2019 and 2018, respectively. As of December 31, 2020, we had an accumulated deficit of \$404.6 million. We expect to incur significant expenses and operating losses for the foreseeable future.

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

As we seek to develop and commercialize SGT-001 or any other product candidates, we anticipate that our expenses will increase significantly and that we will need substantial additional funding to support our continuing operations. Until such time as we can generate significant revenue from product sales, if ever, we expect to finance our operations through a combination of public or private equity financings, debt financings or other sources, which may include licensing agreements or strategic collaborations. We may be unable to raise additional funds or enter into such agreements or arrangements when needed on favorable terms, if at all. If we fail to raise capital or enter into such agreements as and when needed, we may have to significantly delay, scale back or discontinue the development or commercialization of SGT-001 or our other product candidates.

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or determine when or if we will be able to achieve or maintain profitability. Even if we are able to generate revenue from product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

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

In July 2019, we completed a private placement of shares of our common stock and pre-funded warrants to purchase shares of our common stock resulting in net proceeds to us of \$57.9 million, after deducting offering costs.

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

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

During the year ended December 31, 2020, we sold 6,309,632 shares pursuant to a sales agreement, dated March 13, 2019, or the ATM Sales Agreement, by and between us and Jefferies LLC, or Jefferies, resulting in net proceeds of \$23.2 million.

As of December 31, 2020, we had cash and cash equivalents of \$154.7 million. Based upon our current and projected cash flow, we note there is substantial doubt about our ability to continue as a going concern within one year after the date that these financial statements are issued. As a result, in order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate. We believe that our cash and cash equivalents as of December 31, 2020 will enable us to fund our operating expenses and capital expenditure requirements into the first quarter of 2022. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate.

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

Corporate conversion

We operated as a Delaware limited liability company under the name Solid Biosciences, LLC until immediately prior to the effectiveness of our registration statement on Form S-1 on January 25, 2018, at which time we converted into a Delaware corporation pursuant to a statutory conversion and changed our name to Solid Biosciences Inc. In addition, immediately following the statutory conversion, entities formed solely for the purpose of holding membership interests in our limited liability company were merged with and into us. We refer to the corporate conversion and the mergers, collectively as the Corporate Conversion. As a result of the Corporate Conversion, the holders of the Series 1 and 2 Senior Preferred and Junior Preferred Units and Series A, B, C and D Common Units of Solid Biosciences, LLC became holders of common stock of Solid Biosciences Inc.

The consolidated financial statements included elsewhere in this Annual Report on Form 10-K are those of Solid Biosciences Inc. and its subsidiaries.

Financial operations overview

Revenue

We have not generated any revenue to date and do not expect to generate any revenue from the sale of our products for the next few years, if ever. If our development efforts for SGT-001 or our other product candidates are successful and result in marketing approval, we may generate revenue in the future from product sales. In October 2020, we executed the Collaboration Agreement and we expect to recognize revenue in 2021.

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 studies and clinical trials;
- salaries, benefits and other related costs, including equity-based compensation expense, for personnel engaged in research and development functions;
- costs of outside consultants, engaged to assist in our research and development activities, including their fees, equity-based compensation and related travel expenses;
- costs of laboratory supplies and acquiring, developing and manufacturing preclinical study and clinical trial materials;
- costs incurred in seeking regulatory approval of SGT-001 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 and clinical trial costs, based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors, collaborators and third-party service providers. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our consolidated financial statements as prepaid or accrued research and development expenses.

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

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

| (in thousands) | For the Year Ended December 31, | | |
|---|---------------------------------|------------------|------------------|
| | 2020 | 2019 | 2018 |
| SGT-001 | \$ 29,526 | \$ 45,281 | \$ 29,033 |
| Other product candidates | 3,114 | 4,941 | 3,506 |
| Unallocated research and development expenses | | | |
| Personnel related expenses | 22,009 | 29,372 | 18,511 |
| External expenses | 10,232 | 15,143 | 6,915 |
| Total unallocated research and development expenses | 32,241 | 44,515 | 25,426 |
| Total research and development expenses | <u>\$ 64,881</u> | <u>\$ 94,737</u> | <u>\$ 57,965</u> |

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 of our product candidates for which we obtain marketing approval or our other research and development expenses. We may never succeed in obtaining marketing approval for any of our product candidates. The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors, including:

- the scope, rate of progress, expense and results of any clinical trials of SGT-001 or other product candidates and other research and development activities that we may conduct;
- the imposition of regulatory restrictions on clinical trials, including full and partial clinical holds and the time and activities required to lift any such holds;
- uncertainties in clinical trial design and patient enrollment or drop out or discontinuation rates;
- significant and changing government regulation and regulatory guidance;
- potential additional studies or clinical trials requested by regulatory agencies;
- the timing and receipt of any marketing approvals; and
- the expense of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect that our research and development expenses will 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.

Restructuring charges

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

Other income (expense)

Interest income

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

Other income

We have received funding from charitable organizations, which is 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

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

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

Critical accounting policies and use of estimates

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

While our significant accounting policies are described in more detail in the notes to our consolidated financial statements appearing at the end of this Annual Report on Form 10-K, we believe that the following accounting policies are those most critical to the judgments and estimates used in the preparation of our consolidated financial statements.

Revenue Recognition

As discussed in Note 2 to our consolidated audited financial statements appearing at the end of this prospectus, we adopted Accounting Standards Codification (ASC) 606, Revenue from Contracts with Customers ("ASC 606") as of January 1, 2017. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services.

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

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

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

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

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

Accrued research and development expenses

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

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

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

Equity-based compensation

In connection with the completion of our initial public offering, we adopted the 2018 Omnibus Incentive Plan, or the 2018 Plan, which provides for the issuance of share-based awards, including options to purchase common stock. The 2018 Plan provides for the awarding of up to 5,001,000 shares of common stock for equity awards. On June 16, 2020, our stockholders approved the 2020 Equity Incentive Plan or the “2020 Plan” which consists of (i) 3,000,000 shares of common stock and (ii) additional shares of common stock (up to 4,879,025) as is equal to (i) the number of shares reserved under the 2018 Plan that remained available for grant under the 2018 Plan as of immediately prior to the date the 2020 Plan was approved by our stockholders and (ii) the number of shares subject to awards granted under the 2018 Plan which awards expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right.

We measure all stock options and other stock-based awards granted to employees, directors and non-employees based on the fair value on the date of the grant and recognize compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. We apply the straight-line method of expense recognition to all awards with only service-based vesting conditions. We have not issued any awards with performance-based vesting conditions. For stock-based awards granted to non-employees, compensation expense is recognized over the period during which services are rendered by such non-employees until completed.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. We historically have been a private company and lack company-specific historical and implied volatility information. Therefore, we estimate our expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expect to continue to do so until such time as we have adequate historical data regarding the volatility of our own traded stock price. For options with service-based vesting conditions, the expected term of our stock options has been determined utilizing the “simplified” method for awards that qualify as “plain-vanilla” options. Through December 31, 2018, the expected term of stock options granted to non-employees is equal to the contractual term of the option award and effective January 1, 2019, the “simplified” method is used. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future, if ever.

Results of operations

Comparison of the years ended December 31, 2020 and 2019

The following table summarizes our results of operations for the years ended December 31, 2020 and 2019:

| | For the Year Ended | | Increase (decrease) |
|------------------------------|--------------------|---------------------|------------------------|
| | December 31, | | |
| | 2020 | 2019 | |
| (in thousands) | | | |
| Revenue | \$ - | \$ - | \$ - |
| Operating expenses: | | | |
| Research and development | 64,881 | 94,737 | (29,856) |
| General and administrative | 21,581 | 24,581 | (3,000) |
| Restructuring expense | 1,944 | - | 1,944 |
| Total operating expenses | 88,406 | 119,318 | (30,912) |
| Loss from operations | (88,406) | (119,318) | 30,912 |
| Other income (expense): | | | |
| Interest income | 115 | 1,580 | (1,465) |
| Other income | 1 | 515 | (514) |
| Total other income (expense) | 116 | 2,095 | (1,979) |
| Net loss | <u>\$ (88,290)</u> | <u>\$ (117,223)</u> | <u>\$ 28,933</u> |

Research and development expenses

| | For the Year Ended | | Increase (decrease) |
|---|--------------------|------------------|------------------------|
| | December 31, | | |
| | 2020 | 2019 | |
| (in thousands) | | | |
| SGT-001 | \$ 29,526 | \$ 45,281 | \$ (15,755) |
| Other product candidates | 3,114 | 4,941 | (1,827) |
| Unallocated research and development expenses | | | |
| Personnel related expenses | 22,009 | 29,372 | (7,363) |
| External expenses | 10,232 | 15,143 | (4,911) |
| Total unallocated research and development expenses | 32,241 | 44,515 | (12,274) |
| Total research and development expenses | <u>\$ 64,881</u> | <u>\$ 94,737</u> | <u>\$ (29,856)</u> |

Research and development expenses for the year ended December 31, 2020 were \$64.9 million, compared to \$94.7 million for the year ended December 31, 2019. The decrease of \$29.9 million in research and development costs was due to a decrease in unallocated research and development costs of \$12.3 million primarily due to a reduction in personnel and facility related expenses as a result of the restructuring that occurred in January 2020, and a \$15.8 million decrease in costs related to our lead product candidate SGT-001 driven by a reduction in manufacturing costs of \$17.3 million partially offset by an increase in clinical costs of \$1.5 million primarily driven by clinical consulting costs. The reduction in research and development was also driven by a \$1.8 million decrease in costs related to other product candidates as we focused on advancing SGT-001.

General and administrative expenses

General and administrative expenses were \$21.6 million for the year ended December 31, 2020, compared to \$24.6 million for the year ended December 31, 2019. The decrease of \$3.0 million was due to a decrease in personnel related expenses of \$2.9 million partially due to the restructuring that occurred in January 2020 and a net decrease in corporate expenses of \$0.1 million.

Restructuring charges

During the year ended December 31, 2020, we recorded charges of \$1.9 million related to severance and other employee-related costs. We paid approximately \$1.9 million during the year ended December 31, 2020 and expect to pay approximately \$62 thousand during the first quarter of 2021.

Interest income

Interest income was \$0.1 million and \$1.6 million for the years ended December 31, 2020 and 2019, respectively. The decrease in interest income was due to a reduction of available-for-sale securities included within our portfolio.

Other income

Other income for the year ended December 31, 2020 was \$0.1 million compared to \$0.5 million for the year ended December 31, 2019. Other income relates to contributions from charitable organizations. We do not expect these contributions to significantly increase in future periods.

Comparison of the years ended December 31, 2019 and 2018

The following table summarizes our results of operations for the years ended December 31, 2019 and 2018:

| | For the Year Ended December 31, | | Increase (decrease) |
|------------------------------|------------------------------------|--------------------|------------------------|
| | 2019 | 2018 | |
| (in thousands) | | | |
| Revenue | \$ - | \$ - | \$ - |
| Operating expenses: | | | |
| Research and development | 94,737 | 57,965 | 36,772 |
| General and administrative | 24,581 | 17,722 | 6,859 |
| Total operating expenses | 119,318 | 75,687 | 43,631 |
| Loss from operations | (119,318) | (75,687) | (43,631) |
| Other income (expense): | | | |
| Interest income | 1,580 | 619 | 961 |
| Other income | 515 | 270 | 245 |
| Total other income (expense) | 2,095 | 889 | 1,206 |
| Net loss | <u>\$ (117,223)</u> | <u>\$ (74,798)</u> | <u>\$ (42,425)</u> |

Research and development expenses

| | For the Year Ended December 31, | | Increase (decrease) |
|---|------------------------------------|------------------|------------------------|
| | 2019 | 2018 | |
| (in thousands) | | | |
| SGT-001 | \$ 45,281 | \$ 29,033 | \$ 16,248 |
| Other product candidates | 4,941 | 3,506 | 1,435 |
| Unallocated research and development expenses | | | |
| Personnel related expenses | 29,372 | 18,511 | 10,861 |
| External expenses | 15,143 | 6,915 | 8,228 |
| Total unallocated research and development expenses | 44,515 | 25,426 | 19,089 |
| Total research and development expenses | <u>\$ 94,737</u> | <u>\$ 57,965</u> | <u>\$ 36,772</u> |

Research and development expenses for the year ended December 31, 2019 were \$94.7 million, compared to \$58.0 million for the year ended December 31, 2018. The increase of \$36.8 million in research and development costs was due to an increase in total unallocated research and development costs of \$19.1 million primarily due to personnel and facility related expenses, including costs incurred to operate our lab facility, a \$16.2 million increase in costs related to our lead product candidate SGT-001 driven by higher clinical development and manufacturing activities of \$13.2 million and contract cancellation fees of \$3.0 million as well as \$1.4 million increase in costs related to our other product candidates.

General and administrative expenses

General and administrative expenses were \$24.6 million for the year ended December 31, 2019, compared to \$17.7 million for the year ended December 31, 2018. The increase of \$6.9 million was driven by a \$4.4 million increase in equity-based compensation as a result of grants issued during the year ended December 31, 2019, and other personnel and facility related expenses of \$1.6 million as well as an increase of \$0.9 million for professional services and other corporate expenses.

Interest income

Interest income was \$1.6 million and \$0.6 million for the years ended December 31, 2019 and 2018, respectively. The increase in interest income was due to an increase in available-for-sale securities in our portfolio during the first half of 2019.

Other income

Other income for the year ended December 31, 2019 was \$0.5 million compared to \$0.3 million for the year ended December 31, 2018. The increase of \$0.2 million was due to an increase of income from charitable organizations. We do not expect these contributions to significantly increase in future periods.

Liquidity and capital resources

Sources of liquidity

To date, we have financed our operations primarily through private placements of preferred units and our initial public offering as well as private placements of shares of our common stock and pre-funded warrants to purchase shares of our common stock. Through December 31, 2020, we raised an aggregate of \$144.6 million of gross proceeds from our sales of preferred units prior to the completion of our initial public offering, an aggregate of \$129.1 million of net proceeds from the sale of our common stock after deducting underwriting discounts and commission and offering expenses in our initial public offering, an aggregate \$144.1 million of net proceeds, after deducting offering costs, from our July 2019 and December 2020 private placements, and approximately \$40 million from our sale of common stock to Ultragenyx.

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

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

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

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

In March 2019, we entered into the ATM Sales Agreement under which we may offer and sell, from time to time, shares of our common stock having aggregate gross proceeds of up to \$50.0 million through Jefferies as sales agent. Any such sales being made by any method that is deemed an "at-the-market offering" as defined in Rule 415 promulgated under the Securities Act. We will pay Jefferies a commission of up to 3% of the gross proceeds of any sales of common stock pursuant to the ATM Sales Agreement. During the year ended December 31, 2020, we sold 6,309,632 shares pursuant to the ATM Sales Agreement resulting in net proceeds of \$23.2 million.

As of December 31, 2020, we had cash and cash equivalents of \$154.7 million and had no debt outstanding.

Cash flows

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

| | For the Year Ended December 31, | | |
|--|---------------------------------|--------------------|------------------|
| | 2020 | 2019 | 2018 |
| (in thousands) | | | |
| Cash used in operating activities | \$ (56,599) | \$ (92,714) | \$ (70,197) |
| Cash provided by (used in) investing activities | 6,600 | 24,516 | (26,762) |
| Cash provided by financing activities | 128,700 | 57,875 | 131,507 |
| Net increase (decrease) in cash and cash equivalents | <u>\$ 78,701</u> | <u>\$ (10,323)</u> | <u>\$ 34,548</u> |

Operating activities

During the year ended December 31, 2020, operating activities used \$56.6 million of cash, primarily resulting from our net loss of \$88.3 million offset by non-cash charges of \$15.5 million due primarily to equity-based compensation of \$11.6 million and depreciation expense of \$3.9 million as well as cash provided by changes in our operating assets and liabilities. Net cash provided by changes in our operating assets and liabilities included a decrease in accounts payable, accrued expenses and non-current liabilities of \$4.6 million due to the timing of payments and an increase in deferred revenue of \$20.7 million as a result of the Ultragenyx Collaboration Agreement. Net cash provided by changes in our operating assets and liabilities also consisted primarily of an increase in prepaid expenses and other assets of \$0.1 million which was primarily due to an increase in prepayments related to research and development activities.

During the year ended December 31, 2019, operating activities used \$92.7 million of cash, primarily resulting from our net loss of \$117.2 million offset by non-cash charges of \$16.8 million due primarily to equity-based compensation of \$14.2 million and depreciation expense of \$2.8 million as well as cash provided by changes in our operating assets and liabilities. Net cash provided by changes in our operating assets and liabilities consisted primarily of a decrease in prepaid expenses and other assets of \$4.5 million which was primarily due to a decrease in prepayments related to research and development activities. Net cash provided by changes in our operating assets and liabilities also included an increase in accounts payable of \$3.8 million due to the timing of payments.

During the year ended December 31, 2018, operating activities used \$70.2 million of cash, primarily resulting from our net loss of \$74.8 million and cash used in changes in our operating assets and liabilities of \$2.8 million offset by non-cash charges of \$7.4 million due primarily to equity-based compensation of \$6.0 million and depreciation expense of \$1.6 million. Net cash used in changes in our operating assets and liabilities consisted primarily of an increase in prepaid expenses and other assets of \$4.9 million which was primarily due to an increase in prepayments related to research and development activities. Net cash used in changes in our operating assets and liabilities also included a decrease in accounts payable of \$1.6 million due to the timing of payments. These changes were offset by an increase in accrued expenses and other liabilities of \$2.9 million as well as proceeds received from our landlord for tenant improvements related to the buildout of our lab space of \$0.8 million.

Investing activities

During the year ended December 31, 2020, investing activities provided \$6.6 million of cash, consisting primarily of net proceeds on the sale and maturity of available-for-sale securities partially offset by purchases of property and equipment.

During the year ended December 31, 2019, investing activities provided \$24.5 million of cash, consisting primarily of net proceeds on the sale and maturity of available-for-sale securities partially offset by purchases of property and equipment.

During the year ended December 31, 2018, investing activities used \$26.8 million of cash, consisting primarily of net purchases of investments as well as the acquisition of property and equipment.

Financing activities

During the year ended December 31, 2020, net cash provided by financing activities was \$128.7 million, due to the net proceeds from our private placement of shares of our common stock that we completed in December 2020 and sales under the ATM Sales Agreement as well as proceeds from the issuance of common stock to Ultragenyx.

During the year ended December 31, 2019, net cash provided by financing activities was \$57.9 million, due to the net proceeds from our private placement of shares of our common stock and pre-funded warrants to purchase shares of our common stock that we completed in July 2019.

During the year ended December 31, 2018, net cash provided by financing activities was \$131.5 million, primarily due to the net proceeds from our initial public offering.

Funding requirements

We expect our expenses to increase substantially in connection with our ongoing development activities related to SGT-001. In addition, we expect to incur additional costs associated with operating as a public company. We expect that our expenses will increase substantially if and as we:

- continue to enroll patients in IGNITE DMD and continue clinical development of SGT-001;
- move other product candidates into clinical trials;
- 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 activities;
- acquire or in-license other drugs, technologies and intellectual property;
- fund a portion of the development or commercialization of products in collaboration with Ultragenyx pursuant to the Collaboration Agreement; and
- add operational, financial and management information systems and personnel.

As of December 31, 2020, we had cash and cash equivalents of \$154.7 million. We believe that our existing cash and cash equivalents as of December 31, 2020 will be sufficient to fund our operating expenses and capital requirements into the first quarter of 2022. As a result, in order to continue to operate our business beyond that time, we will need to raise additional funds. However, there can be no assurance that we will be able to generate funds on terms acceptable to us, on a timely basis, or at all. In addition, we have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently anticipate.

Because of the numerous risks and uncertainties associated with the development of SGT-001 and other product candidates and programs and because the extent to which we may enter collaborations with third parties for development of our product candidates is unknown, we are unable to estimate the timing and amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements will depend on many factors, including:

- the progress and results of IGNITE DMD and future clinical trials of SGT-001 and our other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001 and our other product candidates;
- the scope, progress, results and costs of discovery, laboratory testing, manufacturing, preclinical development and clinical trials for other product candidates that we may pursue in the future, if any;
- the costs associated with our manufacturing process development and evaluation of third-party manufacturers;
- whether we decide to construct and validate our own manufacturing facility and the associated costs;

- revenue, if any, received from commercial sale of SGT-001 or our other product candidates, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining, defending and enforcing our intellectual property rights and defending intellectual property-related claims;
- the outcome of any lawsuits filed against us;
- the terms of our current and any future license agreements and collaborations;
- the success of our collaboration with Ultragenyx;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other product candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to the COVID-19 pandemic and its collateral consequences.

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

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

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

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

Contractual obligations and commitments

The following table summarizes our contractual obligations at December 31, 2020 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

| (in thousands) | Payments due by period | | | | |
|---|------------------------|---------------------|----------------|----------------|-------------------------|
| | Total | Less Than 1 Year | 1 - 3 Years | 3 - 5 Years | More Than 5 Years |
| Operating lease commitments ⁽¹⁾ | \$ 5,332 | \$ 2,444 | \$ 2,110 | \$ 778 | \$ - |
| Finance lease commitments ⁽²⁾ | 852 | 276 | 576 | - | - |
| Unconditional purchase commitments ⁽³⁾ | 2,066 | 1,013 | 1,053 | - | - |
| Total | \$ 8,250 | \$ 3,733 | \$ 3,739 | \$ 778 | \$ - |

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

In January 2018, we executed a lease agreement for lab space in Cambridge, Massachusetts. The lease consists of approximately 9,500 square feet with an initial term of five years with the option to extend the term for one additional two-year term.

In January 2018, we executed a lease agreement for office space in Cambridge, Massachusetts. The lease serves as our corporate headquarters and consists of approximately 16,000 square feet. The term of the lease runs through February 2022.

In January 2019, we executed a lease agreement for additional office space in Cambridge, Massachusetts. The space serves as office space supporting the Company's lab operations and consists of approximately 5,000 square feet. The term of the lease runs through October 2025.

- (2) Represents minimum payments due for our lease of lab equipment which is in use in our lab facility in Cambridge, Massachusetts.

In November 2018, we executed a finance lease for lab equipment.

- (3) Unconditional purchase commitments represent minimum payments due to purchase goods that are enforceable and legally binding and that specify all significant terms, including fixed or minimum quantities to be purchased, fixed or variable price provisions, and the approximate timing of the transaction.

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 are cancelable by us upon prior notice of 30 days and, as a result, are not included in the table of contractual obligations above.

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 Annual Report on Form 10-K, such standards will not have a material impact on our consolidated financial statements or do not otherwise apply to our operations.

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.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Interest Rate Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2020, cash equivalents consisted of money market accounts that have contractual maturities of less than 90 days from the date of acquisition. Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, because of the short-term nature of our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

Inflation Risk

We do not believe that inflation had a material effect on our business, financial condition or results of operations in the last three years.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 is included at the end of this Annual Report on Form 10-K beginning on page F-1.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure.

Not applicable.

Item 9A. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Interim Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of December 31, 2020. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act, refers to controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that such information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to a company's management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that disclosure controls and procedures, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the objectives of the disclosure controls and procedures are met. Additionally, in designing disclosure controls and procedures, our management necessarily was required to apply its judgment in evaluating the cost-benefit relationship of possible disclosure controls and procedures. The design of any system of controls also is based in part upon certain assumptions about the likelihood of future events, and there can be no assurance that any design will succeed in achieving its stated goals under all potential future conditions; over time, controls may become inadequate because of changes in conditions, or the degree of compliance with policies or procedures may deteriorate. Because of the inherent limitations in a control system, misstatements due to error or fraud may occur and not be detected.

Based on the evaluation of our disclosure controls and procedures, our Chief Executive Officer and Interim Chief Financial Officer concluded that, as of December 31, 2020, our disclosure controls and procedures were effective at the reasonable assurance level.

Management's Annual Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act. Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Management conducted an evaluation of the effectiveness of our internal control over financial reporting as of December 31, 2020 based on the framework in *Internal Control-Integrated Framework* (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Based on that evaluation, management has concluded that the Company's internal control over financial reporting was effective as of December 31, 2020.

This Annual Report on Form 10-K does not include an attestation report of our independent registered public accounting firm due to a transition period established by rules of the SEC for “emerging growth companies”.

Changes in Internal Control over Financial Reporting

There have been no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the three months ended December 31, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

Not applicable.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

Set forth below are the names, ages and positions of our current executive officers and directors as of March 15, 2021.

| Name | Age | Position(s) held |
|-------------------------------|-----|---|
| Executive Officers | | |
| Ilan Ganot | 47 | Co-founder, President, Chief Executive Officer and Director |
| Erin Powers Brennan | 50 | Chief Legal Officer |
| Stephen DiPalma | 62 | Interim Chief Financial Officer and Treasurer |
| Carl Morris, Ph.D. | 51 | Chief Scientific Officer |
| Joel Schneider, Ph.D. | 36 | Chief Operating Officer |
| Non-Employee Directors | | |
| Ian Smith | 54 | Chairman of the Board of Directors |
| Matthew Arnold | 51 | Director |
| Martin Freed, M.D., F.A.C.P. | 60 | Director |
| Robert Huffines | 55 | Director |
| Clare Kahn | 69 | Director |
| Georgia Keresty | 59 | Director |
| Adam Koppel, M.D., Ph.D. | 51 | Director |
| Sukumar Nagendran, M.D. | 55 | Director |
| Rajeev Shah | 43 | Director |
| Adam Stone | 41 | Director |
| Lynne Sullivan | 54 | Director |

Executive officers

Ilan Ganot is one of our founders and has served as our Chief Executive Officer and as a member of our board of directors since our inception in 2013. Mr. Ganot has served as our President since June 2018. Previously, Mr. Ganot served as an investment banker at JPMorgan Chase & Co., a leading global financial services firm, from September 2011 to September 2013. From October 2008 to August 2011, Mr. Ganot served as a banker at Nomura Securities Co., Ltd., a securities and investment banking company, and from September 2003 to September 2008, at Lehman Brothers, a global financial services firm. Mr. Ganot received his M.B.A. from London Business School and holds law and business degrees from the Interdisciplinary Center in 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 Duchenne patients and his extensive leadership experience.

Erin Powers Brennan has served as our Chief Legal Officer and Secretary since March 2021. Prior to joining Solid, she served as Senior Vice President, General Counsel and Secretary at Covetrus, a publicly traded, global animal health pharmacy, technology, and services company from 2019 to 2020 and previously the General Counsel, Vets First Choice from 2018 to 2019, before it merged with Covetrus. Previously, she was a partner at Morgan, Lewis & Bockius, where she worked from 2013 to 2018. Ms. Brennan holds a J.D. from Boston College Law School, an M.A. in Law and Diplomacy from The Tufts University Fletcher School, and a B.A. in Government and Latin American Studies from Scripps College.

Stephen DiPalma has served as our Interim Chief Financial Officer and Treasurer since January 2021. He is currently a managing director of Danforth Advisors, LLC, a financial consultancy firm that specializes in working with life sciences companies. Prior to, and during his tenure at Danforth, Mr. DiPalma has served as Chief Financial Officer to a number of public companies, in addition to many private companies in various stages of development. Immediately prior to joining Danforth in 2014, he served as Chief Financial Officer at Forum Pharmaceuticals from 2009 to 2014. He holds a Bachelor of Science from the University of Massachusetts and a master's in business administration from Babson College.

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, or Pfizer'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., a pharmaceutical company acquired by Pfizer. 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 Operating Officer since March 2021. Prior to this role, Dr. Schneider served as the Chief Technology Officer and Head of Exploratory Research and Development from 2017 to 2021. 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 us, 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.

Non-employee directors

Ian F. Smith has served as a member of our board of directors since April 2020. Mr. Smith has served as a consultant to us since February 2020. Mr. Smith previously served as Executive Vice President and Chief Operating Officer of Vertex Pharmaceuticals, a public biotechnology company, from September 2017 to January 2019 and prior to that served as Chief Financial Officer from October 2001 to September 2017. Prior to joining Vertex in 2001, Mr. Smith served as a partner in the Life Science and Technology Practice of Ernst & Young LLP, an accounting firm. Mr. Smith currently serves as director and Executive Chair of the board of ViaCyte, Inc., a private biotechnology company and also serves on the board of AavantiBio, Inc., and is a Senior Advisor to Bain Capital Life Sciences. Previously, Mr. Smith served on the board of directors of Acorda Therapeutics, Inc., and Infinity Pharmaceuticals, both public biotechnology companies, as well as other private biotechnology companies. He received a B.A. with honors in accounting and finance from Manchester Metropolitan (UK). Mr. Smith is qualified to serve on our board of directors because of his more than 20 years of finance and broad operating experience for public companies in the biopharmaceutical industry.

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.

Martin Freed, M.D., F.A.C.P., has served as a member of our board of directors since June 2018. Dr. Freed has served as an independent consultant to several private pharmaceutical, biotechnology, and healthcare companies, specializing in clinical and general pharmaceutical development and clinical and regulatory strategy since February 2015. He co-founded and served as chief medical officer of Civitas Therapeutics, Inc., a biopharmaceutical company acquired by Acorda, from December 2010 to October 2014, and as senior vice president, clinical development of Acorda from October 2014 through January 2015. He has also served as chief medical officer at Adnexus Therapeutics, Inc. (acquired by Bristol-Myers Squibb) and Vitae Pharmaceuticals, Inc. Dr. Freed spent nearly 14 years at GlaxoSmithKline and its predecessor, SmithKline Beecham Pharmaceuticals or SmithKline Beecham, where he served numerous roles including vice president, clinical development and medical affairs in the metabolism therapeutic area. Dr. Freed currently serves on the board of directors of Dicerna Pharmaceuticals, Inc. and of Sojournix, Inc. Dr. Freed has been Board Certified in Internal Medicine, Nephrology and Clinical Pharmacology. He performed his internal medicine residency at Temple University Hospital and nephrology fellowship at Yale-New Haven Hospital. A Fellow of the American College of Physicians, Dr. Freed received a B.S. with distinction in biology from the University of Delaware and an M.D. from Pennsylvania State University's College of Medicine. Dr. Freed 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.

Robert Huffines has served as a member of our board of directors since December 2013. Mr. Huffines joined J.P. Morgan, a leading global financial services firm, in 1991 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.

Clare Kahn, Ph.D. has served as a member of our board of directors since March 2021. Dr. Kahn has served as R&D Strategy Officer at X-VAX Technology Inc. ("X-VAX"), a biotechnology company developing vaccines against pathogens acquired by mucosal infection, since October 2019. She served as Chief Regulatory and Preclinical Development Officer at X-VAX from October 2018 to October 2019. Dr. Kahn has also been the president of Clare Kahn Pharma Consulting LLC, through which she provides consulting services on regulatory strategy since June 2016. Dr. Kahn was previously Vice President, Worldwide Regulatory Strategy, Global Innovative Pharma at Pfizer from January 2014 to June 2016 and Vice President, Worldwide Regulatory Strategy, Specialty Care Business at Pfizer from June 2010 to December 2013. Prior to Pfizer, she was Vice President of Regulatory Affairs at GlaxoSmithKline from 1999 to 2010. Dr. Kahn has a Ph.D. in Biochemical Pharmacology from The Royal Postgraduate Medical School, London and served as Assistant Professor of Pharmacology and of Pathology and Laboratory Medicine at The University of Pennsylvania from 1981-1985. Dr. Kahn is qualified to serve on our board of directors because of her extensive leadership experience and her experience working in the healthcare sector.

Georgia Keresty, Ph.D. has served as a member of our board of directors since March 2021. Dr. Keresty has served as a senior advisor to Takeda, a global research and development driven biopharmaceutical company, since 2021. She served as the R&D chief operating officer of Takeda from 2017 to 2020. From 2003 to 2017 and from 1997 to 1999, she was an executive at Johnson & Johnson. From 1999 to 2003 and from 1983 to 1997, she held roles at Bristol-Myers Squibb Company and Novartis Pharmaceuticals Corporation, respectively. Dr. Keresty holds BSc degrees in Chemical Engineering and Computer Science from Clarkson University and Ramapo College of New Jersey, an M.S. degree in Information Systems from Pace University, an MBA in Operations Management from Rutgers University, a Ph.D. in Operations Management from Rutgers University, and an MPH in Global Health Leadership from the University of Southern California. Dr. Keresty is qualified to serve on our board of directors because of her extensive leadership experience and her experience working in the healthcare sector.

Adam Koppel, M.D., Ph.D., has served as a member of our board of directors since October 2017. Dr. Koppel rejoined Bain Capital, a global investment firm, 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 from mid-2014 to mid-2016, Dr. Koppel worked at Biogen Inc., or Biogen, a biotechnology company, 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., a management consulting firm, where he served a variety of healthcare companies. Dr. Koppel currently serves on the board of directors of Aptinix Inc., Cerevel Therapeutics LLC, Dicerca Pharmaceuticals, Inc., Foghorn Therapeutics Inc., and ViaCyte, Inc. Previously, Dr. Koppel served on the board of directors of Trevena Inc. and PTC Therapeutics, 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.

Sukumar Nagendran, M.D., has served as a member of our board of directors since September 2018. Since February 2020, Dr. Nagendran has served as Chief Medical Officer of an unnamed biopharmaceutical holding company. Prior to that, he was most recently the Chief Medical Officer & Senior Vice President of AveXis Inc., a clinical-stage gene therapy company, or AveXis, from September 2015 to July 2018, prior to the company's acquisition by Novartis. Prior to AveXis, Dr. Nagendran was Vice President of Medical Affairs of Quest Diagnostics, a provider of diagnostic information services, from March 2013 to September 2015. Prior to Quest Diagnostics, Dr. Nagendran held key leadership positions at Pfizer, Novartis, Daiichi Sankyo, and Reata Pharmaceuticals. Prior to moving to the biotech industry, Dr. Nagendran practiced internal medicine, with a focus on diabetes and cardiovascular disease. He is a Mayo Alumni Laureate and founding member of the Robert Wood Johnson Legacy Society. He is also the sponsor for the Fonseca-Nagendran Scholar award at the American Diabetes Association to enhance research in minority populations. Dr. Nagendran received his undergraduate degree in Biochemistry from Rutgers University and his M.D. from Rutgers Medical School and trained in Internal Medicine at Mayo Clinic, Rochester. Dr. Nagendran is qualified to serve on our board of directors because of his extensive leadership 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 has been a managing partner at RA Capital Management, L.P., a multi-stage investment manager dedicated to evidence-based investing in public and private healthcare and life science companies that are developing drugs, medical devices, and diagnostics, since 2004. Mr. Shah is also a member of the board of directors of Kala Pharmaceuticals, Inc., Satsuma Pharmaceuticals, Inc. and Black Diamond Therapeutics, Inc., each a public biopharmaceutical company. Mr. Shah was previously a member of the board of directors of Eidos Therapeutics, Inc., KalVista Pharmaceuticals, Inc. and Ra Pharmaceuticals, Inc., all public pharmaceutical companies. Mr. Shah received 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, a life science focused hedge fund, 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. Ms. Sullivan has served as the Chief Financial Officer for UNITY Biotechnology, Inc., a biotechnology company, since August 2020. Prior to that, Ms. Sullivan served as the Chief Financial Officer for Compass Therapeutics, LLC, a biotechnology company, or Compass, from December 2018 to August 2019. Prior to Compass, Ms. Sullivan served as Biogen's Senior Vice President of Finance from 2016 to December 2018, 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 BiomX Inc., and Inozyme, Inc. all of which are public biopharmaceutical companies. Ms. Sullivan also serves on the board of Inheris Biopharma, Inc., a private biopharmaceutical company. 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 Accountant 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 and her experience working in the healthcare sector.

Delinquent Section 16(a) Reports

Section 16(a) of the Exchange Act requires our directors and executive officers and holders of more than 10% of our common stock to file with the SEC initial reports of ownership and reports of changes in ownership of our common stock and other equity securities.

Based solely on a review of reports furnished to us, or written representations from reporting persons, we believe all directors, executive officers, and 10% owners timely filed all reports regarding transactions in our securities required to be filed for the fiscal year ended December 31, 2020 by Section 16(a) under the Exchange Act, with the exception of a Form 4/A filed by Ilan Ganot on September 14, 2020 to report an option grant to Mr. Ganot's wife that, at the time of filing the original Form 4 on July 29, 2020, was inadvertently omitted. Ms. Ganot is our employee.

Audit Committee

Our board of directors has established an audit committee, which operates under a charter that has been approved by our board of directors and that is available on the Investor Relations portion of our website, www.solidbio.com. Our audit committee consists of Ms. Sullivan, Mr. Arnold and Dr. Koppel, 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 Nasdaq. Each member of our audit committee can read and understand fundamental financial statements in accordance with 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.

Our audit committee's responsibilities include, among other things:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from that firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our risk assessment and risk management policies;
- establishing procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management;

- reviewing and approving or ratifying any related person transactions;
- reviewing on a periodic basis our investment policy; and
- preparing the audit committee report required by SEC rules.

Our board of directors has determined 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.

Code of business conduct and ethics

We have adopted a Code of Business Conduct and Ethics, or the Code of Conduct, that applies to our directors, executive officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. The Code of Conduct is available on the Investor Relations portion of our website, *www.solidbio.com*. The nominating and corporate governance committee of our board of directors is responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. In addition, we intend to post on our website all disclosures that are required by law or the listing standards of Nasdaq concerning any amendments to, or waivers of, any provision of the Code of Conduct.

Item 11. Executive Compensation.

Compensation of our Named Executive Officers

The following information describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers, or the Named Executive Officers. The Named Executive Officers for the year ended December 31, 2020 are:

- Ilan Ganot, our President and Chief Executive Officer;
- Carl Morris, Ph.D., our Chief Scientific Officer; and
- Jennifer Ziolkowski, our former Chief Financial Officer.

Summary Compensation Table

The following table contains information about the compensation paid to or earned by each of our Named Executive Officers for the years ended December 31, 2020 and 2019.

| Name and Principal Position | Year | Salary (\$) | Bonus (\$) ⁽¹⁾ | Option Awards (\$) ⁽²⁾ | Restricted Stock Units (\$) ⁽²⁾ | Total (\$) |
|---|------|----------------|------------------------------|--------------------------------------|--|------------|
| Ilan Ganot, President and Chief Executive Officer | 2020 | 535,000 | 294,250 | 405,382 | 246,370 | 1,481,002 |
| | 2019 | 520,000 | 193,050 | 2,622,879 | — | 3,335,929 |
| Carl Morris, Ph.D., Chief Scientific Officer | 2020 | 391,000 | 156,400 | 134,176 | 542,560 | 1,224,136 |
| Jennifer Ziolkowski, Chief Financial Officer ⁽³⁾ | 2020 | 391,000 | 156,400 | 134,176 | 542,560 | 1,224,136 |

- (1) Represents annual bonuses paid to the Named Executive Officers after the completion of each calendar year at the discretion of our board of directors.
- (2) 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 11 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.
- (3) Ms. Ziolkowski's employment terminated on January 31, 2021.

Narrative to Summary Compensation Table

Base Salary. In 2020, we paid our Named Executive Officers base salaries as follows: Mr. Ganot: \$535,000; Dr. Morris: \$391,000; and Ms. Ziolkowski: \$391,000. For 2021, our board of directors increased the base salary amount for Mr. Ganot to \$551,100 and for Dr. Morris to \$410,000.

We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our Named Executive Officers. None of our Named Executive Officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary.

Annual Bonus. Our board of directors may, in its discretion, award bonuses to our Named Executive Officers from time to time. Our employment agreements with our Named Executive Officers provide (or provided, in the case of Ms. Ziolkowski) that they will be eligible for annual performance-based bonuses up to a specified percentage of their salary, subject to approval by our board of directors. Performance-based bonuses, which are calculated as a percentage of base salary, are designed to motivate our employees to achieve annual goals based on our strategic, financial and operating performance objectives. From time to time, our board of directors has approved discretionary annual cash bonuses to our Named Executive Officers with respect to their prior year performance.

With respect to 2020, our board of directors awarded discretionary bonuses of \$294,250, \$156,400 and \$156,400 to Mr. Ganot, Dr. Morris and Ms. Ziolkowski, respectively.

Equity Incentives. Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incents our executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our Named Executive Officers and from time to time may grant equity incentive awards to them in the form of stock options.

In January 2020, we granted options to purchase 142,000 shares of our common stock to Mr. Ganot and 47,000 shares of our common stock to each of Dr. Morris and Ms. Ziolkowski. These options vest in equal annual installments over a term of four years from the date of grant.

In January 2020, we also granted restricted stock units with respect to 71,000 shares of our common stock to Mr. Ganot and 23,000 shares of restricted stock to each of Dr. Morris and Ms. Ziolkowski. These restricted stock units vested in equal semi-annual installments over a term of one year from the date of grant. In March 2020 we granted 75,000 shares of restricted stock units to each of Dr. Morris and Ms. Ziolkowski. These restricted stock units vest in equal annual installments over a term of two years from the date of grant. In June 2020 we granted 75,000 shares of restricted stock units to each of Dr. Morris and Ms. Ziolkowski. These restricted stock units vested in equal semi-annual installments over a term of one year from the date of grant.

In January 2021, we granted options to purchase 438,000 shares of our common stock to Mr. Ganot and 175,000 shares of our common stock to Dr. Morris. These options vest in equal annual installments over a term of four years from the date of grant.

Our employees and executives are eligible to receive stock options and other stock-based awards pursuant to our 2020 Equity Incentive Plan, or the 2020 Plan.

We use stock options to compensate our executive officers in the form of initial grants in connection with the commencement of employment and also at various times, often but not necessarily annually, if we have performed as expected or better than expected. In 2020, we also used restricted stock units to compensate our executive officers. None of our executive officers is currently party to an employment agreement that provides for automatic award of stock options or restricted stock units. We have granted stock options to our executive officers with time-based vesting. The options that we have granted to our executive officers typically become exercisable as to 25% of the shares underlying the option on the first anniversary of the grant date and as to an additional 25% of the original number of shares underlying the option annually thereafter. Vesting rights cease upon termination of employment and exercise rights cease shortly after termination, except that vesting is fully accelerated upon certain terminations in connection with a change of control and exercisability is extended in the case of death or disability. Prior to the exercise of an option, the holder has no rights as a stockholder with respect to the shares subject to such option, including no voting rights and no right to receive dividends or dividend equivalents.

The exercise price of all stock options granted after the closing of our initial public offering is equal to the fair market value of shares of our common stock on the date of grant, which is determined by reference to the closing market price of our common stock on the date of grant.

Employment Agreements

We have entered into employment agreements with each of our Named Executive Officers. The employment agreements set forth the terms of the Named Executive Officers' compensation, including their base salary, and annual performance bonus opportunity. In addition, the employment agreements provide that, subject to eligibility requirements under the plan documents governing such programs and our policies, the Named Executive Officers are (or were, in the case of Ms. Ziolkowski) entitled, on the same basis as our other employees, to participate in and receive benefits under, any medical, vision and dental insurance policy maintained by us and we will pay, consistent with our then-current employee benefit policy, a portion of the cost of the premiums for any such insurance policy in which the Named Executive Officer elects to participate. Each Named Executive Officer will also be eligible (or was eligible, in the case of Ms. Ziolkowski) to receive paid vacation time, sick time, and Company holidays consistent with our policies as then in effect from time to time and equity awards at such times and on such terms and conditions as the board of directors may determine. Each Named Executive Officer's employment is (or was, in the case of Ms. Ziolkowski) at will.

Employment Agreement with Ilan Ganot

On January 25, 2019, we entered into an employment agreement with Mr. Ganot, our President and Chief Executive Officer, which employment agreement amended and restated the terms of his existing agreement (with the exception of the restrictive covenant provisions contained therein).

Pursuant to his employment agreement, Mr. Ganot is entitled to an annual base salary of \$520,000, effective as of January 1, 2019, which base salary will be reviewed by the board of directors from time to time and is subject to change in the discretion of the board of directors. Mr. Ganot is also eligible to earn an annual performance bonus, with a target bonus amount equal to up to 55% of his base salary, based upon the board's assessment of his performance and the Company's attainment of targeted goals as set by the board in its sole discretion. The bonus may be in the form of cash, equity award(s), or a combination of cash and equity.

Mr. Ganot will remain bound by proprietary rights, non-disclosure, developments, non-competition and non-solicitation obligations pursuant to the restrictive covenants in his existing employment agreement, which provisions shall remain in full force and effect. Under these restrictive covenants, he has agreed not to compete with us during his employment and for a period of one year after the termination of his employment (provided that he is not restricted from promoting treatments for, or endeavoring to cure, Duchenne), not to solicit our employees, consultants, or actual or prospective customers or business relations during his employment and for a period of one year after the termination of his employment, and to protect our confidential and proprietary information indefinitely.

Mr. Ganot's employment agreement and his employment may be terminated: (1) upon his death or at our election due to his "disability"; (2) at our election, with or without "cause"; and (3) at his election, with or without "good reason" (as such terms are defined in his employment agreement).

In the event of the termination of Mr. Ganot's employment by us without cause, or by Mr. Ganot for good reason, prior to or more than twelve months following a "change in control" (as defined in his employment agreement), Mr. Ganot is entitled to receive his base salary that has accrued and to which he is entitled as of the termination date, to the extent consistent with Company policy, accrued but unused paid time off through and including the termination date, unreimbursed business expenses for which expenses he has timely submitted appropriate documentation, and other amounts or benefits to which he is entitled in accordance with the terms of the benefit plans then-sponsored by us, which we refer to collectively as the Ganot Accrued Obligations. In addition, subject to his execution and nonrevocation of a release of claims in our favor, Mr. Ganot is entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 12 months and (2) provided he is eligible for and timely elects to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 12 months following his date of termination.

In the event of the termination of Mr. Ganot's employment by us without cause, or by Mr. Ganot for good reason, within twelve months following a change in control, he is entitled to receive the Ganot Accrued Obligations. In addition, subject to his execution and nonrevocation of a release of claims in our favor, he is entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 18 months, (2) provided he is eligible for and timely elects to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 18 months following his date of termination, (3) a lump sum payment equal to 150% of his target bonus for the year in which his employment is terminated or, if higher, his target bonus immediately prior to the change in control and (4) full vesting acceleration of any then-unvested equity awards that vest based solely based on the passage of time held by Mr. Ganot, such that any such equity awards held by him become fully exercisable or non-forfeitable as of the termination date.

If Mr. Ganot's employment is terminated for any other reason, including as a result of his death or disability, for cause, or voluntarily by him without good reason, our obligations under the employment agreement cease immediately, and he is only entitled to receive the Ganot Accrued Obligations.

Employment Agreement with Carl Morris

On January 25, 2019, we entered into an employment agreement with Dr. Morris, our Chief Scientific Officer, which employment agreement amended and restated the terms of his existing agreement (with the exception of the restrictive covenant provisions contained therein).

Pursuant to his employment agreement, Dr. Morris is entitled to an annual base salary of \$380,000, effective as of January 1, 2019, which base salary will be reviewed by the board of directors from time to time and is subject to change in the discretion of the board of directors. Dr. Morris is also eligible to earn an annual performance bonus, with a target bonus amount equal to up to 40% of his base salary, based upon the board's assessment of his performance and the Company's attainment of targeted goals as set by the board in its sole discretion. The bonus may be in the form of cash, equity award(s), or a combination of cash and equity.

Dr. Morris will remain bound by proprietary rights, non-disclosure, developments, non-competition and non-solicitation obligations pursuant to the restrictive covenants in his existing employment agreement, which provisions shall remain in full force and effect. Under these restrictive covenants, he has agreed not to compete with us during his employment and for a period of one year after the termination of his employment, not to solicit our employees, consultants, or actual or prospective customers or business relations during his employment and for a period of one year after the termination of his employment, and to protect our confidential and proprietary information indefinitely.

Dr. Morris's employment agreement and his employment may be terminated: (1) upon his death or at our election due to his "disability"; (2) at our election, with or without "cause"; and (3) at his election, with or without "good reason" (as such terms are defined in his employment agreement).

In the event of the termination of Dr. Morris's employment by us without cause, or by Dr. Morris for good reason, prior to or more than twelve months following a "change in control" (as defined in his employment agreement), Dr. Morris is entitled to receive his base salary that has accrued and to which he is entitled as of the termination date, to the extent consistent with Company policy, accrued but unused paid time off through and including the termination date, unreimbursed business expenses for which expenses he has timely submitted appropriate documentation, and other amounts or benefits to which he is entitled in accordance with the terms of the benefit plans then-sponsored by us, which we refer to collectively as the Morris Accrued Obligations. In addition, subject to his execution and nonrevocation of a release of claims in our favor, Dr. Morris is entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 12 months and (2) provided he is eligible for and timely elects to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 12 months following his date of termination.

In the event of the termination of Dr. Morris's employment by us without cause, or by Dr. Morris for good reason, within twelve months following a change in control, he is entitled to receive the Morris Accrued Obligations. In addition, subject to his execution and nonrevocation of a release of claims in our favor, he is entitled to (1) continued payment of his base salary, in accordance with our regular payroll procedures, for a period of 12 months, (2) provided he is eligible for and timely elects to continue receiving group medical insurance under COBRA and the payments would not result in the violation of nondiscrimination requirements of applicable law, payment by us of the portion of health coverage premiums we pay for similarly-situated, active employees who receive the same type of coverage, for a period of up to 12 months following his date of termination, (3) a lump sum payment equal to 100% of his target bonus for the year in which his employment is terminated or, if higher, his target bonus immediately prior to the change in control and (4) full vesting acceleration of any then-unvested equity awards that vest based solely based on the passage of time held by Dr. Morris, such that any such equity awards held by him become fully exercisable or non-forfeitable as of the termination date.

If Dr. Morris's employment is terminated for any other reason, including as a result of his death or disability, for cause, or voluntarily by him without good reason, our obligations under the employment agreement cease immediately, and he is only entitled to receive the Morris Accrued Obligations.

Employment Agreement with Jennifer Ziolkowski

On January 25, 2019, we entered into an employment agreement with Ms. Ziolkowski, our Chief Financial Officer, which employment agreement amended and restated the terms of her existing agreement (with the exception of the restrictive covenant provisions contained therein). Ms. Ziolkowski's employment terminated on January 31, 2021.

Pursuant to her employment agreement, Ms. Ziolkowski was entitled to an annual base salary of \$380,000, effective as of January 1, 2019. Ms. Ziolkowski was also eligible to earn an annual performance bonus, with a target bonus amount equal to up to 40% of her base salary, based upon the board's assessment of her performance and the Company's attainment of targeted goals as set by the board in its sole discretion.

Ms. Ziolkowski will remain bound by proprietary rights, non-disclosure, developments, non-competition and non-solicitation obligations pursuant to the restrictive covenants in her existing employment agreement, which provisions shall remain in full force and effect. Under these restrictive covenants, she has agreed not to compete with us during her employment and for a period of one year after the termination of her employment, not to solicit our employees, consultants, or actual or prospective customers or business relations during her employment and for a period of one year after the termination of her employment, and to protect our confidential and proprietary information indefinitely.

2020 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, 2020.

| Name | Option Awards | | | | Stock Awards | |
|---------------------|---|---|----------------------------|------------------------|--|---|
| | Number of securities underlying unexercised options (#) exercisable | Number of securities underlying unexercised options (#) unexercisable | Option exercise price (\$) | Option expiration date | Number of shares of stock that have not vested (#) | Market value of shares of stock that have not vested (\$) ⁽¹¹⁾ |
| Ilan Ganot | 96,500 ⁽¹⁾ | 96,500 ⁽¹⁾ | 37.89 | July 25, 2028 | | |
| | 38,750 ⁽²⁾ | 116,250 ⁽²⁾ | 22.93 | January 23, 2029 | | |
| | - ⁽⁴⁾ | 142,000 ⁽⁴⁾ | 3.47 | January 27, 2030 | | |
| | | | | | 35,500 ⁽⁶⁾ | 269,090 |
| Carl Morris | 101,928 ⁽³⁾ | 101,930 ⁽³⁾ | 26.23 | February 14, 2028 | | |
| | 15,000 ⁽²⁾ | 45,000 ⁽²⁾ | 22.93 | January 23, 2029 | | |
| | - ⁽⁴⁾ | 47,000 ⁽⁴⁾ | 3.47 | January 27, 2030 | | |
| | | | | | 5,298 ⁽⁵⁾ | 40,159 |
| | | | | | 11,500 ⁽⁶⁾ | 87,170 |
| | | | | | 75,000 ⁽⁷⁾ | 568,500 |
| | | | | | 75,000 ⁽⁸⁾ | 568,500 |
| Jennifer Ziolkowski | 33,426 ⁽³⁾ | 33,426 ⁽³⁾ | 26.23 | February 14, 2028 | | |
| | 15,000 ⁽²⁾ | 45,000 ⁽²⁾ | 22.93 | January 23, 2029 | | |
| | - ⁽⁴⁾ | 47,000 ⁽⁴⁾ | 3.47 | January 27, 2030 | | |
| | | | | | 15,906 ⁽⁹⁾ | 120,567 |
| | | | | | 15,960 ⁽¹⁰⁾ | 120,977 |
| | | | | | 11,500 ⁽⁶⁾ | 87,170 |
| | | | | | 75,000 ⁽⁷⁾ | 568,500 |
| | | | | | 75,000 ⁽⁸⁾ | 568,500 |

- (1) This option was granted on July 25, 2018 under the 2018 Plan and is subject to vesting in equal annual installments over four years from the vesting start date through and including July 25, 2022.
- (2) This option was granted on January 23, 2019 under the 2018 Plan and is subject to vesting in equal annual installments over four years from the vesting start date through and including January 23, 2023.
- (3) This option was granted on February 14, 2018 under the 2018 Plan and is subject to vesting in equal annual installments over four years from the vesting start date through and including February 14, 2022.
- (4) This option was granted on January 27, 2020 under the 2018 Plan and is subject to vesting in equal annual installments over four years from the vesting start date through and including January 27, 2024.

- (5) Consists of restricted stock awards granted under our Solid Biosciences, LLC Amended and Restated Equity Incentive Plan. The grant was made on March 30, 2017, vested as to 25% on the first anniversary of the vesting start date and is subject to equal semiannual vesting installments thereafter through and including March 30, 2021.
- (6) Consists of restricted stock awards granted under our Solid Biosciences, LLC Amended and Restated Equity Incentive Plan. The grant was made on January 27, 2020, vested as to 50% on the first anniversary of the vesting start date and 50% on the second anniversary of the vesting date. Ms. Ziolkowski's employment terminated on January 31, 2021.
- (7) Consists of restricted stock awards granted under our Solid Biosciences, LLC Amended and Restated Equity Incentive Plan. The grant was made on March 11, 2020, vested as to 50% on the first anniversary of the vesting start date and 50% on the second anniversary of the vesting date. Ms. Ziolkowski's employment terminated on January 31, 2021.
- (8) Consists of restricted stock awards granted under our Solid Biosciences, LLC Amended and Restated Equity Incentive Plan. The grant was made on June 16, 2020, vested as to 50% on March 11, 2021 and 50% on March 11, 2022. Ms. Ziolkowski's employment terminated on January 31, 2021.
- (9) Consists of restricted stock awards granted under our Solid Biosciences, LLC Amended and Restated Equity Incentive Plan. The grant was made on May 31, 2017, vested as to 25% on the first anniversary of the vesting start date and is subject to equal semiannual vesting installments thereafter through and including May 31, 2021. Ms. Ziolkowski's employment terminated on January 31, 2021.
- (10) Consists of restricted stock awards granted under our Solid Biosciences, LLC Amended and Restated Equity Incentive Plan. The grant was made on December 7, 2017, vested as to 25% on the first anniversary of the vesting start date and is subject to equal semiannual vesting installments thereafter through and including December 7, 2021. Ms. Ziolkowski's employment terminated on January 31, 2021.
- (11) Based on the \$7.58 closing sale price of our common stock on December 31, 2020 as reported by the Nasdaq Global Select Market.

On January 25, 2021, we granted a stock option to Mr. Ganot and Dr. Morris for the right to buy 438,000 and 175,000 shares of our common stock, respectively. The options have an exercise price of \$6.80 per share, vest in four equal annual installments beginning on January 25, 2022 and expire on January 25, 2031.

Equity Incentive Plans

Solid Biosciences, LLC Amended and Restated Equity Incentive Plan

Prior to the closing of our initial public offering, Solid Biosciences, LLC maintained its Amended and Restated Equity Incentive Plan, or the Existing Plan, under which Solid Biosciences, LLC granted Series D Common Units to its employees, consultants and other service providers. The Existing Plan was frozen in connection with our initial public offering. 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.

2018 Omnibus Incentive Plan

Prior to our initial public offering, the board of managers of Solid Biosciences, LLC adopted the Solid Biosciences Inc. 2018 Omnibus Incentive Plan, or 2018 Plan, contingent upon the consummation of our initial public offering. The unitholders of Solid Biosciences, LLC approved the 2018 Plan contingent upon the consummation of our initial public offering.

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

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 was filed as Exhibit 99.1 to our registration statement on Form S-8 filed with the SEC on January 29, 2018 and is incorporated herein by reference.

Administration of the plan

The board of managers of Solid Biosciences, LLC 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.

Types of awards

Stock options. The 2018 Plan authorized the Administrator to grant incentive stock options, or 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 determined 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 are exercisable at such times and subject to such terms as were 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 (a) in cash or by check, bank draft or money order; (b) 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 (c) 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 authorized the Administrator to grant restricted stock. Any recipients of restricted stock were required to 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, 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 authorized 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 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 authorized the Administrator to grant cash awards that are payable or otherwise based on the attainment of pre-established performance goals during a performance period. Any such performance goals are based on the attainment of a certain target level of, or a specified increase in (or decrease where noted), criteria selected by the Administrator.

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.

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

On January 29, 2018, we filed 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 available for issuance under the 2018 Plan.

2020 Equity Incentive Plan

Our 2020 Equity Incentive Plan, or the 2020 Plan, which became effective on June 16, 2020, was adopted by our board of directors in April 2020 and approved by our stockholders approved in June 2020.

The material terms of the 2020 Plan are summarized below. The following summary is qualified in its entirety by reference to the complete text of the 2020 Plan, a copy of which was filed as Exhibit 99.1 to our current report on Form 8-K filed with the SEC on June 18, 2020 and is incorporated herein by reference.

Types of Awards; Shares Available for Awards

The 2020 Plan provides for the grant of incentive stock options intended to qualify under Section 422 of the Code, nonstatutory stock options, SARs, restricted stock, restricted stock units, other stock-based awards and cash awards as described below, which we collectively refer to as awards.

Subject to adjustment in the event of stock splits, stock dividends or similar events, awards may be made under the 2020 Plan for up to the sum of 3,000,000 shares of our common stock plus such additional number of shares of our common stock (up to 4,879,025 shares) as is equal to (x) the number of shares of our common stock reserved for issuance under the 2018 Plan that remained available for grant under the 2018 Plan immediately prior to the date that the 2020 Plan was approved by our stockholders and (y) the number of shares of common stock subject to awards granted under the 2018 Plan, which awards expire, terminate or are otherwise surrendered, cancelled or forfeited or repurchased by us pursuant to a contractual repurchase right.

The 2020 Plan provides that the maximum amount of cash and equity compensation (calculated based on grant date fair value for financial reporting purposes) granted to any individual non-employee director in his or her capacity as a non-employee director in any calendar year may not exceed \$500,000 in the case of an incumbent non-employee director or \$1,000,000 in the case of the first year of service of a non-employee director. Exceptions to this limitation may only be made by our board, in its discretion, in extraordinary circumstances, provided that the non-employee director receiving the additional compensation does not participate in the decision to award such compensation. Cash and awards granted under the 2020 Plan to non-employee directors in their capacity as our consultants or advisors are not subject to this limitation.

Descriptions of Awards

Options. Optionees receive the right to purchase a specified number of shares of common stock at a specified exercise price and subject to the other terms and conditions that are specified in connection with the option grant. An option that is not intended to be an “incentive stock option” is a “nonstatutory stock option”. Options may not be granted at an exercise price that is less than 100% of the fair market value of our common stock on the date of grant. Under the terms of the 2020 Plan, options may not be granted for a term in excess of ten years. The 2020 Plan permits participants to pay the exercise price of options using one or more of the following manners of payment: (i) payment by cash, by check, (ii) except as may otherwise be provided in the applicable option agreement or approved by our board, in connection with a “cashless exercise” through a broker, (iii) to the extent provided in the applicable option agreement or approved by our board, and subject to certain conditions, by delivery of shares of common stock to us owned by the participant valued at their fair market value, (iv) to the extent provided in an applicable nonstatutory stock option agreement or approved by our board, by delivery of a notice of “net exercise” as a result of which we will retain a number of shares of common stock otherwise issuable pursuant to the stock option equal to the aggregate exercise price for the portion of the option being exercised divided by the fair market value of our common stock on the date of exercise, (v) to the extent permitted by applicable law and provided for in the applicable option agreement or approved by our board, by any other lawful means (but not by a promissory note of the participant), or (vi) by any combination of these forms of payment.

Stock Appreciation Rights. An SAR is an award entitling the holder, upon exercise, to receive a number of shares of our common stock, or cash (or a combination of shares of our common stock and cash) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of our common stock over the measurement price. The 2020 Plan provides that the measurement price of an SAR may not be less than the fair market value of our common stock on the date the SAR is granted and that SARs may not be granted with a term in excess of 10 years.

Restricted Stock Awards. Restricted stock awards entitle recipients to acquire shares of our common stock, subject to our right to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) in the event that the conditions specified in the applicable award are not satisfied prior to the end of the applicable restriction period established for such award.

Restricted Stock Unit Awards. Restricted stock units, or RSUs, entitle the recipient to receive shares of our common stock, or cash equal to the fair market value of such shares, to be delivered at the time such award vests pursuant to the terms and conditions established by our board.

Other Stock-Based Awards. Under the 2020 Plan, our board may grant other awards of shares of our common stock, and other awards that are valued in whole or in part by reference to, or are otherwise based on, shares of our common stock or other property, having such terms and conditions as our board may determine. We refer to these types of awards as other stock-based awards. Other stock-based awards may be available as a form of payment in settlement of other awards granted under the 2020 Plan or as payment in lieu of compensation to which a participant is otherwise entitled. Other stock-based awards may be paid in shares of our common stock or in cash, as our board may determine.

Cash Awards. Under the 2020 Plan, the board has the right to grant cash-based awards including awards subject to performance conditions.

Performance Conditions. Our board may specify that the degree of granting, vesting and/or payout of any award is subject to the achievement of one or more of performance measures enumerated in the 2020 Plan and established by the board.

Eligibility to Receive Awards

All of our employees, officers, and directors, as well as our consultants and advisors, are eligible to receive awards under the 2020 Plan. However, incentive stock options may only be granted to our employees.

Transferability of Awards

Except as our board may permit, awards may not be sold, assigned, transferred, pledged or otherwise encumbered by a participant, either voluntarily or by operation of law, except by will or the laws of descent and distribution or, other than in the case of an incentive stock option, pursuant to a qualified domestic relations order.

Administration

The 2020 Plan is administered by our board. Pursuant to the terms of the 2020 Plan, our board may delegate any or all of its powers under the 2020 Plan to one or more committees or subcommittees of our board. The board has authorized our compensation committee to administer certain aspects of the 2020 Plan, including the granting of awards to executive officers. The board of directors, or any such committee, may delegate to one of our officers, the authority to make grants under the 2020 Plan, subject to the limitations set forth in the 2020 Plan.

Subject to any applicable limitations contained in the 2020 Plan, the board, our compensation committee, or any other committee or officer to whom the board delegates authority, as the case may be, selects the recipients of awards and determines (i) the number of shares of common stock, cash or other consideration covered by awards and the terms and conditions of such awards, including the dates upon which such awards become exercisable or otherwise vest, (ii) the exercise or measurement price of awards, if any, and (iii) the duration of awards.

In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock, other than an ordinary cash dividend, we are required to make equitable adjustments (or make substituted awards, as applicable), in the manner determined by our board, to (i) the number and class of securities available under the 2020 Plan, (ii) the share counting rules and sublimit set forth in the 2020 Plan, (iii) the number and class of securities and exercise price per share of each outstanding option, (iv) the share- and per-share provisions and the measurement price of each outstanding SAR, (v) the number of shares subject to and the repurchase price per share subject to each outstanding award of restricted stock, and (vi) the share and per-share-related provisions and the purchase price, if any, of each outstanding RSU award and each outstanding other stock-based award.

Amendment of Awards

Except as otherwise provided under the 2020 Plan with respect to repricing outstanding stock options or SARs, our board may amend, modify or terminate any outstanding award, including but not limited to, substituting therefor another award of the same or a different type, changing the date of exercise or realization, and converting an incentive stock option to a nonstatutory stock option, provided that the participant's consent to any such action will be required unless our board determines that the action, taking into account any related action, does not materially and adversely affect the participant's rights under the 2020 Plan or the change is otherwise permitted under the terms of the 2020 Plan in connection with a change in capitalization or reorganization event.

Reorganization Events

The 2020 Plan contains provisions addressing the consequences of any reorganization event. A reorganization event is defined under the 2020 Plan as (a) any merger or consolidation of us with or into another entity as a result of which all of our common stock is converted into or exchanged for the right to receive cash, securities or other property, or is cancelled, (b) any transfer or disposition of all of our common stock for cash, securities or other property pursuant to a share exchange or other transaction or (c) our liquidation or dissolution.

Amendment or Termination

No award may be granted under the 2020 Plan after June 15, 2030, but awards previously granted may extend beyond that date. Our board may amend, suspend or terminate the 2020 Plan or any portion of the 2020 Plan at any time, except that no amendment that would amend the prohibitions on repricings without stockholder approval may be amended without stockholder approval and no amendment that would require stockholder approval under the rules of the national securities exchange on which we then maintains our primary listing may be made effective unless and until such amendment has been approved by our stockholders.

Registration of Shares

On August 6, 2020, we filed a registration statement on Form S-8 under the Securities Act to register the full number of shares of common stock available for issuance under the 2020 Plan.

Non-employee director compensation

During 2020, we did not provide any compensation to Mr. Ganot, our President and Chief Executive Officer, for his service as a member of our board. Mr. Ganot's compensation as an executive officer is set forth above under "*Executive Compensation--2020 Summary Compensation Table.*"

Non-employee director compensation is set by our board of directors at the recommendation of our compensation committee. In 2020, the compensation committee retained Radford, an AON Hewitt company, to assist in assessing our non-employee director compensation program and provide recommendations with respect to the compensation program.

Under our current director compensation program, we pay our non-employee directors a cash retainer for their service on the board of directors and for their service on each committee of which the director is a member. The chairs of each committee receive higher retainers for such service. These fees are payable in arrears in equal semi-annual installments not later than the 15th business day following the end of the second and fourth calendar quarters, provided that the amount of such payment will be prorated for any portion of such semi-annual period that the director is not serving on the board, on such committee or in such position, and no fee shall be payable in respect of any period prior to the completion of our initial public offering. The fees paid to non-employee directors for their service on the board of directors and for their service on each committee of the board of directors of which the director is a member are as follows:

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

We also reimburse our non-employee directors for reasonable out-of-pocket business expenses incurred in connection with the performance of their duties as directors, including travel expenses in connection with their attendance in person at our board of director and committee meetings.

In addition, under our current director compensation program, each new non-employee director elected to our board of directors receives an option to purchase 40,000 shares of our common stock under our 2020 Plan. Each of these options vest in equal annual installments over a three-year period measured from the date of grant, subject to the director's continued service as a director. Further, on the date of our annual meeting of stockholders, each non-employee director that has served on our board of directors for at least six months prior to such annual meeting receives an option to purchase 10,000 shares of our common stock under our 2020 Plan. Each of these options vest in full on the earlier to occur of the one-year anniversary of the grant date and immediately prior to our first annual meeting of stockholders occurring after the grant date, subject to the director's continued service as a director. All options granted to our non-employee directors under our director compensation program will be issued at exercise prices equal to the fair market value of our common stock on the date of grant and will become exercisable in full in the event of a change in control.

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

The table below shows the compensation paid to our non-employee directors during 2020.

| Name | Fees Earned or Paid in | | | Total (\$) |
|------------------------------|---------------------------------|--|--|------------|
| | Cash (\$) | Option Awards (\$) ⁽¹⁾⁽²⁾ | All Other Compensation (\$) ⁽⁷⁾ | |
| Andrey Zarur, Ph.D. | 31,836 | 35,586 ⁽³⁾ | 58,333 ⁽⁷⁾ | 125,755 |
| Ian Smith | 44,110 | 460,528 ⁽⁶⁾ | - | 504,638 |
| Matthew Arnold | 42,500 | 34,290 ⁽⁴⁾ | - | 76,790 |
| Martin Freed, M.D., F.A.C.P. | 54,000 | 45,720 ⁽⁵⁾ | - | 99,720 |
| Robert Huffines | 35,000 | 34,290 ⁽⁴⁾ | - | 69,290 |
| Adam Koppel, M.D., Ph.D. | 60,000 | 45,720 ⁽⁵⁾ | - | 105,720 |
| Sukumar Nagendran, M.D. | 42,500 | 45,720 ⁽⁵⁾ | - | 88,220 |
| Rajeev Shah | 40,000 | 34,290 ⁽⁴⁾ | - | 74,290 |
| Adam Stone | 48,000 | 34,290 ⁽⁴⁾ | - | 82,290 |
| Lynne Sullivan | 54,000 | 34,290 ⁽⁴⁾ | - | 88,290 |

- (1) 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 11 to our audited consolidated financial statements appearing elsewhere in this Annual Report on Form 10-K.
- (2) As of December 31, 2020, our non-employee directors held options to purchase shares of our common stock as follows: Mr. Smith: 300,000 shares; Mr. Arnold: 50,000 shares; Dr. Freed: 70,000 shares; Mr. Huffines: 50,000 shares; Dr. Koppel: 60,000 shares; Dr. Nagendran: 70,000 shares; Mr. Shah: 50,000 shares; Mr. Stone: 50,000 shares; and Ms. Sullivan: 50,000 shares.
- (3) Consists of an option to purchase 10,000 shares of our common stock granted on January 2, 2020. Dr. Zarur resigned as a member of our board of directors in June 2020.
- (4) Consists of an option to purchase 30,000 shares of our common stock granted on June 16, 2020.
- (5) Consists of an option to purchase 40,000 shares of our common stock granted on June 16, 2020.
- (6) Consists of: (i) an option to purchase 40,000 shares of our common stock granted on April 15, 2020 and (ii) in respect of his services as a consultant to us for the year ended December 31, 2020, (a) an option to purchase 60,000 shares of our common stock granted on February 10, 2020 which was modified and deemed fully vested as of June 16, 2020 and (b) an option to purchase 200,000 shares of our common stock granted on June 16, 2020. The compensation arrangements we have with Mr. Smith for his services to us as a consultant are described below in Item 13, "*Certain Relationships and Related Transactions, and Director Independence—Other arrangements.*"
- (7) Consists of payments made to Dr. Zarur in consideration for his consulting services provided to the company.

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.

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

Security Ownership of Certain Beneficial Owners and Management

The following table sets forth information regarding the beneficial ownership of our common stock as of February 16, 2021 by (i) each person whom we know to beneficially own more than 5% of our outstanding common stock, or a 5% stockholder, (ii) each director, (iii) each Named Executive Officer and (iv) all current directors and executive officers as a group. Unless otherwise indicated, the address of each executive officer and director is c/o Solid Biosciences Inc., 141 Portland Street, Fifth Floor, Cambridge, MA 02139.

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 February 16, 2021. The percentage of beneficial ownership in the table below is based on 85,130,417 shares of common stock deemed to be outstanding as of February 16, 2021.

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.

| Name of Beneficial Owner | Number of Shares Beneficially Owned | Percentage of Shares Beneficially Owned |
|---|-------------------------------------|---|
| 5% Stockholders: | | |
| Perceptive Life Sciences Master Fund LTD ⁽¹⁾ | 10,803,857 | 12.69% |
| Entities affiliated with RA Capital Management, L.P. ⁽²⁾ | 9,976,569 | 11.72% |
| EcoR1 Capital, LLC ⁽³⁾ | 7,977,702 | 9.37% |
| Ultragenyx Pharmaceutical Inc. ⁽⁴⁾ | 7,825,797 | 9.19% |
| BCLS SB Investco, LP ⁽⁵⁾ | 7,060,353 | 8.29% |
| Boxer Capital, LLC ⁽⁶⁾ | 6,427,903 | 7.36% |
| Gilad Hayeem ⁽⁷⁾ | 4,441,972 | 5.22% |
| Named Executive Officers and Directors: | | |
| Ilan Ganot ⁽⁸⁾ | 1,704,341 | 2.00% |
| Carl Morris, Ph.D. ⁽⁹⁾ | 493,643 | * |
| Jennifer Ziolkowski ⁽¹⁰⁾ | 410,812 | * |
| Ian Smith ⁽¹¹⁾ | 590,852 | * |
| Matthew Arnold ⁽¹²⁾ | 3,953,429 | 4.64% |
| Martin Freed, M.D., F.A.C.P. ⁽¹³⁾ | 77,096 | * |
| Robert Huffines ⁽¹⁴⁾ | 20,000 | * |
| Adam Koppel, M.D., Ph.D. ⁽¹⁵⁾ | 7,080,353 | 8.32% |
| Sukumar Nagendran, M.D. ⁽¹⁶⁾ | 57,591 | * |
| Rajeev Shah ⁽¹⁷⁾ | 9,996,569 | 11.74% |
| Adam Stone ⁽¹⁸⁾ | 20,000 | * |
| Lynne Sullivan ⁽¹⁹⁾ | 20,000 | * |
| All current directors and executive officers as a group (13 persons) ⁽²⁰⁾ | 24,447,053 | 28.37% |

* Less than one percent.

- (1) Consists of shares held by Perceptive Life Sciences Master Fund LTD, or the Master Fund. Perceptive Advisors LLC is the investment manager to Master Fund and may be deemed to beneficially own the securities directly held by the Master Fund. 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 the Master Fund. The address of Perceptive is 51 Astor Place, 10th Floor, New York, NY 10003. Perceptive reports that it holds shared voting power and shared dispositive power with respect to all shares held by it. Based on information set forth in a Schedule 13D/A filed with the SEC on December 17, 2020.

- (2) Consists of (a) 8,709,882 shares held by RA Capital Healthcare Fund, L.P. (“RA Capital Fund”) and (b) 1,266,687 shares held by Blackwell Partners LLC—Series A (“Blackwell”). RA Capital Management, L.P. (“RA Capital”) is the investment manager for RA Capital fund and Blackwell. The general partner of RA Capital Management, L.P. is RA Capital Management GP, LLC, of which Dr. Peter Kolchinsky and Mr. Shah are the managing members. 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. RA Capital Management, L.P., RA Capital Management GP, LLC, Dr. Kolchinsky and Mr. Shah may be deemed indirect beneficial owners of the shares held by RA Capital Healthcare Fund, L.P. and Blackwell Partners LLC – Series A. RA Capital Management, L.P., RA Capital Management GP, LLC, Dr. Kolchinsky and Mr. Shah expressly disclaim beneficial ownership over all shares held by RA Capital Healthcare Fund, L.P. and Blackwell Partners LLC – Series A, except to the extent of their pecuniary interest therein, and disclaim any pecuniary interest in the shares held by Blackwell Partners LLC – Series A. The address for each of RA Capital Fund, Blackwell, and RA Capital is c/o 200 Berkeley Street, 18th Floor, Boston, MA 02116. Entities affiliated with RA Capital report that they hold shared voting power and shared dispositive power with respect to all shares held by them. Based on information set forth in a Schedule 13D/A filed with the SEC on December 16, 2020.
- (3) Consists of (a) 1,238,770 shares held by EcoR1 Capital, LLC (“EcoR1”) and (b) 6,738,932 shares held by EcoR1 Capital Fund Qualified, L.P. (“Qualified Fund”), where EcoR1 and Oleg Nodelman hold shared voting power and shared dispositive power. The address of EcoR1, the Qualified Fund and Oleg Nodelman is 357 Tehama Street #3, San Francisco, CA 94103. Based on information set forth in a Schedule 13G/A filed with the SEC on February 16, 2021.
- (4) Consists of shares held by Ultragenyx Pharmaceuticals, Inc. with principal executive offices located at 60 Leveroni Court, Novato, California 94949.
- (5) Consists of shares held by BCLS SB Investco, LP (“BCLS”). 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, a member of our board of directors. 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. Based on information set forth in a Schedule 13D/A filed with the SEC on December 11, 2020.
- (6) Consists of (a) 4,234,574 shares held by Boxer Capital, LLC (“Boxer Capital”) for which Boxer Capital, Boxer Asset Management Inc. (“Boxer Management”) and Joe Lewis hold shared voting power and shared dispositive power, (b) 1,965,687 shares of common stock which may be acquired upon the exercise of warrants held by Boxer Capital within 60 days of February 16, 2021 for which Boxer Capital, Boxer Management and Joe Lewis hold shared voting power and shared dispositive power, (c) 35,000 shares held by MVA Investors, LLC (“MVA Investors”) for which MVA Investors and Aaron Davis hold shared voting power and shared dispositive power. Boxer Management is the managing member and majority owner of Boxer Capital. Joe Lewis is the sole indirect beneficial owner of and controls Boxer Management. MVA Investors is the independent, personal investment vehicle of certain employees of Boxer Capital. Aaron Davis is a member of and has voting and dispositive power over securities held by MVA Investors. The address of Boxer Capital, MVA Investors, LLC and Aaron Davis is 11682 El Camino Real, Suite 320, San Diego, CA 92130. The address of Boxer Management and Joe Lewis is Cay House, EP Taylor Drive N7776, Lyford Cay, New Providence, Bahamas. Based on information set forth in a Schedule 13G/A filed with the SEC on February 16, 2021.
- (7) All shares are held by DTMG Bermuda Limited (“DTMG”), which is owned and controlled by Gilad Hayeem. Mr. Hayeem and DTMG report that they hold shared voting power and shared dispositive power with respect to all shares held. The address for Mr. Hayeem and DTMG is c/o Hunton Andrews Kurth LLP, Attn: Eric Markus, 2200 Pennsylvania Avenue, NW, Washington, DC 20037. 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. Based on information set forth in a Schedule 13G/A filed with the SEC on February 14, 2020 and on information provided to us by Mr. Hayeem.
- (8) Consists of (a) 1,139,190 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, (c) 290,914 shares held by Mr. Adam Ganot and Ms. Ganot, as trustees for the Ilan Ganot 2017 Irrevocable Trust, (d) 209,500 shares of common stock underlying options held by Mr. Ganot that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date, (e) 3,026 shares held by Mr. Ganot’s wife and (f) 1,080 shares of common stock underlying options held by Mr. Ganot’s wife that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (9) Consists of (a) 299,000 shares of common stock owned by Dr. Morris and (b) 194,643 shares of common stock underlying options held by Dr. Morris that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.

- (10) Consists of (a) 335,636 shares of common stock owned by Ms. Ziolkowski and (b) 75,176 shares of common stock underlying options held by Ms. Ziolkowski that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (11) Consists of (a) 270,270 shares of common stock owned by Mr. Smith and (b) 350,582 shares of common stock underlying options held by Mr. Smith that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (12) Consists of (a) 3,933,429 shares of common stock owned by Mr. Arnold and (b) 20,000 shares of common stock underlying options held by Mr. Arnold that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (13) Consists of (a) 53,763 shares of common stock owned by Dr. Freed and (b) 23,333 shares of common stock underlying options held by Dr. Freed that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (14) Consists of 20,000 shares of common stock underlying options held by Mr. Huffines that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (15) 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. In addition, the amount consists of 20,000 shares of common stock underlying options held by Dr. Koppel that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (16) Consists of (a) 34,258 shares of common stock owned by Dr. Nagendran and (b) 23,333 shares of common stock underlying options held by Dr. Nagendran that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (17) Consists of shares held by RA Capital as described in Footnote (2) above. Mr. Shah disclaims beneficial ownership of all shares held by RA Capital Fund and Blackwell, except to the extent of his pecuniary interest therein, and disclaims any pecuniary interest in the shares held by Blackwell Partners LLC – Series A. The address for each of RA Capital Fund, Blackwell, and RA Capital is c/o 200 Berkeley Street, 18th Floor, Boston, MA 02116. Entities affiliated with RA Capital report that they hold shared voting power and shared dispositive power with respect to all shares held by them. In addition, the amount consists of 20,000 shares of common stock underlying options held by Mr. Shah that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (18) 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. In addition, the amount consists of 20,000 shares of common stock underlying options held by Mr. Stone that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (19) Consists of 20,000 shares of common stock underlying options held by Ms. Sullivan that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.
- (20) Includes 1,013,478 shares of common stock underlying options that are exercisable as of February 16, 2021 or will become exercisable within 60 days after such date.

Securities Authorized for Issuance under Equity Compensation Plans

The following table provides information about our equity compensation plans as of December 31, 2020.

| Plan Category | (a) Number of securities to be issued upon the exercise of outstanding options, warrants and rights | (b) Weighted-average exercise price of outstanding options, warrants and rights (2) | (c) Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) |
|---|--|--|--|
| Equity compensation plans approved by security holders ⁽¹⁾ | 4,036,170 | \$ 14.58 | 2,227,659 |
| Equity compensation plans not approved by security holders | - | - | - |
| Total | 4,036,170 | \$ 14.58 | 2,227,659 |

(1) Reflects shares issuable upon exercise of options and settlement of RSUs.

(2) The weighted-average exercise price does not include RSUs, which have no exercise price.

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

In addition to the executive officer and director compensation arrangements discussed above under “Compensation of our executive officers and directors,” we describe transactions since January 1, 2019 to which we have been or will be a participant, in which the amount involved in the transaction exceeds the lesser of \$120,000 or 1% of our total assets at year-end for each of the last two completed fiscal years 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.

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 of our stockholders, or the Investors, which includes our 5% Security Holders and entities affiliated with certain of our directors. The Registration Rights Agreement provides the Investors the right, subject to certain conditions, to demand that we file a registration statement or to request that their shares be covered by a registration statement that we are otherwise filing.

Private placement

On July 25, 2019, we entered into a definitive agreement with respect to the private placement of (i) 10,607,525 shares of our common stock at a price per share of \$4.65 and (ii) 2,295,699 pre-funded warrants to purchase shares of our common stock at a price per warrant of \$4.64, to a group of accredited investors. Each pre-funded warrant is exercisable for one share of common stock at an exercise price of \$0.01 and the pre-funded warrants have no expiration date. We completed this private placement on July 30, 2019, resulting in approximately \$60.0 million in gross proceeds to us, before deducting offering costs of \$2.1 million. The number of shares that each of our directors, executive officers and holders of more than 5% of our voting securities purchased and the aggregate purchase price paid for such shares is set forth in the table below.

| Name (1) | Number of Shares of Common Stock Purchased | Number of Warrant Shares Underlying Pre-Funded Warrants Purchased | Purchase Price |
|---|--|---|-------------------|
| DTMG Bermuda Limited | 903,226 | | \$ 4,200,000.90 |
| Perceptive Life Sciences Master Fund, Ltd. | 2,822,581 | | \$ 13,125,001.65 |
| BCLS SB Investco, LP | 1,881,720 | | \$ 8,749,998.00 |
| RA Capital Healthcare Fund, L.P. | 1,593,629 | | \$ 7,410,374.85 |
| Blackwell Partners LLC - Series A | 288,091 | | \$ 1,339,623.15 |
| Matthew B. Arnold | 677,419 | | \$ 3,149,998.35 |
| Martin I. Freed | 53,763 | | \$ 249,997.95 |
| Sukumar Nagendran | 32,258 | | \$ 149,999.70 |
| Ilan Ganot | 21,505 | | \$ 99,998.25 |
| Carl Morris | 21,505 | | \$ 99,998.25 |
| MVA Investors, LLC | | 137,370 | \$ 637,396.80 |
| Boxer Capital, LLC | | 2,158,329 | \$ 10,014,646.56 |

On December 15, 2020, we entered into a definitive agreement with respect to the private placement of 24,324,320 shares of our common stock at a price per share of \$3.70. We completed this private placement on December 15, 2020, resulting in approximately \$90.0 million in gross proceeds to us, before deducting offering costs of \$3.8 million. The number of shares that each of our directors, executive officers and holders of more than 5% of our voting securities purchased and the aggregate purchase price paid for such shares is set forth in the table below.

| Name (1) | Number of Shares of Common Stock Purchased | Purchase Price |
|---|--|------------------|
| RA Capital Healthcare Fund, L.P. | 4,938,282 | \$ 18,271,643.40 |
| Blackwell Partners LLC - Series A | 467,123 | \$ 1,728,355.10 |
| Perceptive Life Sciences Master Fund, Ltd | 4,054,054 | \$ 14,999,999.80 |
| BCLS SB Investco, LP | 3,189,189 | \$ 11,799,999.30 |
| EcoR1 Capital Fund, L.P. | 421,622 | \$ 1,560,001.40 |
| EcoR1 Capital Fund Qualified, L.P. | 2,281,080 | \$ 8,439,996.00 |
| Boxer Capital, LLC | 2,702,702 | \$ 9,999,997.40 |
| Matthew Arnold | 432,432 | \$ 1,599,998.40 |
| Ian Smith | 270,270 | \$ 999,999.00 |
| Ilan Ganot | 27,027 | \$ 99,999.90 |

(1) See “Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters” for more information about the shares held by the above identified entities.

Ultragenyx collaboration agreement

In October 2020, we entered into a collaboration and license agreement, or the Collaboration Agreement, with Ultragenyx Pharmaceutical Inc., or Ultragenyx, pursuant to which we granted Ultragenyx an exclusive worldwide license under certain intellectual property rights controlled by us. In connection with the execution of the Collaboration Agreement, we also entered into a stock purchase agreement with Ultragenyx, pursuant to which we issued and sold 7,825,797 shares of our common stock to Ultragenyx for an aggregate purchase price of approximately \$40 million, resulting in Ultragenyx becoming a holder of more than 5% of our outstanding common stock. For a further description of the Collaboration Agreement with Ultragenyx, see “Business—Strategic partnerships and collaborations/licenses—Ultragenyx Collaboration Agreement.”

Other arrangements

We employ Annie Ganot, one of our Co-Founders and 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 and bonus payments of less than \$250,000.

In respect of his services as a consultant to us for the three years ended December 31, 2018, on January 2, 2019, we granted Dr. Zarur an option to purchase 100,000 shares of our common stock. In respect of his services as a consultant to us for the year ended December 31, 2019, (i) on January 2, 2019, we granted Dr. Zarur an option to purchase 10,000 shares of our common stock, and (ii) we paid him \$100,000. In respect of his services as a consultant to us for the year ending December 31, 2020, (i) on January 2, 2020, we granted Dr. Zarur an option to purchase 10,000 shares of our common stock, and (ii) we paid him \$58,000.

In connection with the termination of his employment, we entered into a consulting agreement with Jorge Quiroz, M.D., our former Chief Medical Officer, effective as of January 15, 2020, pursuant to which Dr. Quiroz assisted with the transition of his duties to our executive management team. Dr. Quiroz was compensated at a rate of \$500 per hour for his services under the consulting agreement. The term of the consulting agreement continued until July 15, 2020.

In connection with the termination of his employment, we entered into a consulting agreement with Alvaro Amorrortu, our former Chief Operating Officer, effective as of January 15, 2020, pursuant to which Mr. Amorrortu assisted with the transition of his duties to our executive management team. Mr. Amorrortu was compensated at a rate of \$500 per hour for his services under the consulting agreement. The term of the consulting agreement continued until July 15, 2020.

In respect of his services as a consultant to us for the year ended December 31, 2020, (i) on February 10, 2020, we granted Mr. Smith an option to purchase 60,000 shares of our common stock and (ii) on June 16, 2020, we granted Mr. Smith an option to purchase 200,000 shares of our common stock. In respect of his services as a consultant to us for the year ending December 31, 2021, on January 4, 2021, we granted Mr. Smith an option to purchase 389,000 shares of our common stock.

In November 2020, we entered into a consulting agreement with Danforth Advisors, LLC, or Danforth, an affiliate of Stephen DiPalma, our interim chief financial officer. Pursuant to the consulting agreement, Danforth provides us with the chief financial officer services of Mr. DiPalma, and other services, including financial planning, offering support and accounting services, in exchange for fees payable to Danforth based on hourly rates. We have paid Danforth approximately \$0.1 million to date. In accordance with the consulting agreement, in November 2020, we issued to Danforth a warrant to purchase 30,000 shares of our common stock at an exercise price per share of \$3.29. The consulting agreement may be terminated by either party without cause upon 60 days' prior written notice to the other party and with cause upon 30 days' prior written notice to the other party.

Indemnification agreements

We have entered into agreements to indemnify our directors and executive officers. These agreements require us, among other things, 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.

Policy for approval of related-person transactions

We have adopted a written related-person transaction policy that sets forth our procedures for the identification, review, consideration and approval or ratification 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 will be reviewed and approved by the compensation committee of our board of directors in the manner specified in its charter.

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. Audit committee members must also satisfy independence criteria set forth in Rule 10A-3 under the Exchange Act and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under 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 or her 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. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: (1) the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and (2) whether the director is affiliated with the company or any of its subsidiaries or affiliates.

Our board of directors has determined that all members of the board of directors, except Ilan Ganot and Ian Smith, 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. Our board of directors previously made a determination that Andrey Zarur, who served as a member of our board of directors until June 2020, was not an independent director.

Our board of directors has determined that the composition of our committees currently complies with all applicable independence requirements of Nasdaq and the rules and regulations of the SEC.

Item 14. Principal Accounting Fees and Services.

The following table represents aggregate fees billed to us by PricewaterhouseCoopers LLP, or PwC, for the fiscal years ended December 31, 2020 and 2019:

| | 2020 | 2019 |
|--------------------|-------------------|-------------------|
| Audit fees | \$ 690,000 | \$ 646,500 |
| Audit-related fees | - | - |
| Tax fees | 36,290 | 20,148 |
| All other fees | 2,756 | 2,756 |
| Total | \$ 729,046 | \$ 669,404 |

The services rendered by PwC in connection with the fees presented above were as follows:

Audit Fees

Audit fees consist of fees billed for professional services for the audit of our annual consolidated financial statements, the review of interim consolidated financial statements, and related services that are normally provided in connection with registration statements.

Audit-Related Fees

Audit-related fees consist of fees billed for assurance and related services that are reasonably related to the performance of the audit or review of our consolidated financial statements.

Tax Fees

Tax fees consist of fees for professional services related to tax compliance and consultations.

All other fees

All other fees include license fees for web-based accounting research tools.

Pre-approval policies

The audit committee has not adopted policies and procedures for the pre-approval of audit and non-audit services rendered by our independent registered public accounting firm and, consequently, all audit and non-audit services are pre-approved by the whole audit committee or the chair of the audit committee. All fees for the fiscal years ended December 31, 2020 and 2019 were so pre-approved.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(1) Financial Statements:

| | Page |
|--|-------------|
| Report of Independent Registered Public Accounting Firm | F-1 |
| Consolidated Balance Sheets at December 31, 2020 and 2019 | F-2 |
| Consolidated Statements of Operations for the Years Ended December 31, 2020, 2019 and 2018 | F-3 |
| Consolidated Statements of Comprehensive Loss for the Years Ended December 31, 2020, 2019 and 2018 | F-4 |
| Consolidated Statements of Preferred Units and Stockholders'/Members' Equity/(Deficit) for the Years Ended December 31, 2020, 2019 and 2018 | F-5 |
| Consolidated Statements of Cash Flows for the Years Ended December 31, 2020, 2019 and 2018 | F-6 |
| Notes to Consolidated Financial Statements | F-7 |

(2) Financial Statement Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits. The following is a list of exhibits filed as part of this Annual Report on Form 10-K.

| Exhibit Number | Description |
|----------------|---|
| 2.1 | Agreement and Plan of Merger, dated March 29, 2017, by and between Solid Biosciences, LLC and Solid GT, LLC (incorporated by reference to Exhibit 2.1 to the Registration Statement on Form S-1 filed on December 29, 2017). |
| 2.2 | Plan of Conversion, dated January 25, 2018 (incorporated by reference to Exhibit 2.2 to the Annual Report on Form 10-K filed on March 29, 2018). |
| 2.3 | Agreement and Plan of Merger, dated January 25, 2018, by and among Solid Biosciences Inc., Bain Capital Life Sciences Fund, L.P., BCIP Life Sciences Associates, LP, BCLS Solid Bio, Inc., Foresite Capital Fund III, L.P. and FC Fund III Solid Holdings, Inc. (incorporated by reference to Exhibit 2.3 to the Annual Report on Form 10-K filed on March 29, 2018). |
| 3.1 | Certificate of Incorporation of Solid Biosciences Inc. (incorporated by reference to Exhibit 4.1 to the Registration Statement on Form S-8 filed on January 29, 2018). |
| 3.2 | Bylaws of Solid Biosciences Inc. (incorporated by reference to Exhibit 4.2 to the Registration Statement on Form S-8 filed on January 29, 2018). |
| 4.1 | Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registration Statement on Form S-1 filed on December 29, 2017). |
| 4.2 | Description of the Company's Securities Registered under Section 12 of the Exchange Act (incorporated by reference to Exhibit 4.2 to the Annual Report on Form 10-K filed on March 12, 2020). |
| 10.1† | Amended and Restated Registration Rights Agreement dated March 29, 2017 by and among Solid Biosciences, LLC and certain investors (incorporated by reference to Exhibit 10.17 to the Registration Statement on Form S-1 filed on December 29, 2017). |
| 10.2† | Employment Agreement, dated as of January 25, 2019, by and between Solid Biosciences Inc. and Ilan Ganot. (incorporated by reference to Exhibit 10.2 to the Annual Report on Form 10-K filed on March 13, 2019) |
| 10.3*† | Employment Agreement, dated as of January 25, 2019, by and between Solid Biosciences Inc. and Jennifer Ziolkowski. |
| 10.4† | Employment Agreement, dated as of January 25, 2019, by and between Solid Biosciences Inc. and Carl Morris. Ph.D. (incorporated by reference to Exhibit 10.4 to the Annual Report on Form 10-K filed on March 13, 2019). |
| 10.5† | Solid Biosciences, LLC Amended and Restated Equity Incentive Plan and form of unit restriction agreement (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 29, 2018). |
| 10.6† | Solid Biosciences Inc. 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 99.1 to the Registration Statement on Form S-8 filed on January 29, 2018). |
| 10.7† | Form of Incentive Stock Option Agreement under 2018 Omnibus Incentive Plan. (incorporated by reference to Exhibit 10.7 to the Annual Report on Form 10-K filed on March 13, 2019). |
| 10.8† | Form of Nonqualified Stock Option Agreement under 2018 Omnibus Incentive Plan. (incorporated by reference to Exhibit 10.8 to the Annual Report on Form 10-K filed on March 13, 2019). |
| 10.9† | Form of Restricted Stock Agreement under 2018 Omnibus Incentive Plan (incorporated by reference to Exhibit 10.9 to the Registration Statement on Form S-1 filed on December 29, 2017). |
| 10.10# | Exclusive Patent License Agreement, dated as of October 16, 2015, by and between Solid GT, LLC and the University of Washington (incorporated by reference to Exhibit 10.10 to the Registration Statement on Form S-1 filed on December 29, 2017). |
| 10.11# | Patent License Agreement, dated as of March 10, 2016, by and between Solid GT, LLC and the Regents of the University of Michigan (incorporated by reference to Exhibit 10.11 to Amendment No. 1 to the Registration Statement on Form S-1 filed on January 16, 2018). |
| 10.12# | License Agreement, dated as of October 15, 2015, by and between Solid GT, LLC and The Curators of the University of Missouri (incorporated by reference to Exhibit 10.12 to Amendment No. 1 to the Registration Statement on Form S-1 filed on January 16, 2018). |
| 10.13# | Cell Line License Agreement, dated as of November 20, 2016, by and between Solid Biosciences, LLC and Life Technologies Corporation (incorporated by reference to Exhibit 10.13 to Amendment No. 1 to the Registration Statement on Form S-1 filed on January 16, 2018). |

- 10.14# License Agreement, dated as of June 23, 2016, by and between Solid GT, LLC and the President and Fellows of Harvard College (incorporated by reference to Exhibit 10.14 to the Registration Statement on Form S-1 filed on December 29, 2017).
- 10.15# License Agreement, dated as of August 3, 2017, by and between Solid Biosciences, LLC and the President and Fellows of Harvard College (incorporated by reference to Exhibit 10.15 to the Registration Statement on Form S-1 filed on December 29, 2017).
- 10.16 First Amendment to Patent License Agreement, dated as of March 15, 2017, by and between Solid GT, LLC and the Regents of the University of Michigan (incorporated by reference to Exhibit 10.16 to Amendment No. 1 to the Registration Statement on Form S-1 filed on January 16, 2018).
- 10.17 Form of Indemnification Agreement for Directors and Officers (incorporated by reference to Exhibit 10.16 to the Registration Statement on Form S-1 filed on December 29, 2017).
- 10.18 Sublease, dated as of January 30, 2018, by and between Solid Biosciences, LLC and Twitter, Inc. (incorporated by reference to Exhibit 10.20 to the Annual Report on Form 10-K filed on March 29, 2018).
- 10.19 Lease Agreement, dated as of December 22, 2017, by and between Solid Biosciences, LLC and ARE-MA Region No. 59, LLC (incorporated by reference to Exhibit 10.21 to the Annual Report on Form 10-K filed on March 29, 2018).
- 10.20† Summary of Non-Employee Director Compensation Program. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on August 6, 2020).
- 10.21 Securities Purchase Agreement, dated July 25, 2019, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on July 26, 2019).
- 10.22 Form of Pre-Funded Warrant to Purchase Common Stock to be issued pursuant to the Securities Purchase Agreement (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on July 26, 2019).
- 10.23 Registration Rights Agreement, dated July 25, 2019, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.3 to the Current Report on Form 8-K filed on July 26, 2019).
- 10.24*† Consulting Agreement, dated as of November 19, 2020, by and between Solid Biosciences Inc. and Danforth Advisors, LLC.
- 10.25† Consulting Agreement, dated as of February 1, 2020, by and between Solid Biosciences Inc. and Ian F. Smith (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on May 7, 2020).
- 10.26† Consulting Agreement, dated as of June 16, 2020, by and between Solid Biosciences Inc. and Ian F. Smith (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on August 6, 2020).
- 10.27† 2020 Equity Incentive Plan (incorporated by reference to Exhibit 99.1 to the Current Report on Form 8-K filed on June 18, 2020).
- 10.28† Form of Stock Option Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on August 6, 2020).
- 10.29† Form of Restricted Stock Unit Agreement under the 2020 Equity Incentive Plan (incorporated by reference to Exhibit 10.5 to the Quarterly Report on Form 10-Q filed on August 6, 2020).
- 10.30# Collaboration and License Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.1 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
- 10.31 Stock Purchase Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.2 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
- 10.32# Investor Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc. (incorporated by reference to Exhibit 10.3 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
- 10.33# First Amendment, dated as of October 9, 2020, to the Exclusive Patent License by and between the Company and the University of Washington (incorporated by reference to Exhibit 10.4 to the Quarterly Report on Form 10-Q filed on November 5, 2020).
- 10.34 Securities Purchase Agreement, dated December 10, 2020, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.1 to the Current Report on Form 8-K filed on December 11, 2020).
- 10.35 Registration Rights Agreement, dated December 10, 2020, by and among the Company and the other parties thereto (incorporated by reference to Exhibit 10.2 to the Current Report on Form 8-K filed on December 11, 2020).
- 10.36*# First Amendment, dated as of January 27, 2021, to the Exclusive Patent License by and between the Company and the Curators of the University of Missouri

| | |
|----------|--|
| 21.1* | Subsidiaries of Solid Biosciences Inc. |
| 23.1* | Consent of PricewaterhouseCoopers LLP, Independent Registered Public Accounting Firm. |
| 31.1* | Certification of Chief Executive Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. |
| 31.2* | Certification of Chief Financial Officer of the Registrant Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002. |
| 32.1** | Certification of Chief Executive Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. |
| 32.2** | Certification of Chief Financial Officer of the Registrant Pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002. |
| 101.INS | Inline XBRL Instance Document. |
| 101.SCH | Inline XBRL Taxonomy Extension Schema Document. |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Linkbase Document. |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase Document. |
| 1010.LAB | Inline XBRL Taxonomy Extension Label Linkbase Document. |
| 101.PRE | Inline XBRL Taxonomy Extension Presentation Linkbase Document. |
| 104 | Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101). |

* Filed herewith.

** Furnished herewith.

† Indicates management contract or compensatory plan.

Certain portions of this exhibit have been omitted because they are not material and contain information that the Registrant customarily and actually treats as private or confidential.

Item 16. Form 10-K Summary.

Not applicable.

Report of Independent Registered Public Accounting Firm

To the Board of Directors and Stockholders of Solid Biosciences Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Solid Biosciences Inc. and its subsidiaries (the “Company”) as of December 31, 2020 and 2019, and the related consolidated statements of operations, of comprehensive loss, of preferred units and stockholders’/members’ equity (deficit) and of cash flows for each of the three years in the period ended December 31, 2020, including the related notes (collectively referred to as the “consolidated financial statements”). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2020 and 2019, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2020 in conformity with accounting principles generally accepted in the United States of America.

Substantial Doubt About the Company’s Ability to Continue as a Going Concern

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 losses and negative cash flows from operations since inception, 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 consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Change in Accounting Principle

As discussed in Note 2 to the consolidated financial statements, the Company changed the manner in which it accounts for leases in 2019.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company’s management. Our responsibility is to express an opinion on the Company’s consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits of these consolidated financial statements in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company’s internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ PricewaterhouseCoopers LLP
Boston, Massachusetts
March 15, 2021

We have served as the Company’s auditor since 2017.

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

| | December 31, | |
|---|---------------------|-------------------|
| | 2020 | 2019 |
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 154,744 | \$ 76,043 |
| Available-for-sale securities | - | 7,481 |
| Prepaid expenses and other current assets | 4,157 | 2,778 |
| Total current assets | 158,901 | 86,302 |
| Operating lease, right of use asset | 3,579 | 4,988 |
| Property and equipment, net | 8,153 | 11,645 |
| Other non-current assets | 209 | 209 |
| Restricted cash | 327 | 327 |
| Total assets | <u>\$ 171,169</u> | <u>\$ 103,471</u> |
| Liabilities and Stockholders' Equity | | |
| Current liabilities: | | |
| Accounts payable | \$ 3,274 | \$ 7,124 |
| Accrued expenses | 8,640 | 9,178 |
| Operating lease liabilities | 1,999 | 1,736 |
| Finance lease liabilities | 208 | 186 |
| Deferred revenue - related party | 10,359 | - |
| Other current liabilities | - | 52 |
| Total current liabilities | 24,480 | 18,276 |
| Operating lease liabilities, excluding current portion | 2,419 | 4,414 |
| Finance lease obligations, excluding current portion | 525 | 733 |
| Deferred revenue - related party, excluding current portion | 10,359 | - |
| Other non-current liabilities | 1,300 | - |
| Total liabilities | 39,083 | 23,423 |
| Commitments and Contingencies (Note 13) | | |
| Stockholders' Equity: | | |
| Preferred stock, \$0.001 par value; 10,000,000 shares authorized at December 31, 2020 and December 31, 2019; no shares issued and outstanding at December 31, 2020 and December 31, 2019 | - | - |
| Common stock, \$0.001 par value; 300,000,000 shares authorized at December 31, 2020 and December 31, 2019; 84,893,994 shares issued and outstanding at December 31, 2020 and 45,987,571 shares issued and outstanding at December 31, 2019; 2,158,329 pre-funded warrants outstanding at December 31, 2020 and 2,295,699 pre-funded warrants outstanding at December 31, 2019 | 87 | 48 |
| Additional Paid-in Capital | 536,568 | 396,278 |
| Accumulated other comprehensive gain (loss) | - | 1 |
| Accumulated deficit | (404,569) | (316,279) |
| Total stockholders' equity | 132,086 | 80,048 |
| Total liabilities and stockholders' equity | <u>\$ 171,169</u> | <u>\$ 103,471</u> |

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

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

| | Year Ended December 31, | | |
|--|--------------------------------|--------------|-------------|
| | 2020 | 2019 | 2018 |
| Revenue | \$ - | \$ - | \$ - |
| Operating expenses: | | | |
| Research and development | 64,881 | 94,737 | 57,965 |
| General and administrative | 21,581 | 24,581 | 17,722 |
| Restructuring expense | 1,944 | - | - |
| Total operating expenses | 88,406 | 119,318 | 75,687 |
| Loss from operations | (88,406) | (119,318) | (75,687) |
| Other income (expense): | | | |
| Interest income | 115 | 1,580 | 619 |
| Other income | 1 | 515 | 270 |
| Total other income (expense), net | 116 | 2,095 | 889 |
| Net loss | \$ (88,290) | \$ (117,223) | \$ (74,798) |
| Net loss per share, basic and diluted | \$ (1.70) | \$ (2.91) | \$ (2.25) |
| Weighted average common stock outstanding, basic and diluted | 51,937,147 | 40,289,290 | 33,262,597 |

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

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS
(In thousands)

| | Year Ended December 31, | | |
|---|-------------------------|--------------|-------------|
| | 2020 | 2019 | 2018 |
| Net loss | \$ (88,290) | \$ (117,223) | \$ (74,798) |
| Other comprehensive income (loss): | | | |
| Unrealized gain (loss) on available-for-sale securities | (1) | 6 | 8 |
| Comprehensive loss | \$ (88,291) | \$ (117,217) | \$ (74,790) |

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

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF PREFERRED UNITS AND STOCKHOLDERS'/MEMBERS' EQUITY/(DEFICIT)
(In thousands except for share data)

| | Series 2 Senior Preferred Units | | Series 1 Senior Preferred Units | | Junior Preferred Units | | Series A, B, C and D Common Units | | Common Stock | Additional paid in capital | Accumulated other comprehensive income (loss) | Accumulated Deficit | Total Members'/ Stockholders' Equity (Deficit) |
|---|---------------------------------|----------|---------------------------------|----------|------------------------|----------|-----------------------------------|------------|--------------|----------------------------|---|---------------------|--|
| | Amount | Units | Amount | Units | Amount | Units | Amount | Units | | | | | |
| Balance at December 31, 2017 | 4,886,000 | 55,002 | 2,500,000 | 25,000 | 4,414,356 | 44,177 | 19,438,552 | 65,014 | - | - | (13) | \$(124,258) | \$(59,257) |
| Conversion of units into shares of common stock | (4,886,000) | (55,002) | (2,500,000) | (25,000) | (4,414,356) | (44,177) | (19,429,620) | (65,180) | 26,498,559 | 189,333 | - | - | 124,179 |
| Issuance of common stock upon initial public offering, net of issuance costs of \$4,592 | - | - | - | - | - | - | - | - | 8,984,375 | 129,087 | - | - | 129,096 |
| Equity-based compensation | - | - | - | - | - | - | 166 | - | 5,789 | - | - | - | 5,955 |
| Net loss | - | - | - | - | - | - | - | - | - | - | - | (74,798) | (74,798) |
| Forfeiture of restricted stock/unit awards | - | - | - | - | - | - | (8,932) | - | (50,474) | - | - | - | - |
| Unrealized gain on available-for-sale securities | - | - | - | - | - | - | - | - | \$ | - | 8 | - | 8 |
| Balance at December 31, 2018 | - | - | - | - | - | - | - | 35,432,460 | 35 | 324,209 | (5) | (199,056) | 125,183 |
| Equity-based compensation | - | - | - | - | - | - | - | - | 14,207 | - | - | - | 14,207 |
| Sale of common stock, net of issuance costs of \$2,102 | - | - | - | - | - | - | - | - | 10,607,525 | 47,212 | - | - | 47,223 |
| Sale of pre-funded warrants | - | - | - | - | - | - | - | - | 2,295,699 | 10,650 | - | - | 10,652 |
| Net loss | - | - | - | - | - | - | - | - | - | - | - | (117,223) | (117,223) |
| Forfeiture of restricted stock awards | - | - | - | - | - | - | - | (52,414) | - | - | - | - | - |
| Unrealized gain on available-for-sale securities | - | - | - | - | - | - | - | - | \$ | 6 | - | - | 6 |
| Balance at December 31, 2019 | - | - | - | - | - | - | - | 48,283,270 | 48 | 396,278 | 1 | \$(316,279) | 80,048 |
| Equity-based compensation | - | - | - | - | - | - | - | - | 11,629 | - | - | - | 11,629 |
| Sale of common stock, net of issuance costs of \$3,790 | - | - | - | - | - | - | - | - | 30,633,952 | 109,387 | - | - | 109,418 |
| Issuance of common stock, to a related party, in connection with the Stock Purchase Agreement | - | - | - | - | - | - | - | - | 7,825,797 | 8 | 19,274 | - | 19,282 |
| Net loss | - | - | - | - | - | - | - | - | - | - | - | (88,290) | (88,290) |
| Vesting of restricted stock units | - | - | - | - | - | - | - | - | 407,700 | - | - | - | - |
| Forfeiture of restricted stock awards | - | - | - | - | - | - | - | (98,396) | - | - | - | - | - |
| Unrealized loss on available-for-sale securities | - | - | - | - | - | - | - | - | - | (1) | - | - | (1) |
| Balance at December 31, 2020 | - | - | - | - | - | - | - | 87,052,323 | 87 | 536,568 | - | \$(404,569) | 132,086 |

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

SOLID BIOSCIENCES INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(In thousands)

| | Year Ended December 31, | | |
|---|-------------------------|------------------|------------------|
| | 2020 | 2019 | 2018 |
| Cash flows from operating activities: | | | |
| Net loss | \$ (88,290) | \$ (117,223) | \$ (74,798) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | | |
| Amortization of (discount)/premium on available-for-sale securities | (20) | (279) | (90) |
| Equity-based compensation expense | 11,629 | 14,207 | 5,955 |
| Depreciation expense | 3,922 | 2,824 | 1,566 |
| Loss on sale of property and equipment | - | 2 | 4 |
| Changes in operating assets and liabilities: | | | |
| Proceeds from landlord lease incentive for tenant improvements | - | - | 823 |
| Prepaid expenses and other current and non-current assets | 30 | 4,486 | (4,930) |
| Accounts payable | (3,431) | 3,779 | (1,610) |
| Accrued expenses and other current and non-current liabilities | (1,157) | (510) | 2,883 |
| Deferred revenue - related party, current and non-current | 20,718 | - | - |
| Net cash used in operating activities | <u>(56,599)</u> | <u>(92,714)</u> | <u>(70,197)</u> |
| Cash flows from investing activities: | | | |
| Purchases of property and equipment | (899) | (4,387) | (7,776) |
| Proceeds from sales and maturities of available-for-sale securities | 7,900 | 60,399 | 25,335 |
| Purchases of available-for-sale securities | (401) | (31,496) | (44,321) |
| Net cash provided by (used in) investing activities | <u>6,600</u> | <u>24,516</u> | <u>(26,762)</u> |
| Cash flows from financing activities: | | | |
| Proceeds from the issuance of common stock to a related party in connection with the Stock Purchase Agreement | 19,282 | - | - |
| Proceeds from issuance of common stock | 113,208 | 49,325 | - |
| Payment of offering costs | (3,790) | (2,102) | (2,168) |
| Proceeds from issuance of pre-funded warrants | - | 10,652 | - |
| Proceeds from initial public offering of common stock, net of commissions and underwriting discounts | - | - | 133,688 |
| Principal payments under capital lease obligation | - | - | (13) |
| Net cash provided by financing activities | <u>128,700</u> | <u>57,875</u> | <u>131,507</u> |
| Net (decrease) increase in cash, cash equivalents and restricted cash | 78,701 | (10,323) | 34,548 |
| Cash, cash equivalents, and restricted cash at beginning of period | 76,370 | 86,693 | 52,145 |
| Cash, cash equivalents, and restricted cash at end of period | <u>\$ 155,071</u> | <u>\$ 76,370</u> | <u>\$ 86,693</u> |
| Supplemental disclosure of non-cash investing and financing activities: | | | |
| Conversion of Series A, B, C and D common units into shares of common stock | - | - | \$ 65,180 |
| Conversion of Series 2 Senior Preferred units into shares of common stock | - | - | \$ 55,002 |
| Conversion of Series 1 Senior Preferred units into shares of common stock | - | - | \$ 25,000 |
| Conversion of Junior Preferred units into shares of common stock | - | - | \$ 44,177 |
| Property and equipment included in accounts payable and accruals | \$ 20 | \$ 490 | \$ 952 |
| Property and equipment acquired through a capital lease | - | - | \$ 1,023 |

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

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

1. Nature of the Business and Basis of Presentation

Nature of Business

Solid Biosciences Inc. was organized in March 2013 under the name SOLID Ventures Management, LLC. In October 2013, the company changed its name to Solid Ventures, LLC and in June 2015, the company changed its name to Solid Biosciences, LLC. The company operated as a Delaware limited liability company under the name Solid Biosciences, LLC until immediately prior to the effectiveness of its registration statement on Form S-1 on January 25, 2018, at which time it completed a statutory corporate conversion into a Delaware corporation (the “Corporate Conversion”) and changed its name to Solid Biosciences Inc. (the “Company”) In addition, entities formed solely for the purpose of holding membership interests in the Company’s limited liability company were merged with and into the Company. As a result of the Corporate Conversion, all of the Series 1 and 2 Senior Preferred, Junior Preferred Units, Series A, B, C and D Common Units of Solid Biosciences, LLC converted into shares of common stock of Solid Biosciences Inc. on a one for 0.8485 basis and all of the unit holders of Solid Biosciences, LLC became holders of common stock of Solid Biosciences Inc.

The Company’s mission is to cure Duchenne muscular dystrophy (“Duchenne”), a genetic muscle-wasting disease predominantly affecting boys. It is caused by mutations in the dystrophin gene, which result in the absence or near-absence of dystrophin protein. Dystrophin protein works to strengthen muscle fibers and protect them from daily wear and tear. Without functioning dystrophin and certain associated proteins, muscles suffer excessive damage from normal daily activities and are unable to regenerate, leading to the build-up of fibrotic, or scar, and fat tissue. The Company’s lead product candidate, SGT-001, is a gene transfer candidate under investigation for its ability to drive functional dystrophin protein expression in patients’ muscles and improve the course of the disease. SGT-001 has been granted Rare Pediatric Disease Designation and Fast Track in the United States and Orphan Drug Designations in both the United States and European Union. The Company filed an Investigational New Drug application (“IND”) in September 2017 and initiated a Phase I/II clinical trial for SGT-001 in the United States during the fourth quarter of 2017, which is called IGNITE DMD. In November 2019, IGNITE DMD was placed on clinical hold by the U.S. Food and Drug Administration (“FDA”). In April 2020, the Company submitted a response to the FDA that included changes to the clinical protocol designed to enhance patient safety, as well as information related to improvements to its manufacturing process. The FDA responded by maintaining the clinical hold and requesting further data and analyses relating to this manufacturing process. In June 2020, the Company submitted a response to the FDA that provided data related to manufacturing process improvements. In July 2020, the Company announced that the FDA responded by maintaining the clinical hold and requesting further manufacturing information and updated safety and efficacy data for all patients dosed in the trial, as well as providing direction on the total viral load to be administered per patient. In October 2020, the Company announced that the FDA lifted the clinical hold placed on the Company’s IGNITE DMD Phase I/II clinical trial. The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry, including, but not limited to, development by competitors of new technological innovations, dependence on licenses, protection of proprietary technology, dependence on key personnel, compliance with government regulations and the need to obtain additional financing to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive pre-clinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting capabilities.

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

Initial Public Offering in January 2018

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

Liquidity

The accompanying 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, 2020, the Company has funded its operations primarily with the proceeds from the sale of redeemable preferred units and member units as well as the sale of common stock and prefunded warrants to purchase shares of its common stock in private placements and the sale of common stock in its initial public offering.

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

In October 2020, the Company sold 6,309,632 shares of common stock pursuant to the ATM Sales Agreement resulting in net proceeds to the Company of \$23,209.

On December 15, 2020, the Company issued and sold in a private placement 24,324,320 shares of its common stock at a price per share of \$3.70. The Company received gross proceeds from the private placement of approximately \$90,000 before deducting placement agent fees and offering expenses of \$3,790.

In accordance with ASC 205-40, Going Concern, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the financial statements are issued. As of December 31, 2020, the Company had an accumulated deficit of \$404,569. During the year ended December 31, 2020, the Company incurred a net loss of \$88,290 and used \$56,599 of cash in operations. The Company expects to continue to generate operating losses in the foreseeable future. Based upon its current operating plan, the Company expects that its cash and cash equivalents of \$154,744 as of December 31, 2020, will be sufficient to fund its operating expenses and capital requirements into the first quarter of 2022.

In accordance with the requirements of ASC 205-40, the Company determined that there is substantial doubt about the Company's ability to continue as a going concern within twelve months of the issuance date of these financial statements. The financial statements do not include any adjustments that might result from the outcome of this uncertainty. Although the Company has been successful in raising capital in the past, there is no assurance that it will be successful in obtaining such additional financing on terms acceptable to the Company, if at all, nor is it considered probable under the accounting standards. As such, under the requirements of ASC 205-40, management may not consider the potential for future capital raises or management plans to reduce costs that are not considered probable in its assessment of the Company's ability to meet its obligations for the next twelve months. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its research and development programs, pre-clinical and clinical testing or commercialization efforts, which could adversely affect its business prospects.

2. Summary of Significant Accounting Policies

Use of Estimates

The preparation of the Company's consolidated financial statements in conformity with GAAP requires management to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the consolidated financial statements and the reported amounts of revenues and expenses during the reporting periods. Significant estimates and assumptions reflected in these consolidated financial statements include, but are not limited to, estimates related to revenue recognition, the recognition of research and development expenses and equity-based compensation. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Changes in estimates are recorded in the period in which they become known. Actual results could differ from the Company's estimates.

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

Cash Equivalents

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

Restricted Cash

The Company held restricted cash of \$327 in separate restricted bank accounts as security deposits for leases of the Company's facilities as of December 31, 2020 and December 31, 2019. The Company has included restricted cash of \$327 as a non-current asset as of December 31, 2020 and December 31, 2019. A reconciliation of the amounts of cash and cash equivalents and restricted cash from the cash flow statement to the balance sheet is as follows:

| | December 31, 2020 | December 31, 2019 | December 31, 2018 |
|---|----------------------|----------------------|----------------------|
| Cash and cash equivalents | \$ 154,744 | \$ 76,043 | \$ 86,366 |
| Restricted cash, non-current | 327 | 327 | 327 |
| Cash and cash equivalents and restricted cash | <u>\$ 155,071</u> | <u>\$ 76,370</u> | <u>\$ 86,693</u> |

Available-for-Sale Securities

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

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

Concentration of Credit Risk and of Significant Suppliers

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

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

Fair Value Measurements

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

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

The Company's cash equivalents and available-for-sale securities are carried at fair value, determined according to the fair value hierarchy described above. See Note 5, *Fair Value of Financial Assets and Liabilities*, for additional information. The carrying values of the Company's accounts payable and accrued expenses and other current liabilities approximate their fair value due to the short-term nature of these liabilities.

Leases

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

Property and Equipment

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

Impairment of Long-Lived Assets

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

Revenue Recognition

The Company adopted ASC Topic 606, Revenue from Contracts with Customers (“ASC 606”) on January 1, 2017. The adoption had no impact as the Company had no revenue generating arrangements until the year ended December 31, 2020. Under ASC 606, an entity recognizes revenue when its customer obtains control of promised goods or services, in an amount that reflects the consideration which the entity expects to receive in exchange for those goods or services. To determine the appropriate amount of revenue to be recognized for arrangements determined to be within the scope of ASC 606, the Company performs the following five steps: (i) identification of the contract(s) with the customer; (ii) identification of the promised goods or services in the contract and determination of whether the promised goods or services are performance obligations; (iii) measurement of the transaction price; (iv) allocation of the transaction price to the performance obligations; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation. The Company only applies the five-step model to contracts when it is probable that the entity will collect consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, the Company assesses the goods or services promised within each contract and determines those that are performance obligations and assesses whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

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

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

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

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

Exclusive Licenses

If the license granted in the arrangement is determined to be distinct from the other promises or performance obligations identified in the arrangement, which generally include research and development services, the Company recognizes revenue from non-refundable, upfront fees allocated to the license when the license is transferred to the customer and the customer is able to use and benefit from the license. In assessing whether a license is distinct from the other promises, the Company considers relevant facts and circumstances of each arrangement, including the research and development capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can benefit from the license for its intended purpose without the receipt of the remaining promise, whether the value of the license is dependent on the unsatisfied promise, whether there are other vendors that could provide the remaining promise, and whether it is separately identifiable from the remaining promise. For licenses that are combined with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time and, if over time, the appropriate method of measuring progress for purposes of recognizing revenue. The Company evaluates the measure of progress each reporting period and, if necessary, adjusts the measure of performance and related revenue recognition. The measure of progress, and thereby periods over which revenue should be recognized, are subject to estimates by management and may change over the course of the arrangement.

Research and Development Services

The promises under the Company's collaboration and license agreements generally include research and development services to be performed by the Company on behalf of the collaboration partner. For performance obligations that include research and development services, the Company generally recognizes revenue allocated to such performance obligations based on an appropriate measure of progress. The Company utilizes judgment to determine the appropriate method of measuring progress for purposes of recognizing revenue, which is generally an input measure, such as costs incurred.

Milestone Payments

At the inception of each arrangement that includes milestone payments based on certain events, the Company evaluates whether the milestones are considered probable of being achieved and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the control of the Company, such as regulatory approvals, are not considered probable of being achieved until those approvals are received. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the particular milestone in making this assessment. There is considerable judgment involved in determining whether it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur. At the end of each subsequent reporting period, the Company reevaluates the probability of achievement of all milestones subject to constraint and, if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect revenues and earnings in the period of adjustment. If a milestone or other variable consideration relates specifically to the Company's efforts to satisfy a single performance obligation or to a specific outcome from satisfying the performance obligation, the Company generally allocates the milestone amount entirely to that performance obligation once it is probable that a significant reversal in the amount of cumulative revenue recognized would not occur.

Royalties

For arrangements that include sales-based royalties, including milestone payments based on a level of sales, and the license is deemed to be the predominant item to which the royalties relate, the Company recognizes revenue at the later of (i) when the related sales occur or (ii) when the performance obligation to which some or all of the royalty has been allocated has been satisfied (or partially satisfied). To date, the Company has not recognized any royalty revenue resulting from any of its licensing arrangements.

Collaboration Revenue

The Company analyzes its collaboration arrangements to assess whether they are within the scope of ASC 808, *Collaborative Arrangements* (“ASC 808”) to determine whether such arrangements involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement. For collaboration arrangements within the scope of ASC 808 that contain multiple elements, the Company first determines which elements of the collaboration are deemed to be within the scope of ASC 808 and those that are more reflective of a vendor-customer relationship and therefore within the scope of ASC 606. For elements of collaboration arrangements that are accounted for pursuant to ASC 808, an appropriate recognition method is determined and applied consistently, generally by analogy to ASC 606. For those elements of the arrangement that are accounted for pursuant to ASC 606, the Company applies the five-step model described above.

Costs Associated with License and Collaborative Arrangements

All costs associated with license and collaborative arrangements are expensed as incurred and recorded in research and development expense in the consolidated statements of operations.

Research and Development Costs

Research and development costs are expensed as incurred. Research and development expenses include salaries, equity-based compensation and benefits of employees, third-party license fees and other operational costs related to the Company’s research and development activities, including allocated facility-related expenses and external costs of outside vendors engaged to conduct both 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 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 all stock options and other stock-based awards granted to employees, directors and non-employees based on the fair value on the date of the grant and recognizes compensation expense of those awards, over the requisite service period, which is generally the vesting period of the respective award. Forfeitures are accounted for as they occur. The Company applies the straight-line method of expense recognition to all awards with only service-based vesting conditions. The Company has not issued any awards with performance-based vesting conditions.

The fair value of each stock option grant is estimated on the date of grant using the Black-Scholes option-pricing model. The Company historically has been a private company and lacks company-specific historical and implied volatility information. Therefore, it estimates its expected stock volatility based on the historical volatility of a publicly traded set of peer companies and expects to continue to do so until such time as it has adequate historical data regarding the volatility of its own traded stock price. For options with service-based vesting conditions, the expected term of the Company's stock options has been determined utilizing the "simplified" method for awards that qualify as "plain-vanilla" options. Through December 31, 2018, the expected term of stock options granted to non-employees is equal to the contractual term of the option award and effective January 1, 2019, the "simplified" method is used. The risk-free interest rate is determined by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. Expected dividend yield is based on the fact that the Company has never paid cash dividends and does not expect to pay any cash dividends in the foreseeable future.

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

Income Taxes

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

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

Segment Data

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

Comprehensive Loss

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

Net Loss per Share

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

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

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

Funding from Charitable Organizations

The Company has received funding from charitable organizations to perform research and development services to identify therapies for people with Duchenne. 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 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 \$1, \$515 and \$270 for the years ended December 31, 2020, 2019 and 2018, respectively, which is included in the consolidated statements of operations.

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

The Company adopted Accounting Standards Update ("ASU") No. 2016-02, *Leases (Topic 842)*, as of January 1, 2019 using the modified retrospective transition approach with no restatement of prior periods or cumulative adjustment to accumulated deficit. Upon adoption, the Company elected the package of transition practical expedients, which allowed the Company to carry forward prior conclusions related to whether any expired or existing contracts are or contain leases, the lease classification for any expired or existing leases and initial direct costs for existing leases. The Company also made an accounting policy election not to recognize leases with an initial term of 12 months or less within its consolidated balance sheets and to recognize those lease payments on a straight-line basis in its consolidated statements of operations over the lease term. Upon adoption of ASU 2016-02, the Company recognized operating lease, right-of-use assets of approximately \$4,600 and a corresponding operating lease liability of approximately \$5,900, which are included in the Company's consolidated balance sheet. The adoption of ASU 2016-02 did not have any impact on the Company's consolidated statements of operations and comprehensive loss.

In August 2018 the Financial Accounting Standards Board issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework - Changes to the Disclosure Requirements for Fair Value Measurement*. This new standard modifies certain disclosure requirements on fair value measurements. This new standard was effective for the Company on January 1, 2020. The adoption of this new standard did not have a material impact on the disclosures.

3. Collaborations

Ultragenyx Collaboration Agreement

Collaboration Agreement

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

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

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

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

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

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

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

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

The Collaboration Agreement continues on a country-by-country and Licensed Product-by-Licensed Product basis until the expiration of all payment obligations under the agreement. With respect to any Licensed Product for which the Company has exercised an Income Share Option, the Collaboration Agreement continues until there are no longer sales of such Licensed

Product in the United States or Europe. Either party has the right to terminate the agreement if the other party has materially breached in the performance of its obligations under the agreement and such breach has not been cured within the applicable cure period. Ultragenyx may also terminate the Collaboration Agreement in its sole discretion upon 90 days' prior written notice to the Company.

Stock Purchase Agreement

In connection with the execution of the Collaboration Agreement, Ultragenyx and the Company also entered into a stock purchase agreement (the "Stock Purchase Agreement") on October 22, 2020 (the "Closing Date"), pursuant to which the Company issued and sold 7,825,797 shares of its common stock (the "Shares") to Ultragenyx at a price of \$5.1113 per share for an aggregate purchase price of approximately \$40,000. The Stock Purchase Agreement contains customary representations, warranties and covenants of each of the parties thereto. Following the sale of the Shares, Ultragenyx beneficially owned approximately 14.45% of the Company's outstanding common stock. As of December 31, 2020, Ultragenyx beneficially owned approximately 9.2% of the Company's outstanding common stock.

Investor Agreement

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

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

Accounting Treatment

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

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

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

milestones relate solely to the license of intellectual property and are therefore excluded from the transaction price under the sales- or usage-based royalty exception of ASC 606. Revenue related to these royalties and sales milestones will only be recognized when the associated sales occur, and relevant thresholds are met.

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

Ultragenyx is a related party since Ultragenyx is one of the Company's significant stockholders. No amounts have been recognized as related party revenue as the Company has not performed any services under the Collaboration Agreement for the year ended December 31, 2020. Further, the Company has made no payments to Ultragenyx during the year ended December 31, 2020. There is no amount due from Ultragenyx as of December 31, 2020. The amount received is deferred as a contract liability on the Company's consolidated balance sheet as the performance obligation has not been fully satisfied as of December 31, 2020. The aggregate amount of the transaction price allocated to the Company's unsatisfied performance obligation is recorded in related party deferred revenue at December 31, 2020.

4. 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.

(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 11, *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 Share*, 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, *Members' Deficit*, 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 10, *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 10, *Members' Deficit*, for additional information.

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 |

5. Fair Value of Financial Assets and Liabilities

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

| | Fair Value Measurements as of December 31, 2020 Using: | | | |
|------------------|--|-----------|---------|-----------|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Cash equivalents | \$ - | \$ 84,462 | \$ - | \$ 84,462 |
| | \$ - | \$ 84,462 | \$ - | \$ 84,462 |

| | Fair Value Measurements as of December 31, 2019 Using: | | | |
|-------------------------------|--|-----------|---------|-----------|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Cash equivalents | \$ - | \$ 50,037 | \$ - | \$ 50,037 |
| Available-for-sale securities | - | 7,481 | - | 7,481 |
| | \$ - | \$ 57,518 | \$ - | \$ 57,518 |

As of December 31, 2020 there were no available-for-sale securities. As of December 31, 2019, the fair values of the Company's available-for-sale securities were determined using level two inputs. During the years ended December 31, 2020 and 2019, there were no transfers between Level 1, Level 2 and Level 3.

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

6. Available-for-Sale Securities

As of December 31, 2020 there no available-for-sale securities. As of December 31, 2019, the fair value of available-for-sale debt securities by type of security was as follows:

| | December 31, 2019 | | | Fair Value |
|---------------------------|-------------------|-----------------------|-----------------------|-----------------|
| | Amortized Cost | Gross Unrealized Gain | Gross Unrealized Loss | |
| Investments: | | | | |
| Corporate bond securities | 1,502 | 1 | - | 1,503 |
| Commercial paper | 5,978 | - | - | 5,978 |
| | <u>\$ 7,480</u> | <u>\$ 1</u> | <u>\$ -</u> | <u>\$ 7,481</u> |

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

| | December 31, 2019 | |
|-------------------------------------|-------------------|-----------------|
| | Amortized Cost | Fair Value |
| Due in one year or less | \$ 7,480 | \$ 7,481 |
| Total available-for-sale securities | <u>\$ 7,480</u> | <u>\$ 7,481</u> |

The average maturity of the Company's available-for-sale securities as of December 31, 2019 was approximately 0.2 years.

7. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

| | December 31, | |
|---|-----------------|-----------------|
| | 2020 | 2019 |
| Prepaid research and development expenses | \$ 2,674 | \$ 1,290 |
| Prepaid expenses and other assets | 1,483 | 1,488 |
| | <u>\$ 4,157</u> | <u>\$ 2,778</u> |

8. Property and Equipment

Property and equipment consists of the following:

| | December 31, | |
|-------------------------------|-----------------|------------------|
| | 2020 | 2019 |
| Furniture and fixtures | \$ 203 | \$ 203 |
| Laboratory equipment | 9,631 | 9,425 |
| Leasehold improvements | 4,713 | 4,686 |
| Computer equipment | 436 | 428 |
| Computer software | 553 | 372 |
| Construction in process | 1,330 | 1,322 |
| | <u>16,866</u> | <u>16,436</u> |
| Less accumulated depreciation | 8,713 | 4,791 |
| | <u>\$ 8,153</u> | <u>\$ 11,645</u> |

Depreciation expense was \$3,922, \$2,824 and \$1,566 for the years ended December 31, 2020, 2019 and 2018 respectively.

9. Accrued Expenses

Accrued expenses and other current liabilities consist of the following:

| | December 31, | |
|----------------------------------|-----------------|-----------------|
| | 2020 | 2019 |
| Accrued research and development | \$ 2,091 | \$ 3,742 |
| Accrued compensation | 3,834 | 3,583 |
| Accrued other | 2,715 | 1,853 |
| | <u>\$ 8,640</u> | <u>\$ 9,178</u> |

10. Members' Deficit

Series A, B, C and D Common Units

There were no Series A, B, C and D Common Units authorized, issued or outstanding as of December 31, 2020, 2019 or 2018.

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.

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 continued to vest pursuant to the original vesting terms under the restricted Series A Common Units agreements and the Company continued 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 has recorded the additional compensation expense in the amount of \$904 over the remaining vesting period of the Series B Common Units during the year ended December 31, 2017.

Non-Founder Series A Common Units

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 continued 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 vested units, which represents the incremental fair value of the units before and after the Merger and Recapitalization. The Company recorded additional compensation expense in the amount of \$115 over the remaining vesting period of the Series D Common units of which \$0, \$13 and \$54 were recognized during the years ended December 31, 2020, 2019 and 2018, respectively.

The holders of the Series A, B, C and D Common Units were entitled to the following rights and priorities:

Voting Rights

Holders of Series A and B Common Units had the right to one vote per unit held by such member. The Series A Common unitholders as a class were entitled to elect two of the eight board members while such units are outstanding. The Series B Common unitholders as a class were entitled to elect three of the eight board members while such units are outstanding.

Holders of Series C and D Common Units did not have the right to vote for the election of board members.

Redemption

The Series A, B, C and D Common Units were not redeemable.

Distributions and Liquidation Preference

The holders of the Series A, B, C and D Common Units were entitled to participate in distributions after preferential distributions were made to the Series 1 and Series 2 Senior Preferred and Junior Preferred unitholders as follows:

- The Series A, B, C and D Common unitholders were 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 equaled the cumulative amount distributed to one Junior Preferred Unit.
- The Junior Preferred unitholders and the Series A, B, C and D Common unitholders were 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 equaled the cumulative amount distributed to one Series 1 Senior Preferred Unit.
- The Series 1 Senior Preferred, Junior Preferred unitholders and the Series A, B, C and D Common unitholders were entitled to participate in distributions on a pro rata basis, subject to certain limitations, until the cumulative amount distributed with respect to one Series 1 Senior Preferred Unit, Junior Preferred Unit, Series A Common Unit, Series B Common Unit, Series C Common Unit and vested Series D Common Unit equaled the cumulative amount distributed to one Series 2 Senior Preferred Unit.
- All unitholders were 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, 2020, 2019 and 2018.

Corporate Conversion

Immediately prior to the effectiveness of the Company's registration statement on Form S-1, which occurred on January 25, 2018, the Company completed the Corporate Conversion whereby all the Series A, B, C, and D Common Units converted into shares of common stock.

11. Equity-Based Compensation

2018 Omnibus Incentive Plan

In connection with the closing of the Company's initial public offering, the board of directors and stockholders approved the 2018 Plan, which provides for the reservation of 5,001,000 shares of common stock for equity awards.

2020 Omnibus Incentive Plan

On June 16, 2020, the Company's stockholders approved the 2020 Plan which consists of (i) 3,000,000 shares of common stock and (ii) additional shares of common stock (up to 4,879,025) as is equal to (i) the number of shares reserved under the 2018 Plan that remained available for grant under the 2018 Plan as of immediately prior to the date the 2020 Plan was approved by the Company's stockholders and (ii) the number of shares subject to awards granted under the 2018 Plan which awards expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right.

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

Stock Options

The following table summarizes the Company's stock option activity for the year ended December 31, 2020:

| | Number of Options | Weighted Average Exercise Price |
|---|------------------------------|--|
| Outstanding at December 31, 2019 | 2,610,975 | \$ 22.18 |
| Granted | 1,513,556 | 3.27 |
| Forfeitures | (1,006,756) | 17.27 |
| Outstanding at December 31, 2020 | <u>3,117,775</u> | <u>14.58</u> |
| Vested and expected to vest as of December 31, 2020 | <u>3,117,775</u> | <u>\$ 14.58</u> |
| Exercisable at December 31, 2020 | <u>1,089,779</u> | <u>\$ 19.46</u> |

At December 31, 2020, the Company had an aggregate of \$12,291 of unrecognized equity-based compensation cost related to stock options outstanding which is expected to be recognized over a weighted average period of 2.0 years. The intrinsic value of stock options was \$910, \$0 and \$2,260 as of December 31, 2020, 2019, and 2018, respectively.

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

| | 2020 | 2019 |
|--------------------------|-------------------|-------------------|
| Expected volatility | 21.3% - 119.5% | 85.1% - 104.7% |
| Expected dividends | 0.0% | 0.0% |
| Expected term (in years) | 4.50 - 10.00 | 5.10 - 6.25 |
| Risk-free rate | 0.6% - 3.4% | 1.5% - 2.6% |

The weighted average fair value of options to purchase shares of common stock granted during the year ended December 31, 2020 and 2019 was \$2.69 and \$13.13, respectively.

Restricted Stock Units

In 2020 and 2019, the board of directors issued restricted stock units to employees. Restricted stock unit grants typically vest over one or two years.

The following table summarizes the Company's restricted stock unit activity for the year ended December 31, 2020:

| | Units | Weighted-Average Grant Date Fair Value |
|----------------------------------|----------------|--|
| Unvested at December 31, 2019 | 245,100 | \$ 5.81 |
| Granted | 1,393,720 | 3.16 |
| Vested | (407,700) | 4.42 |
| Forfeitures | (312,725) | 3.56 |
| Outstanding at December 31, 2020 | <u>918,395</u> | <u>\$ 3.17</u> |
| Unvested as of December 31, 2020 | <u>918,395</u> | <u>\$ 3.17</u> |

At December 31, 2020, the Company had an aggregate of \$1,600 of unrecognized equity-based compensation cost related to restricted stock units outstanding. The unrecognized expense for the restricted stock units is expected to be recognized over a weighted average period of 1.1 years.

Restricted Common Stock

In connection with the Company's Corporate Conversion on January 25, 2018, all restricted Series B and D common units were converted to restricted shares of common stock. The following table summarizes the Company's unvested restricted shares of common stock activity for the year ended December 31, 2020:

| | Units | Weighted-Average Grant Date Fair Value |
|--|---------------|--|
| Unvested restricted Series D Common Units at December 31, 2019 | 352,952 | \$ 8.07 |
| Releases | (184,504) | 6.90 |
| Forfeitures | (98,396) | 9.02 |
| Unvested restricted Series D Common Units at December 31, 2020 | <u>70,052</u> | <u>\$ 9.83</u> |

The aggregate intrinsic value of restricted common units that vested during the years ended December 31, 2020, 2019, and 2018 were \$522, \$135, and \$8,721 respectively.

At December 31, 2020, the Company had an aggregate of \$506 of unrecognized equity-based compensation related restricted shares of common stock, which is expected to be recognized over a weighted average period of 0.7 years.

The Company recorded equity-based compensation expense in the following captions within its consolidated statements of operations for the years ended December 31, 2020, 2019, and 2018:

| | For the Year Ended December 31, | | |
|-------------------------------------|---------------------------------|------------------|-----------------|
| | 2020 | 2019 | 2018 |
| Research and development expenses | \$ 5,822 | \$ 8,006 | \$ 4,180 |
| General and administrative expenses | 4,956 | 6,201 | 1,775 |
| Restructuring expenses | 851 | - | - |
| | <u>\$ 11,629</u> | <u>\$ 14,207</u> | <u>\$ 5,955</u> |

12. Leases

The Company leases real estate, including laboratories and office space, and certain equipment related to its research and development. The Company's leases have remaining lease terms ranging from one to six years. Certain leases include an option to renew, exercisable at the Company's sole discretion, with renewal terms that can extend the lease for two years. The Company evaluated the renewal options in its leases to determine if it was reasonably certain that the renewal option would be exercised, and therefore should be included in the calculation of the operating lease assets and operating lease liabilities. Given the Company's current business structure, uncertainty of future growth, and the associated impact to real estate, the Company concluded that it is not reasonably certain that any renewal options would be exercised. Therefore, the operating lease assets and lease liabilities only contemplate the initial lease terms.

In January 2018, the Company executed a lease agreement for lab space in Cambridge, Massachusetts. The lease consists of approximately 9,500 square feet with an initial term of five years with the option to extend the term for one additional two year term. The future minimum rent commitment for the initial five-year term is approximately \$1,900. In addition to rent, the lease requires the Company to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

In January 2018, the Company executed a lease agreement for office space in Cambridge, Massachusetts. The space serves as the Company's corporate headquarters and consists of approximately 16,000 square feet. The term of the lease runs through February 2022. The future minimum rent commitment for the lease term is approximately \$1,500. In addition to rent, the lease requires the Company to pay additional amounts for taxes, insurance, maintenance and other operating expenses.

In November 2018, the Company entered into a 48-month capital lease for certain lab equipment to be used at its facility in Cambridge, Massachusetts. The future minimum lease commitment for the lease term is approximately \$900.

In January 2019, the Company executed a lease agreement for additional office space in Cambridge, Massachusetts. The space serves as office space supporting the Company's lab operations and consists of approximately 5,000 square feet. The term of the lease runs through October 2025. The future minimum rent commitment for the lease term is approximately \$2,000.

As of December 31, 2020, minimum future lease payments for these operating and finance leases were as follows:

| | Finance Leases | Operating Leases |
|-------------------------|-----------------------|-------------------------|
| 2021 | \$ 276 | \$ 2,444 |
| 2022 | 276 | 1,424 |
| 2023 | 300 | 686 |
| 2024 | — | 419 |
| Thereafter | — | 358 |
| Total | 852 | 5,331 |
| Less: Imputed Interest | 119 | 913 |
| Total Lease Liabilities | <u>\$ 733</u> | <u>\$ 4,418</u> |

As of December 31, 2019, minimum future lease payments for these operating and finance leases were as follows:

| | Finance Leases | Operating Leases |
|------------|-----------------------|-------------------------|
| 2020 | \$ 276 | \$ 2,408 |
| 2021 | 276 | 2,444 |
| 2022 | 276 | 1,424 |
| 2023 | 300 | 686 |
| 2024 | — | 778 |
| Thereafter | — | — |
| Total | <u>\$ 1,128</u> | <u>\$ 7,740</u> |

The Company recorded rent expense of \$2,562, \$2,500 and \$2,524 for the years ended December 31, 2020, 2019, and 2018, respectively.

Short-term lease and variable lease costs were not material for the year ended December 31, 2020.

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

| | For the Year Ended December 31, 2020 |
|--|---|
| Other information | |
| Cash paid for amounts included in the measurement of lease liabilities | \$ 2,408 |
| Operating lease liabilities arising from obtaining right-of-use-assets | \$ 1,629 |
| Finance lease liabilities arising from obtaining right-of-use assets | \$ — |
| Weighted-average remaining lease term (in years) | |
| Operating lease | 2.8 |
| Finance lease | 2.3 |
| Weighted-average discount rate | |
| Operating lease | 12.6% |
| Finance lease | 10.7% |

13. Commitments and Contingencies

Letter of Credit

The Company had outstanding letters of credit in the amounts of \$327 and \$327 at December 31, 2020 and 2019, respectively, which were required as a condition of the Company's office and laboratory leases.

Indemnification Agreements

In the ordinary course of business, the Company may provide indemnification of varying scope and terms to vendors, lessors, business partners and other parties with respect to certain matters, including, but not limited to, losses arising out of breach of such agreements or from intellectual property infringement claims made by third parties. In addition, the Company has entered into indemnification agreements with its executive officers and members of its board of directors that require the Company, among other things, to indemnify them against certain liabilities that may arise by reason of their status or service as executive officers or directors. The maximum potential amount of future payments the Company could be required to make under these indemnification agreements is, in many cases, unlimited. To date, the Company has not incurred any material costs as a result of such indemnification arrangements.

The Company does not believe that the outcome of any claims under indemnification arrangements will have a material effect on its financial position, results of operations or cash flows, and it has not accrued any liabilities related to such obligations in its consolidated financial statements as of December 31, 2020 and 2019.

Legal Proceedings

On March 28, 2018, a purported stockholder of the Company, filed a putative class action complaint alleging violations of the federal securities laws, in the Business Litigation Section of the Superior Court of the Commonwealth of Massachusetts (Civil Action No. 1884-00984), against the Company, Ilan Ganot, Jennifer Ziolkowski, the Company's directors and certain of the underwriters in the Company's initial public offering. The plaintiff in this suit claims to represent purchasers of the Company's common stock in or traceable to the Company's January 25, 2018 initial public offering and seeks unspecified damages arising out of the alleged failure to disclose risks associated with toxicity and potential for adverse events related to the Company's lead product candidate. On April 30, 2018, all defendants including the Company moved to stay the proceedings in favor of the prior-filed federal court securities class action. The plaintiff filed his opposition to this motion on May 14, 2018, and defendants filed a reply in support of their motion on May 24, 2018. After oral argument on June 13, 2018, the court issued an order on June 22, 2018 allowing the motion to stay and directing the parties to advise the court of the status of the federal court action every six months. On December 21, 2018, the parties filed a joint status report informing the court of the voluntary dismissal of the federal actions and of plaintiff's intent to move to vacate the stay. On June 28, 2019, the plaintiff voluntarily dismissed his claims without prejudice.

The Company may periodically become subject to other legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which the Company is focused. Other than the above action, the Company is not aware of any other material claims as of December 31, 2020.

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 Duchenne and related disease indications caused by a lack of functional dystrophin. The Company has the right to grant sublicenses to third parties contingent upon written approval by the University of Washington prior to executing such sublicense, which approval may not be unreasonably withheld.

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Washington for costs incurred in applying for, prosecuting and maintaining patents and pay up to an aggregate of approximately \$1,000 upon the achievement of certain milestones. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017. In October 2020, the license agreement was amended such that the Company was required to pay the University of Washington \$375 in connection with the execution of the Collaboration Agreement. This payment was recorded as a research and development expense in the fourth quarter of 2020. The license agreement was also amended such that the Company is required to pay an aggregate of approximately \$3,400 upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2020, 2019, and 2018. The Company must also pay royalties of a low single digit percentage of future sales by the Company and its sublicensees of products developed under the licensed patent rights. In addition, the Company must pay an annual maintenance fee until certain milestones are achieved, at which time a minimum annual royalty requirement will replace such maintenance fee and will apply to the Company and its sublicensees.

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

The Company recorded research and development expense in the amount of \$446, \$38, and \$47 for the years ended December 31, 2020, 2019, and 2018, respectively, under the agreement.

The University of Missouri License Agreement

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

In consideration for the rights granted by the agreement, the Company paid a one-time, non-refundable license fee, which was recorded as a research and development expense in 2015. The Company is required to reimburse the University of Missouri for costs incurred in applying for, prosecuting and maintaining the licensed patents and pay up to an aggregate of approximately \$1,000 upon the achievement of certain milestones for each product developed based on the licensed patents. In October 2017, the first milestone was achieved under this agreement. The milestone payment was recorded as a research and development expense in the fourth quarter of 2017.

Under the agreement, in the event the Company grants a sublicense to another party, the Company is required to pay the University of Missouri a percentage of the consideration received. The license agreement was amended such that the Company is required to pay the University of Missouri \$750 in 2021 and \$1,300 in 2022 as a result of the execution of the Collaboration Agreement with Ultragenyx in October 2020. These amounts were recorded as a research and development expense in the fourth quarter of 2020. The Company paid \$750 in February 2021. The license agreement was also amended such that the Company is required to pay an aggregate of approximately \$1,900 upon the achievement of certain milestones.

There were no milestones achieved during the years ended December 31, 2020, 2019, and 2018. 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 \$2,111, \$23, and \$10 for the years ended December 31, 2020, 2019, and 2018, respectively, under the agreement.

The University of Michigan License Agreement

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

In consideration for the rights granted by the agreement, the Company paid a one-time license fee and a separate fee to cover past patent prosecution costs, which the Company recorded as a research and development expense in 2016. The Company is required to reimburse the University of Michigan for costs incurred in applying for, prosecuting and maintaining patents, and pay up to an aggregate of approximately \$1,000 upon the achievement of certain milestones. There were no milestones achieved during the years ended December 31, 2020, 2019, and 2018. The Company must also pay a royalty of a low single digit percentage on future sales by the Company or its sublicensees of products developed using the licensed rights, with a minimum annual royalty after certain milestones are achieved. In addition, the Company 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 \$35, \$39 and \$35 for the years ended December 31, 2020, 2019, and 2018, 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, ("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 Duchenne.

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 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 during the years ended December 31, 2020, 2019, and 2018. The Company must pay a royalty of a low single digit percentage on future sales by the Company or its 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 \$0, \$10 and \$20 for the years ended December 31, 2020, 2019, and 2018, respectively, under the agreement.

In August 2017, the Company entered into another license agreement with 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 Duchenne.

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 during the years ended December 31, 2020, 2019, and 2018. The Company must pay a royalty of a low single digit percentage on future sales by the Company or its 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 \$0, \$5 and \$5 for the years ended December 31, 2020, 2019, and 2018, respectively, under the agreement.

Other License Agreements

In 2016, the Company entered into a license agreement with Life Technologies Corporation ("Life Technologies"). In consideration for obtaining a non-exclusive, royalty-free, worldwide license to use certain 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 the Company's 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 Share

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

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

| | For the Year Ended December 31, | | |
|----------|---------------------------------|------------------|-----------------|
| | 2020 | 2019 | 2018 |
| Net loss | <u>(88,290)</u> | <u>(117,223)</u> | <u>(74,798)</u> |

The denominator is as follows:

| | For the Year Ended December 31, | | |
|---|---------------------------------|-------------------|-------------------|
| | 2020 | 2019 | 2018 |
| Weighted average common stock outstanding, basic and diluted | 49,675,603 | 39,326,983 | 33,262,597 |
| Weighted average pre-funded warrants to purchase common stock | 2,261,544 | 962,307 | - |
| Total | <u>51,937,147</u> | <u>40,289,290</u> | <u>33,262,597</u> |

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

| | For the Year Ended December 31, | | |
|---------------------------------------|---------------------------------|------------------|------------------|
| | 2020 | 2019 | 2018 |
| Net loss per share, basic and diluted | <u>\$ (1.70)</u> | <u>\$ (2.91)</u> | <u>\$ (2.25)</u> |

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

| | For the Year Ended December 31, | | |
|--|---------------------------------|------------------|------------------|
| | 2020 | 2019 | 2018 |
| Options to purchase shares of common stock | 3,117,775 | 2,610,975 | 1,153,198 |
| Unvested restricted stock units | 918,395 | 245,100 | - |
| Unvested shares of common stock | 70,052 | 352,952 | 743,564 |
| | <u>4,106,222</u> | <u>3,209,027</u> | <u>1,896,762</u> |

16. Income Taxes

The Company recorded no tax benefit for the years ended December 31, 2020 and 2019 for the net operating losses incurred due to its uncertainty of realizing a benefit from those items.

A reconciliation of income taxes computed using the U.S. federal statutory rate to that reflected in operations as of December 31, 2020 and 2019 is as follows:

| | December 31, 2020 | December 31, 2019 |
|---|----------------------|----------------------|
| Income tax computed at federal statutory tax rate | 21.0% | 21.0% |
| State taxes, net of federal benefit | 6.0% | 6.1% |
| Permanent differences | (0.4)% | (0.4)% |
| Tax credits | 9.6% | 10.9% |
| Other | (0.4)% | (0.1)% |
| Valuation allowance | (35.8)% | (37.5)% |
| | <u>0.0%</u> | <u>0.0%</u> |

The Company established deferred tax assets and liabilities on identified book to tax temporary differences as of the date of conversion to a C-corporation. Deferred income taxes reflect the net tax effects of these temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. Significant components of the Company's net deferred tax assets as of December 31, 2020 and 2019 are as follows:

| | December 31, 2020 | December 31, 2019 |
|--------------------------------|-------------------|-------------------|
| Deferred tax assets: | | |
| Tax loss carryforwards | \$ 53,321 | \$ 43,885 |
| Tax credit carryforwards | 28,154 | 19,717 |
| Lease liability | 1,200 | 1,920 |
| Accrued expenses | 994 | 921 |
| Stock compensation | 5,909 | 4,159 |
| Capitalized R&D expense | 12,034 | - |
| Other | 144 | 257 |
| Total deferred tax assets | <u>101,756</u> | <u>70,859</u> |
| Valuation allowance | <u>(100,740)</u> | <u>(69,171)</u> |
| Deferred tax liabilities: | | |
| Right of use asset | (972) | (1,355) |
| Depreciation | (44) | (333) |
| Total deferred tax liabilities | <u>(1,016)</u> | <u>(1,688)</u> |
| Net deferred taxes | <u>\$ -</u> | <u>\$ -</u> |

As of December 31, 2020, the Company has federal net operating loss carryforwards of \$194,529 which may be available to offset future taxable income and do not expire, but are limited in their usage to an annual deduction equal to 80% of annual taxable income. In addition, as of December 31, 2020, the Company has state net operating loss carryforwards of approximately \$192,558 which may be available to offset future taxable income and begin to expire in 2038. The Company also had federal and state tax credits of \$26,643 and \$1,912, respectively, which may be used to offset future tax liability and each of which begin to expire in 2033. The Company's ability to utilize these federal and state carryforwards may be limited in the future if the Company experiences an ownership change pursuant to Internal Revenue Code Section 382. Ownership changes, as defined in the Internal Revenue Code, including those resulting from the issuance of common stock in connection with the Company's public offerings, may limit the amount of net operating loss and tax credit carryforwards that can be utilized to offset future taxable income or tax liability. The Company has not conducted a study to assess whether a change of control has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined by Section 382, at any time since inception, utilization of the net operating loss carryforwards tax credit carryforwards would be subject to an annual limitation under Section 382. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization. Further, until a study is completed and any limitation is known, no amounts are being presented as an uncertain tax position.

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

The Company files tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's C-Corporation tax years beginning with the year ended December 31, 2018 are open under statute. Any tax credit or net operating loss carryforward can be adjusted in future periods after the respective year of generation's statute of limitation has closed.

As of December 31, 2020 and 2019, the Company did not have unrecognized tax benefits. The Company recognizes interest and penalties related to income taxes as a component of income tax expense. As of December 31, 2020 and 2019, no interest and penalties have been recorded.

17. 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 directors. The Company made no contributions to the plan during the years ended December 31, 2020, 2019 and 2018.

18. Restructuring

In January 2020, the Company's board of directors approved a restructuring plan to reduce operating costs and better align the Company's workforce with the needs of its business following the Company's November 2019 announcement that the SGT-001 IGNITE DMD trial was placed on clinical hold by the U.S. Food and Drug Administration.

Under the restructuring plan, the Company made changes to its management team and reduced headcount by approximately 30 percent. Affected employees were eligible to receive severance payments and outplacement services in connection with the restructuring plan. During year ended December 31, 2020, the Company recorded aggregate restructuring charges of \$1,944 related to severance payments and other employee-related costs. The Company does not expect to incur any additional significant costs associated with this restructuring. During the year ended December 31, 2020, \$1,882 of the estimated restructuring charges were paid. The Company expects the remaining accrued restructuring costs of \$62 will be paid during the first quarter of 2021.

The following table shows the total amount expected to be incurred and the liability related to the 2020 restructuring for the year ended December 31, 2020:

| | One-Time Employee Termination Benefits |
|---|---|
| Accrued restructuring costs as of December 31, 2019 | \$ - |
| Restructuring charges incurred during the period | 1,944 |
| Amounts paid during the period | (1,882) |
| Accrued restructuring costs as of December 31, 2020 | <u>\$ 62</u> |

19. Subsequent Events

Issuance of new option awards

On January 4, 2021, the Company granted options to purchase 389,000 shares of common stock, at an exercise price of \$7.84 per share, to a director as compensation for future services to the Company. The options vest in equal quarterly installments beginning three months from the date of the grant with the final installment vesting date being the later of the completion of a specified service period or 12 months from the date of the grant.

On January 25, 2021, the Company granted options to purchase an aggregate of 1,439,895 shares of common stock, at an exercise price of \$6.80, to employees as compensation for future services. The options vest over four years. Also on January 25, 2021, the Company granted options to purchase 96,000 shares of common stock to non-employees, at an exercise price of \$6.80, as compensation for future services to the Company. The options vest between one and two years.

On March 1, 2021, the Company granted options to purchase 325,000 shares of common stock, at an exercise price of \$8.52 per share, to its Chief Legal Officer as compensation for future services to the Company. The options vest over four years.

On March 2, 2021, the Company granted options to purchase 80,000 shares of common stock, at an exercise price of \$10.72 per share, to newly appointed directors as compensation for future services to the Company. The options vest over three years.

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