UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 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	**90-0943402**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
141 Portland Street, Fifth Floor Cambridge, MA	**02139**
(Address of principal executive offices)	**(Zip Code)**

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

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

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

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

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

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

Large accelerated filer	☐	Accelerated filer	☒
Non-accelerated filer	☐	Smaller reporting company	☒
		Emerging growth company	☒

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of November 1, 2020, the registrant had 60,449,341 shares of common stock, $0.001 par value per share, outstanding.

Table of Contents

Item 1. Financial Statements (unaudited)

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

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 24,797	$ 76,043
Available-for-sale-securities	—	7,481
Prepaid expenses and other current assets	2,365	2,778
Total current assets	27,162	86,302
Property and equipment, net	8,869	11,645
Operating lease, right-of-use assets	3,951	4,988
Other non-current assets	209	209
Restricted cash	327	327
Total assets	$ 40,518	$ 103,471
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,083	$ 7,124
Accrued expenses	8,550	9,178
Operating lease liabilities	1,930	1,736
Finance lease liabilities	202	186
Other current liabilities	—	52
Total current liabilities	14,765	18,276
Operating lease liabilities, excluding current portion	2,943	4,414
Finance lease liabilities, excluding current portion	579	733
Total liabilities	18,287	23,423
Commitments and contingencies (Note 11)		
Preferred stock, $0.001 par value; 10,000,000 shares authorized at September 30, 2020 and December 31, 2019; no shares issued and outstanding at September 30, 2020 and December 31, 2019	—	—
Common stock, $0.001 par value; 300,000,000 shares authorized at September 30, 2020 and December 31, 2019; 46,178,519 shares issued and outstanding at September 30, 2020 and 45,987,571 shares issued and outstanding at December 31, 2019; 2,295,699 pre-funded warrants outstanding at September 30, 2020 and December 31, 2019	48	48
Additional paid-in capital	405,389	396,278
Accumulated other comprehensive income	—	1
Accumulated deficit	(383,206)	(316,279)
Total stockholders' equity	22,231	80,048
Total liabilities and stockholders' equity	$ 40,518	$ 103,471

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	16,045	22,792	49,158	67,671
General and administrative	5,181	6,925	15,957	19,317
Restructuring charges	—	—	1,944	—
Total operating expenses	21,226	29,717	67,059	86,988
Loss from operations	(21,226)	(29,717)	(67,059)	(86,988)
Other income (expense):				
Interest (expense) income	(20)	406	131	1,281
Other income	_	56	1	345
Total other income (expense), net	(20)	462	132	1,626
Net loss	$ (21,246)	$ (29,255)	$ (66,927)	$ (85,362)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.44)	$ (0.67)	$ (1.39)	$ (2.26)
Weighted average shares of common stock outstanding, basic and diluted	48,295,468	43,467,618	48,172,686	37,727,640

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Net loss	$ (21,246)	$ (29,255)	$ (66,927)	$ (85,362)
Other comprehensive loss:				
Unrealized (loss) gain on available-for-sale securities	—	(5)	(1)	3
Comprehensive loss	$ (21,246)	$ (29,260)	$ (66,928)	$ (85,359)

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

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

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

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

	For the Three Months Ended September 30, 2019					
	Common Stock	Amount	Additional paid in capital	Accumulated other comprehensive income (loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at June 30, 2019	35,398,894	$ 35	$ 331,620	$ 3	$ (255,163)	$ 76,495
Equity-based compensation	—	—	3,392	—	—	3,392
Sale of common stock, net of issuance costs of $2,079	10,607,525	11	47,235	—	—	47,246
Sale of pre-funded warrants	2,295,699	2	10,650	—	—	10,652
Net loss	—	—	—	—	(29,255)	(29,255)
Repurchase of shares of common stock	(7,475)	—	—	—	—	—
Unrealized loss on available for sale securities	—	—	—	(5)	—	(5)
Balance at September 30, 2019	48,294,643	$ 48	$ 392,897	$ (2)	$ (284,418)	$ 108,525

	For the Nine Months Ended September 30, 2019					
	Common Stock	Amount	Additional paid in capital	Accumulated other comprehensive income (loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2018	35,432,460	$ 35	$ 324,209	$ (5)	$ (199,056)	$ 125,183
Equity-based compensation	—	—	10,803	—	—	10,803
Sale of common stock, net of issuance costs of $2,079	10,607,525	11	47,235	—	—	47,246
Sale of pre-funded warrants	2,295,699	2	10,650	—	—	10,652
Net loss	—	—	—	—	(85,362)	(85,362)
Repurchase of shares of common stock	(41,041)	—	—	—	—	—
Unrealized gain on available for sale securities	—	—	—	3	—	3
Balance at September 30, 2019	48,294,643	$ 48	$ 392,897	$ (2)	$ (284,418)	$ 108,525

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

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

	Nine Months Ended September 30,	
	2020	**2019**
Cash flows from operating activities:		
Net loss	$ (66,927)	$ (85,362)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of (discount)/premium on available-for-sale securities	(20)	(246)
Equity-based compensation expense	9,111	10,803
Depreciation expense	3,126	2,018
Loss on sale of property and equipment	—	2
Changes in operating assets and liabilities:		
Prepaid expenses and other current and non-current assets	1,450	(1,106)
Accounts payable	(2,602)	8
Accrued expenses and other current and non-current liabilities	(2,044)	2,414
Net cash used in operating activities	(57,906)	(71,469)
Cash flows from investing activities:		
Purchases of property and equipment	(839)	(3,742)
Proceeds from sale and maturities of available-for-sale securities	7,900	56,899
Purchases of available-for-sale securities	(401)	(31,496)
Net cash provided by investing activities	6,660	21,661
Cash flows from financing activities:		
Proceeds from issuance of common stock	—	49,325
Proceeds from issuance of warrants	—	10,652
Payment of offering costs	—	(1,815)
Net cash provided by financing activities	—	58,162
Net (decrease)/increase in cash, cash equivalents and restricted cash	(51,246)	8,354
Cash, cash equivalents, and restricted cash at beginning of period	76,370	86,693
Cash, cash equivalents, and restricted cash at end of period	$ 25,124	$ 95,047
Supplemental disclosure of non-cash investing and financing activities:		
Operating lease liabilities arising from obtaining right-of-use asset	$ —	$ 1,629
Property and equipment included in accounts payable and accruals	$ —	$ 51
Offering costs included in accounts payable and accruals	$ —	$ 264

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

1. Nature of the Business and Basis of Presentation

Nature of Business

Solid Biosciences Inc. was organized in March 2013 under the name SOLID Ventures Management, LLC. 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 preclinical studies and clinical trials and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance and reporting capabilities.

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

Liquidity

The accompanying condensed consolidated financial statements have been prepared on a basis that assumes the Company will continue as a going concern and which contemplates the realization of assets and satisfaction of liabilities and commitments in the ordinary course of business. Through September 30, 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.

In accordance with Accounting Standards Codification ("ASC") 205-40, Going Concern, the Company has evaluated whether there are conditions and events, considered in the aggregate, that raise substantial doubt about the Company's ability to continue as a going concern within one year after the date the financial statements are issued. As of September 30, 2020, the Company had an accumulated deficit of $383,206. During the three and nine months ended September 30, 2020, the Company incurred a net loss of $21,246 and $66,927, respectively, and the Company used $57,906 of cash in operations for the nine months ended September 30, 2020. 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 $24,797 as of September 30, 2020, together with the proceeds of $40,000 received in October 2020 from the sale of shares of common stock to Ultragenyx Pharmaceutical, Inc. ("Ultragenyx") as well as the net proceeds of $23,209 received from the sale of shares of common stock in October 2020 pursuant to a sales agreement, dated March 13, 2019, between the Company and Jefferies LLC (the "ATM Sales Agreement") will be sufficient to fund its operating expenses and capital requirements into the second half of 2021.

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, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects.

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

In the opinion of management, the Company's accompanying unaudited condensed consolidated financial statements include all adjustments, consisting of normal recurring accruals, necessary for a fair statement of the Company's financial statements for interim periods in accordance with GAAP. The information included in this quarterly report on Form 10-Q should be read in conjunction with the Company's consolidated financial statements and the accompanying notes included in the Company's Annual Report on Form 10-K for the year ended December 31, 2019. The year-end condensed consolidated balance sheet data presented for comparative purposes was derived from the Company's audited financial statements but does not include all disclosures required by GAAP. The results of operations for the three and nine months ended September 30, 2020 are not necessarily indicative of the operating results for the full year or for any other subsequent interim period.

2. Summary of Significant Accounting Policies

The Company's accounting policies are described in the "Notes to Consolidated Financial Statements" in its Annual Report on Form 10-K for the year ended December 31, 2019 and updated, as necessary, in this report.

Use of Estimates

The preparation of the Company's condensed consolidated financial statements in conformity with GAAP requires management to make estimates, judgments and assumptions that affect the reported amounts of assets and liabilities, the disclosure of contingent assets and liabilities at the date of the condensed consolidated financial statements and the reported amounts of expenses during the reporting periods. Significant estimates and assumptions reflected in these condensed consolidated financial statements include, but are not limited to, 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. 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 September 30, 2020 and December 31, 2019. The Company has included restricted cash of $327 as a non-current asset as of September 30, 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:

	September 30, 2020	December 31, 2019
Cash and cash equivalents as presented on balance sheet	$ 24,797	$ 76,043
Restricted cash, non-current, as presented on balance sheet	327	327
Cash and cash equivalents and restricted cash as presented on cash flow statement	$ 25,124	$ 76,370

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.

Recently Adopted Accounting Pronouncements

In August 2018, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("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 became effective for the Company on January 1, 2020. The adoption of this new standard did not have a material impact on the Company's disclosures.

Recently Issued Accounting Pronouncements

In June 2016, the FASB issued ASU No. 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments* ("ASU 2016-13"), which requires the measurement and recognition of expected credit losses for financial assets held at amortized cost. ASU 2016-13 replaces the existing incurred loss impairment model with an expected loss model. It also eliminates the concept of other-than-temporary impairment and requires credit losses related to available-for-sale debt securities to be recorded through an allowance for credit losses rather than as a reduction in the amortized cost basis of the securities. These changes may result in earlier recognition of credit losses. In November 2018, the FASB issued ASU No. 2018-19, *Codification Improvements to Topic 326, Financial Instruments—Credit Losses*, which narrowed the scope and changed the effective date for non-public entities for ASU 2016-13. The FASB subsequently issued supplemental guidance within ASU No. 2019-05, *Financial Instruments—Credit Losses (Topic 326): Targeted Transition Relief* ("ASU 2019-05"). ASU 2019-05 provides an option to irrevocably elect the fair value option for certain financial assets previously measured at amortized cost basis. For public entities that are Securities and Exchange Commission filers, excluding entities eligible to be smaller reporting companies, ASU 2016-13 is effective for annual periods beginning after December 15, 2019, including interim periods within those fiscal years. For all other entities, ASU 2016-13 is effective for annual periods beginning after December 15, 2022, including interim periods within those fiscal years. Early adoption is permitted. This standard will be effective for the Company on January 1, 2023. The Company is currently evaluating the potential impact that this standard may have on its financial statements and related disclosures.

3. Fair Value of Financial Assets and Liabilities

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

	Fair Value Measurements as of September 30, 2020 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents	$ —	$ 14,462	$ —	$ 14,462
	$ —	$ 14,462	$ —	$ 14,462

	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 September 30, 2020, there were no available-for-sale securities. As of December 31, 2019, the fair values of the Company's available-for-sale debt securities were determined using Level 2 inputs. At December 31, 2019, the Company's portfolio of available-for-sale securities consisted of corporate bond securities and commercial paper. During the nine months ended September 30, 2020 and the year ended December 31, 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.

4. Available-for-Sale Securities

As of September 30, 2020, there were no available-for-sale securities. As of December 31, 2019, the fair value of available-for-sale securities by type of security was as follows:

	December 31, 2019			
	Amortized Cost	Gross Unrealized Gain	Gross Unrealized Loss	Fair Value
Investments:				
Corporate bond securities	$ 1,502	$ 1	$ —	$ 1,503
Commercial paper	5,978	—	—	5,978
	$ 7,480	$ 1	$ —	$ 7,481

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	$ 7,480	$ 7,481

The weighted average maturity of the Company's available-for-sale securities as of December 31, 2019 was approximately 0.2 years.

5. Property and Equipment

Property and equipment consists of the following:

	September 30, 2020	December 31, 2019
Furniture and fixtures	$ 203	$ 203
Laboratory equipment	9,616	9,425
Leasehold improvements	4,713	4,686
Computer equipment	436	428
Computer software	553	372
Construction in process	1,265	1,322
	16,786	16,436
Less accumulated depreciation	7,917	4,791
	$ 8,869	$ 11,645

Depreciation expense was $766 and $3,126 for the three and nine months ended September 30, 2020, respectively, and $764 and $2,018 for the three and nine months ended September 30, 2019, respectively.

6. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following:

	September 30, 2020	December 31, 2019
Prepaid research and development expenses	$ 587	$ 1,290
Prepaid expenses and other assets	1,778	1,488
	$ 2,365	$ 2,778

7. Accrued Expenses and Other Current Liabilities

Accrued expenses and other current liabilities consist of the following:

	September 30, 2020	December 31, 2019
Accrued research and development	$ 3,099	$ 3,742
Accrued compensation	3,961	3,583
Accrued other	1,490	1,853
	$ 8,550	$ 9,178

8. Stockholders' Equity

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 $2,079.

No warrants were exercised during the three and nine months ended September 30, 2020 and 2019.

9. Equity-Based Compensation

In connection with the closing of the Company's initial public offering, the board of directors and stockholders approved the 2018 Omnibus Incentive Plan, which provides for the reservation of 5,001,000 shares of common stock for equity awards. On June 16, 2020, the Company's stockholders approved the 2020 Equity Incentive Plan ("2020 Plan") which consists of (i) 3,000,000 shares of common stock and (ii) additional shares of common stock (up to 4,879,025) as is equal to (i) the number of shares reserved under the Company's 2018 Omnibus Incentive Plan ("2018 Plan") that remain available for grant under the 2018 Plan as of immediately prior to

the date the 2020 Plan was approved by the Company's stockholders and (ii) the number of shares subject to awards granted under the 2018 Plan which awards expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right.

During the three and nine months ended September 30, 2020, the Company granted options for the purchase of 41,440 and 1,439,356 shares of common stock, respectively. During the three and nine months ended September 30, 2020, the Company granted 0 and 1,393,720 restricted stock units, respectively.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Research and development	$ 1,524	$ 2,070	$ 4,379	$ 6,003
General and administrative	1,240	1,322	3,881	4,800
Restructuring	—	—	851	—
Total	$ 2,764	$ 3,392	$ 9,111	$ 10,803

10. Income Taxes

During the three and nine months ended September 30, 2020 and 2019, the Company recorded no income tax benefits for the net operating losses incurred or for the research and development tax credits and orphan drug credits generated in each year due to its uncertainty of realizing a benefit from those items. The Company has provided a valuation allowance for the full amount of its net deferred tax assets because, at September 30, 2020 and December 31, 2019, it was more likely than not that any future benefit from deductible temporary differences and net operating loss and tax credit carryforwards would not be realized.

As of September 30, 2020, and December 31, 2019, the Company had not recorded any amounts for unrecognized tax benefits. The Company files income tax returns as prescribed by the tax laws of the jurisdictions in which it operates. In the normal course of business, the Company is subject to examination by federal and state jurisdictions, where applicable. There are currently no pending income tax examinations. The Company's C-Corporation tax years beginning with the year ended December 31, 2018 are open under statute. Any tax credit or net operating loss carryforward can be adjusted in future periods after the respective year of generation's statute of limitation has closed.

11. Commitments and Contingencies

Legal Proceedings

The Company may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which the Company is focused. The Company is not aware of any material claims as of September 30, 2020.

12. Net Loss per Share

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Net loss attributable to common stockholders	$ (21,246)	$ (29,255)	$ (66,927)	$ (85,362)

The denominator is as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Weighted average shares of common stock outstanding, basic and diluted	45,999,769	41,945,470	45,876,987	37,214,682
Weighted average shares of pre-funded warrants to purchase common stock	2,295,699	1,522,148	2,295,699	512,958
Total	48,295,468	43,467,618	48,172,686	37,727,640

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Net loss per share attributable to common stockholders	$ (0.44)	$ (0.67)	$ (1.39)	$ (2.26)

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

	2020	2019
Options to purchase shares of common stock	3,324,885	2,662,162
Unvested shares of common stock	102,753	430,683
Unvested restricted stock units	1,254,545	264,650
	4,682,183	3,357,495

13. 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 the three and nine months ended September 30, 2020, the Company recorded aggregate restructuring charges of $0 and $1,944, respectively, 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 three and nine months ended September 30, 2020, $246 and $1,671, respectively, of the estimated restructuring charges were paid. The Company expects the remaining accrued restructuring costs of $273 will be paid in the next three months.

The following table shows the total amount expected to be incurred and the liability related to the 2020 restructuring for the three and nine months ended September 30, 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	(963)
Accrued restructuring costs as of March 31, 2020	$ 981
Amounts paid during the period	(462)
Accrued restructuring costs as of June 30, 2020	$ 519
Amounts paid during the period	(246)
Accrued restructuring costs as of September 30, 2020	$ 273

14. Subsequent Events

Ultragenyx Collaboration Agreement

On October 22, 2020 the Company entered into a collaboration and license agreement (the "Collaboration Agreement") with Ultragenyx, to focus on the development and commercialization of new gene therapies for Duchenne. 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 and other diseases resulting from the lack of functional dystrophin. The Company will conduct certain activities agreed to by the parties with respect to the development of licensed products. Ultragenyx will reimburse the Company for personnel and out-of-pocket costs that the Company incurs in conducting such development activities. Otherwise, Ultragenyx has decision-making authority with respect to the development, manufacturing and commercialization of licensed products. The Company retains exclusive rights to all other uses of its microdystrophin proteins, including under its existing SGT-001 program.

In connection with the execution of the Collaboration Agreement, Ultragenyx and the Company also entered into a stock purchase agreement , pursuant to which the Company issued and sold 7,825,797 shares of its common stock to Ultragenyx at a price of $5.1113 per share, which represented a 33% premium to the volume weighted average price of the Company's common stock for the 10 trading days prior to the closing date, for an aggregate purchase price of approximately $40,000.

Ultragenyx also agreed to pay up to $255,000 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, the Company has the right to opt-in to co-fund collaboration programs in return for participation in a profit share or increased royalty payments.

At-the-Market Offering

In October 2020, the Company sold 6,302,632 shares of common stock pursuant to the ATM Sales Agreement resulting in net proceeds to the Company of $23,209.

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed consolidated financial statements and related notes appearing elsewhere in this quarterly report on Form 10-Q and our audited financial statements and related notes for the year ended December 31, 2019 included in our annual report filed on Form 10-K on March 12, 2020.

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

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

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

Overview

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

In the fourth quarter of 2017, we initiated IGNITE DMD, a randomized, controlled, open-label, single-ascending dose Phase I/II clinical trial, 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., neuronal nitric oxide synthase, or 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 IGNITE DMD protocol and our intention to dose escalate.

In May 2019, we announced that two patients were randomized in the second cohort of the IGNITE DMD study, including one patient dosed with 2E14 vg/kg of SGT-001 and another added to the control group.

In August 2019, we announced that we amended the IGNITE DMD clinical trial protocol. The changes to the protocol included adding an upper weight limit of 25 kg for at least the next patient dosed in the second cohort and removing the matched patient control arm for the rest of the second cohort. We also announced that a patient was dosed under this amended protocol.

In November 2019, we announced that the third patient in the 2E14 vg/kg cohort of IGNITE DMD, dosed in late October 2019, experienced a serious adverse event, or SAE, deemed related to the study drug and that IGNITE DMD was placed on clinical hold by the U.S. Food and Drug Administration, or 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 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 are amending 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. We are currently working to complete all activities necessary to resume dosing, which we expect to occur in the first quarter of 2021.

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% to70% 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 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.

Since our inception, we have devoted substantial resources to identifying and developing SGT-001 and other product candidates, developing our manufacturing processes, organizing and staffing our company and providing general and administrative support for these operations. We do not have any products approved for sale. To date, we have not generated any revenue. Our ability to eventually generate any product revenue sufficient to achieve profitability will depend on the successful development, approval and eventual commercialization of SGT-001 and other product candidates. If successfully developed and approved, we intend to commercialize SGT-001 and we may enter into licensing agreements or strategic collaborations in select 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 other product candidates in a timely manner or obtain regulatory approval of them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

We have never been profitable, and since our inception, we have incurred significant operating losses. Our net losses were $66.9 million and $85.4 million for the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020, we had an accumulated deficit of $383.2 million. We expect to incur significant expenses and operating losses for the foreseeable future.

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

Since our initial public offering, which we completed on January 30, 2018, we have primarily financed our operations through the sale of common stock. In our initial public offering we sold 8,984,375 shares of our common stock, including shares of common stock issued upon the exercise in full of the underwriters' over-allotment option, at a public offering price of $16.00 per share, resulting in net proceeds of $129.1 million, after deducting underwriting discounts and commissions and offering expenses. 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 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 Muscular Dystrophy and other disease indications resulting from a lack of functional dystrophin, including Becker muscular dystrophy. 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.

As of September 30, 2020, we had cash and cash equivalents of $24.8 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 existing cash and cash equivalents as of September 30, 2020, together with the proceeds of $40.0 million received in October 2020 from the sale of shares of our common stock to Ultragenyx as well as the net proceeds of $23.2 million received from the sale of shares of our common stock in October 2020 pursuant to a sales agreement, dated March 13, 2019, or the ATM Sales Agreement, by and between us and Jefferies LLC, or Jefferies, will enable us to fund our operating expenses into the second half of 2021. The financial statements for the quarter ended September 30, 2020 do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classification of liabilities that may result from uncertainty related to our ability to continue as a going concern.

The ongoing novel coronavirus pandemic, commonly referred to as COVID-19, which began in December 2019, has spread worldwide, causing 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.

Financial operations overview

Revenue

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

Operating expenses

Our operating expenses since inception have consisted primarily of research and development activities and general and administrative costs. 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 other product candidates and include:

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

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

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
(In thousands)	2020	2019	2020	2019
SGT-001	$ 8,628	$ 9,323	$ 24,408	$ 29,390
Other product candidates	83	544	465	3,726
Unallocated research and development expenses				
Personnel related expenses	5,179	9,357	16,512	23,335
External expenses	2,155	3,568	7,773	11,220
Total unallocated research and development expenses	7,334	12,925	24,285	34,555
Total research and development expenses	$ 16,045	$ 22,792	$ 49,158	$ 67,671

We cannot determine with certainty the duration, costs and timing of clinical trials of SGT-001 and 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;
- the impact of the COVID-19 outbreak on our ability to conduct clinical trials of SGT-001 and other product candidates;
- uncertainties in clinical trial design and patient enrollment or drop out or discontinuation rates;
- significant and changing government regulation and regulatory guidance;
- potential additional studies requested by regulatory agencies; and
- the timing and receipt of any marketing approvals.

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 decrease in calendar year 2020 as a result of the organizational changes we announced in January 2020, to create a leaner organization focused on advancing SGT-001.

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 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 decrease in calendar year 2020 as a result of the organizational changes we announced in January 2020, to create a leaner organization focused on advancing SGT-001.

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

Income taxes

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

Critical Accounting Policies and Use of Estimates

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

During the three and nine months ended September 30, 2020, there were no material changes to our critical accounting policies. Our critical accounting policies are described under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations—Critical accounting policies and use of estimates" in our Annual Report on Form 10-K for the year ended December 31, 2019 and the notes to the unaudited condensed consolidated financial statements included in Part I, Item 1, "Financial Statements (unaudited)," of this quarterly report on Form 10-Q. We believe that of our critical accounting policies, the following accounting policies involve the most judgment and complexity:

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

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

Results of operations

Comparison of the three months ended September 30, 2020 and 2019

The following table summarizes our results of operations for the three months ended September 30, 2020 and 2019:

(in thousands)	Three Months Ended September 30, 2020	2019	Increase (decrease)
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	16,045	22,792	(6,747)
General and administrative	5,181	6,925	(1,744)
Total operating expenses	21,226	29,717	(8,491)
Loss from operations	(21,226)	(29,717)	8,491
Other income (expense):			
Interest (expense) income	(20)	406	(426)
Other income	—	56	(56)
Total other income (expense)	(20)	462	(482)
Net loss	$ (21,246)	$ (29,255)	$ 8,009

Research and development expenses

(in thousands)	Three Months Ended September 30, 2020	2019	Increase (decrease)
SGT-001	$ 8,628	$ 9,323	$ (695)
Other product candidates	83	544	(461)
Unallocated research and development expenses			
Personnel related expenses	5,179	9,357	(4,178)
External expenses	2,155	3,568	(1,413)
Total unallocated research and development expenses	7,334	12,925	(5,591)
Total research and development expenses	$ 16,045	$ 22,792	$ (6,747)

Research and development expenses for the three months ended September 30, 2020 were $16.0 million, compared to $22.8 million for the three months ended September 30, 2019. The decrease of $6.8 million in research and development costs was due to a decrease in unallocated research and development costs of $5.6 million, primarily due to a reduction in personnel and facility related expenses as a result of the restructuring that occurred in January 2020, a $0.7 million decrease in costs related to our lead product candidate SGT-001, driven by a reduction in manufacturing costs of $0.2 million, and a decrease in clinical costs of $0.5 million. The reduction in research and development was also driven by a $0.5 million decrease in costs related to other product candidates as we focus on advancing SGT-001.

General and administrative expenses

General and administrative expenses were $5.2 million for the three months ended September 30, 2020, compared to $6.9 million for the three months ended September 30, 2019. The decrease of $1.7 million was due to a decrease in personnel related expenses of $1.5 million partially due to the restructuring that occurred in January 2020 and a net decrease in corporate expenses of $0.2 million.

Interest (expense) income

Interest expense was less than $0.1 million for the three months ended September 30, 2020, compared to interest income of $0.4 million for the three months ended September 30, 2019. The decrease was primarily related to a reduction of available-for-sale securities included within our portfolio.

Other income

Other income for the three months ended September 30, 2020 was $0 million compared to $0.1 million for the three months ended September 30, 2019. Other income relates to contributions from charitable organizations. We do not expect these contributions to be significant in future periods.

Comparison of the nine months ended September 30, 2020 and 2019

The following table summarizes our results of operations for the nine months ended September 30, 2020 and 2019:

(in thousands)	Nine Months Ended September 30, 2020	2019	Increase (decrease)
Revenue	$ —	$ —	$ —
Operating expenses:			
Research and development	49,158	67,671	(18,513)
General and administrative	15,957	19,317	(3,360)
Restructuring charges	1,944	—	1,944
Total operating expenses	67,059	86,988	(19,929)
Loss from operations	(67,059)	(86,988)	19,929
Other income (expense):			
Interest income	131	1,281	(1,150)
Other income	1	345	(344)
Total other income (expense)	132	1,626	(1,494)
Net loss	$ (66,927)	$ (85,362)	$ 18,435

Research and development expenses

(in thousands)	Nine Months Ended September 30, 2020	2019	Increase (decrease)
SGT-001	$ 24,408	$ 29,390	$ (4,982)
Other product candidates	465	3,726	(3,261)
Unallocated research and development expenses			
Personnel related expenses	16,512	23,335	(6,823)
External expenses	7,773	11,220	(3,447)
Total unallocated research and development expenses	24,285	34,555	(10,270)
Total research and development expenses	$ 49,158	$ 67,671	$ (18,513)

Research and development expenses for the nine months ended September 30, 2020 were $49.2 million, compared to $67.7 million for the nine months ended September 30, 2019. The decrease of $18.5 million in research and development costs was due to a decrease in unallocated research and development costs of $10.3 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 $5.0 million decrease in costs related to our lead product candidate SGT-001, driven by a reduction in manufacturing costs of $5.6 million partially offset by an increase in clinical costs of $0.6 million primarily driven by clinical consulting costs. The reduction in research and development was also driven by a $3.2 million decrease in costs related to other product candidates as we focus on advancing SGT-001.

General and administrative expenses

General and administrative expenses were $16.0 million for the nine months ended September 30, 2020, compared to $19.3 million for the nine months ended September 30, 2019. The decrease of $3.3 million was due to a decrease in personnel related expenses of $2.8 million partially due to the restructuring that occurred in January 2020 and a net decrease in corporate expenses of $0.5 million.

Restructuring Charges

During the nine months ended September 30, 2020, we recorded charges of $1.9 million related to severance and other employee-related costs. We paid approximately $1.7 million during the nine months ended September 30, 2020 and expect to pay approximately $0.3 million in the next three months.

Interest income

Interest income was $0.1 million for the nine months ended September 30, 2020, compared to $1.3 million for the nine months ended September 30, 2019. The decrease was primarily related to a reduction of available-for-sale securities included within our portfolio.

Other income

Other income for the nine months ended September 30, 2020 was less than $0.1 million compared to $0.3 million for the nine months ended September 30, 2019. Other income relates to contributions 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 a private placements of shares of our common stock and pre-funded warrants to purchase shares of our common stock. Through September 30, 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 and an aggregate of $57.9 million of net proceeds, after deducting offering costs, from our July 2019 private placement.

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

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. As of September 30, 2020, we had cash and cash equivalents of $24.8 million and had no debt outstanding.

In October 2020, we entered into a collaboration and license agreement, or the Collaboration Agreement, with Ultragenyx Pharmaceutical Inc., or 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.

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 nine months ended September 30, 2020, we did not sell any shares under the ATM Sales Agreement. In October 2020, we sold 6,309,632 shares pursuant to the ATM Sales Agreement resulting in net proceeds of $23.2 million.

Cash flows

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

(in thousands)	Nine Months Ended September 30, 2020	2019	Increase (decrease)
Cash used in operating activities	$ (57,906)	$ (71,469)	$ 13,563
Cash provided by investing activities	6,660	21,661	(15,001)
Cash provided by financing activities	—	58,162	(58,162)
Net (decrease)/increase in cash, cash equivalents and restricted cash	$ (51,246)	$ 8,354	$ (59,600)

Operating activities

During the nine months ended September 30, 2020, operating activities used $57.9 million of cash, primarily resulting from our net loss of $66.9 million and cash used in changes in our operating assets and liabilities of $3.2 million offset by non-cash charges of $12.2 million due primarily to equity-based compensation of $9.1 million and depreciation expense of $3.1 million. Net cash used in changes in our operating assets and liabilities during the nine months ended September 30, 2020 consisted primarily of a reduction in

accrued expenses and other liabilities of $2.0 million and a decrease in accounts payable of $2.6 million partially offset by a net decrease in prepaid expenses and other assets of $1.5 million. These changes were primarily due to the timing of payments.

During the nine months ended September 30, 2019, operating activities used $71.5 million of cash, primarily resulting from our net loss of $85.4 million offset by non-cash charges of $12.6 million due primarily to equity-based compensation of $10.8 million and depreciation expense of $2.0 million as well as $1.3 million of cash provided by changes in our operating assets and liabilities. Net cash provided by changes in our operating assets and liabilities during the nine months ended September 30, 2019 consisted primarily of an increase in accrued expenses and other liabilities of $2.4 million partially offset by an increase in prepaid expenses and other assets of $1.1 million, which was primarily due to up-front research and development expenses.

Investing activities

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

During the nine months ended September 30, 2019, investing activities provided $21.7 million of cash, consisting primarily of proceeds from maturities of available-for-sale securities offset by purchases of property and equipment, and available-for-sale securities.

Financing activities

During the nine months ended September 30, 2020, there were no financing activities.

During the nine months ended September 30, 2019, net cash provided by financing activities was $58.2 million, due to the proceeds from a private placement of shares of our common stock and pre-funded warrants to purchase shares of our common stock completed on July 30, 2019, partially offset by $1.8 million of payments made in connection with costs incurred for the private placement.

Funding requirements

We expect that our expenses will increase substantially if and as we:

- seek to enroll patients in IGNITE DMD and continue clinical development of SGT-001;
- move other product candidates into clinical trials;
- continue research and preclinical development of 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.

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

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.

As of September 30, 2020, we had cash and cash equivalents of $24.8 million. We believe that our existing cash and cash equivalents as of September 30, 2020, together with the proceeds of $40.0 million received in October 2020 from the sale of shares of common stock to Ultragenyx as well as the proceeds of $23.2 million received from the sale of shares of common stock in October 2020 pursuant to a sales agreement, dated March 13, 2019, between the Company and Jefferies LLC (the "ATM Sales Agreement"), will be sufficient to fund its operating expenses and capital requirements into the second half of 2021. 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 other product candidates;
- the costs, timing and outcome of regulatory review of SGT-001 and 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 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;
- 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 intend to supply our clinical development program for SGT-001 with drug product produced at a current good manufacturing practices, or cGMP, compliant facility located at our Contract Development Manufacturing Organization partner. We intend to establish the capability and capacity to supply SGT-001 at commercial scale from multiple sources.

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

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

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

Recently Issued Accounting Pronouncements

See Note 2 to the condensed consolidated financial statements included elsewhere in this quarterly report on Form 10-Q for information regarding recently adopted and issued accounting pronouncements. See also Note 2 to our consolidated financial statements included in our annual report on Form 10-K for the year ended December 31, 2019.

Emerging Growth Company Status

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

Off-Balance Sheet Arrangements

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

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related changes in interest rates. As of September 30, 2020, our 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 the investments in our portfolio, an immediate 10% change in market interest rates would not have a material impact on the fair market value of our investment portfolio or on our financial position or results of operations.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our President and Chief Executive Officer and our Chief Financial Officer (our principal executive officer and principal financial officer, respectively), evaluated the effectiveness of our disclosure controls and procedures as of September 30, 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, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2020, our President and Chief Executive Officer and our Chief Financial Officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended September 30, 2020 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

None.

Item 1A. Risk Factors.

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

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 $66.9 million for the nine months ended September 30, 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 September 30, 2020, we had an accumulated deficit of $383.2 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:

- seek 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 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 September 30, 2020 together with the proceeds of $40.0 million received in October 2020 from the sale of shares of common stock to Ultragenyx as well as the net proceeds of $23.2 million received from the sale of shares of common stock in October 2020 pursuant to a sales agreement, dated March 13, 2019, between the Company and Jefferies LLC (the "ATM Sales Agreement"), will be sufficient to fund its operating expenses and capital requirements into the second half of 2021, 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;
- the success of our collaboration with Ultragenyx;
- our ability to establish and maintain additional strategic collaborations, licensing or other arrangements and the financial terms of such arrangements;
- the payment or receipt of milestones, royalties and other collaboration-based revenues, if any;
- the extent to which we acquire or in-license other product candidates, technologies and intellectual property; and
- if and as we need to adapt our business in response to the COVID-19 pandemic and its collateral consequences.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our

product revenue, if any, will be derived from or based on sales of product candidates that may not be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

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

The report from our independent registered public accounting firm for the year ended December 31, 2019 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 COVID-19 pandemic, which began in late 2019 and has spread worldwide, 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 has caused substantial disruption in the financial markets and may adversely impact economies worldwide, both of which could result in adverse effects on our business and operations.

The global outbreak and spread of the novel strain of coronavirus, or the COVID-19 pandemic, which began in December 2019 and has spread worldwide, 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. 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.

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 significant 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. We reported the event to the FDA and the study Data Safety Monitoring Board. Following notification, the FDA placed IGNITE DMD on clinical hold. In December 2019, we reported that the serious adverse event had fully resolved and that the patient had resumed his normal activities. In April 2020, we provided the FDA with information and measures intended to improve patient safety, and in May 2020, we received written communication from the FDA that the trial remained in hold. In June 2020, we submitted a response to the FDA that provided data related to manufacturing process improvements. In July 2020, 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 total viral load to be administered per patient. 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 are amending 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; 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, our lead product candidate, or any other product candidate will increase our costs, slow down our product candidate development and approval process and delay or potentially jeopardize our ability to commence product sales and generate revenue. In addition, many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may also ultimately lead to the denial of regulatory approval of SGT-001 or our 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, 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, 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.

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 further 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 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.

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

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

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

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

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

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

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for SGT-001 or our other product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced, and our ability to realize the full market potential of our product candidates will be harmed.

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

The FDA has established the Office of Tissues and Advanced Therapies, or the OTAT, within the Center for Biologics Evaluation and Research, or the CBER, to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the U.S. National Institutes of Health, or the NIH, also are potentially subject to review by the 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.

While the opportunity to receive a priority review voucher was meant to expire for those companies that had not received a designation by September 30, 2020, Congress authorized a short term extension on that date. According to these new sunset provisions, after December 11, 2020, the FDA may only award a voucher for an approved rare pediatric disease product application if the sponsor has the designation for the drug and that designation was granted by December 11, 2020. After December 11, 2022, the FDA may not award any priority review vouchers. The Creating Hope Reauthorization Act, which was received in the Senate on September 30, 2020, proposes to replace those cutoffs with "September 30, 2024" and "September 30, 2026," respectively, thus extending the authorized period for rare pediatric disease designation and granting of rare pediatric disease priority review vouchers from the 21st Century Cures Act by four years. It remains to be seen, however, whether such legislation will be enacted into law.

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 I 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 intend to utilize materials manufactured by cGMP-compliant third-party suppliers. Even following our potential establishment of a validated cGMP manufacturing facility, we intend to 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.

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

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

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

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

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

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

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

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

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

To our knowledge, 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, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. Following protracted negotiations, the United Kingdom left the European Union on January 31, 2020. Under the withdrawal agreement, there is a transitional period until December 31, 2020, which period is extendable up to two years. Discussions between the United Kingdom and the European Union have so far mainly focused on finalizing withdrawal issues and transition agreements but have been extremely difficult to date. To date, only an outline of a trade agreement has been reached. Much remains open but the United Kingdom's Prime Minister has indicated that the United Kingdom will not seek to extend the transitional period beyond the end of 2020. If no trade agreement has been reached before the end of the transitional period, there may be significant market and economic disruption. The Prime Minister has also indicated that the UK will not accept high regulatory alignment with the EU.

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 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, may force us to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

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

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

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

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

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

Risks related to our business operations

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

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

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

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.

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 during the next Congressional session.

The CARES Act, which was signed into law on March 27, 2020, and designed to provide financial support and resources to individuals and businesses affected by the COVID-19 pandemic, suspended the 2% Medicare sequester from May 1, 2020, through December 31, 2020, and extended the sequester by one year, through 2030, in order to offset the added expense of the 2020 cancellation. We will continue to evaluate the effect that the Health Care Reform Law and additional amendments, including its possible repeal and replacement could have on our business. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace Health Care Reform Law provisions is uncertain in many respects, it is also possible that some of the Health Care Reform Law provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with Health Care Reform Law coverage expansion provisions. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop commercialize product candidates.

The Trump administration has also taken executive actions to undermine or delay implementation of the Health Care Reform Law. Since January 2017, President Trump has signed two Executive Orders designed to delay the implementation of certain provisions of the Health Care Reform Law or otherwise circumvent some of the requirements for health insurance mandated by the Health Care Reform Law. One Executive Order directs 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. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the Health Care Reform Law. Several state Attorneys General filed suit to stop the Trump administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. In addition, CMS has recently 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. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12 billion in Health Care Reform Law risk corridor payments to third-party payors who argued such payments were owed to them. The U.S. Supreme Court subsequently agreed to hear this case and, on April 27, 2020, it overruled the decision of the Court of Appeals for the Federal Circuit.

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.

Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the Health Care Reform Law is an essential and inseverable feature of the Health Care Reform Law, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act of 2017, the remaining provisions of the Health Care Reform Law are invalid as well. The Trump administration and the CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018, the same judge issued an order staying the judgment pending appeal. The Trump administration thereafter represented to the Court of Appeals considering this judgment that it does not oppose the district court's ruling. On July 10, 2019, the Court of Appeals for the Fifth Circuit heard oral argument in this case. In those arguments, the Trump administration argued in support of upholding the lower court decision. On December 18, 2019, the Court of Appeals for the Fifth Circuit 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. On January 21, 2020, the U.S. Supreme Court declined to review this decision on an expedited basis. On March 3, 2020, however, the U.S. Supreme Court agreed to hear this case. On June 25, 2020, the Trump administration and a coalition of 18 states asked the court to strike down the entirety of the Health Care Reform Law. The case is scheduled for oral argument before the court on November 10, 2020. 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 costs of prescription pharmaceuticals in the United States has also been the subject of considerable discussion in the United States, and members of Congress and the Trump administration have stated that they will address such costs through new legislative and administrative measures. The pricing of prescription pharmaceuticals is also subject to governmental control outside the United States. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost effectiveness of our product candidates to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our ability to generate revenues and become profitable could be impaired.

Specifically, there have been several recent U.S. congressional inquiries and proposed federal and proposed and enacted state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. For example, on May 11, 2018, the Trump administration issued a plan to lower drug prices. Under this blueprint for action, the Trump administration indicated that the Department of Health and Human Services, or HHS, will: take steps to end the gaming of regulatory and patent processes by drug makers to unfairly protect monopolies; advance biosimilars and generics to boost price competition; evaluate the inclusion of prices in drug makers' ads to enhance price competition; speed access to and lower the cost of new drugs by clarifying policies for sharing information between insurers and drug makers; avoid excessive pricing by relying more on value-based pricing by expanding outcome-based payments in Medicare and Medicaid; work to give Part D plan sponsors more negotiation power with drug makers; examine which Medicare Part B drugs could be negotiated for a lower price by Part D plans, and improving the design of the Part B Competitive Acquisition Program; update Medicare's drug-pricing dashboard to increase transparency; prohibit Part D contracts that include "gag rules" that prevent pharmacists from informing patients when they could pay less out-of-pocket by not using insurance; and require that Part D plan members be provided with an annual statement of plan payments, out-of-pocket spending, and drug price increases. Finally, the Trump administration's budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower-cost generic and biosimilar drugs.

More recently, President Trump issued five executive orders that are intended to lower the costs of prescription drug products. The first order would require all federally qualified health centers to pass on to patients the discounts the health centers receive on insulin and epinephrine through Medicare's 340B Drug Discount Program. The second order would establish an international pricing index that would set the price Medicare Part B pays for the costliest medications covered under the program to the lowest price in other economically advanced countries.

The third order is intended to reduce the costs of drugs by supporting the safe importation of prescription drugs. Specifically, the order calls upon HHS to facilitate grants to individuals of waivers of the prohibition of importation of prescription drugs that would allow patients to import FDA approved drug products from abroad, so long as doing so would result in lower costs. In addition, the order would allow wholesalers and pharmacies to re-import both biological drugs and insulin that were originally manufactured in the United States and then exported for international sale. This action preceded the finalization of a rulemaking on September 24, 2020 that allows states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants would be 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. At the same time, the FDA 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).

The fourth executive order would end drug rebates used by health plan sponsors, pharmacies or pharmacy benefit managers, or PBMs, in operating the Medicare Part D program. Specifically, the order directs HHS to exclude from safe harbor protections under the federal anti-kickback statute retroactive price reductions that are not applied at the point-of-sale. Instead, the order requires HHS to establish new safe harbors that would allow health plan sponsors, pharmacies, and PBMs to pass on those discounts to consumers at point-of-sale in order to lower the patient's out-of-pocket costs and permit the use of certain bona fide PBM service fees. Each of these orders directs the federal government to implement the initiatives outlined in the orders, meaning they will not have immediate effects.

Finally, the fifth order instructs the federal government to develop a list of "essential" medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including especially China. The order is meant reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States.

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.

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

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.

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 our Annual Report on Form 10-K for the year ended December 31, 2019.

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 63.2%of our capital stock as of November 1, 2020. 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 asubstantial 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, we completed a private placement of 10,607,525 shares of our common stock and 2,295,699 pre-funded warrants to purchase shares of our common stock to several accredited investors. We have filed a registration statement covering the resale of these shares by the purchasers in the private placement and have agreed to keep such registration statement effective until the date the shares covered by the registration statement have been sold or can be resold without restriction under Rule 144 of the Securities Act.

In addition, on January 29, 2018, we filed a Registration Statement on Form S-8 to register approximately 5.0 million shares reserved for future issuance under our 2018 Omnibus Incentive Plan and on August 6, 2020, we filed a Registration Statement on Form S-8 to register approximately 7.9 million shares reserved for future issuance under our 2020 Equity Incentive Plan, which shares will become eligible for sale in the public market in the future, subject to certain legal and contractual limitations. These shares can be freely sold in the public market upon issuance, subject to black-out periods and volume limitations applicable to affiliates.

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

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

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

- results of or developments in preclinical studies and clinical trials of SGT-001 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 in our Annual Report on Form 10-K, 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 other 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, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

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

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

- establish a classified board of directors such that not all members of our board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;

- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called "poison pill," that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least two-thirds of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our certificate of incorporation or bylaws.

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

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

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

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

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

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Recent Sales of Unregistered Securities

On July 1, 2020, we issued a warrant to purchase 8,444 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 $2.92 per share and expires on July 1, 2025. The shares issuable upon exercise of the Warrant vest upon the consultant's achievement of certain specified milestones. We offered and sold the Warrant pursuant to an exemption from registration under Section 4(a)(2) of the Securities Act.

Except as stated in this quarterly report on Form 10-Q, we did not sell any securities that were not registered under the Securities Act during the three months ended September 30, 2020.

Item 6. Exhibits.

Exhibit Number	Description
10.1+	Collaboration and License Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc.
10.2	Stock Purchase Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc.
10.3+	Investor Agreement, dated as of October 22, 2020, by and between the Company and Ultragenyx Pharmaceutical Inc
10.4+	First Amendment, dated as of October 9, 2020, to the Exclusive Patent License by and between the Company and the University of Washington.
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)

+ Certain portions of this exhibit have been omitted because they are not material and would likely cause competitive harm to the Registrant if disclosed.

SIGNATURES

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

Solid Biosciences Inc.

Date: November 5, 2020

By: /s/ Ilan Ganot

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

Date: November 5, 2020

By: /s/ Jennifer Ziolkowski

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

Exhibit 10.1

Execution Version
CONFIDENTIAL

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

COLLABORATION AND LICENSE AGREEMENT

BETWEEN

ULTRAGENYX PHARMACEUTICAL INC.

AND

SOLID BIOSCIENCES INC.

October 22, 2020

TABLE OF CONTENTS

SCHEDULES AND EXHIBITS

COLLABORATION AND LICENSE AGREEMENT

This Collaboration and License Agreement (this "**Agreement**") is entered into as of October 22, 2020 (the "**Effective Date**") by and between Ultragenyx Pharmaceutical Inc., a corporation organized under the laws of the State of Delaware ("**Ultragenyx**"), and Solid Biosciences Inc., a corporation organized under the laws of the State of Delaware ("**Solid**"). Ultragenyx and Solid each may be referred to herein individually as a "**Party**" or collectively as the "**Parties**".

RECITALS

WHEREAS, Solid controls certain Patents and Know-How, technology and expertise relating to an MD5 nNOS binding domain form of microdystrophin;

WHEREAS, Ultragenyx is a biopharmaceutical company committed to bringing patients novel products for the treatment of rare and ultra-rare diseases, with expertise in the development, manufacture and commercialization of biological and pharmaceutical products; and

WHEREAS, the Parties desire to enter into a strategic collaboration whereby Solid will grant to Ultragenyx an exclusive license under the Licensed Technology and Solid's rights in the Joint Technology with respect to Licensed Products, Solid will provide certain non-clinical and clinical development support with respect to the development of Licensed Products, and Ultragenyx will grant to Solid an exclusive option to co-invest in Licensed Products in return for participating in income and losses with respect to such Licensed Products in the Option Territory.

NOW, THEREFORE, in consideration of the respective covenants, representations, warranties and agreements set forth herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties, intending to be legally bound, hereto agree as follows:

ARTICLE 1
DEFINITIONS

For purposes of this Agreement, the following capitalized terms will have the following meanings:

1.1 "**Accounting Standards**" means, with respect to a Party or its Affiliate or Sublicensee, GAAP, as such Party, Affiliate or Sublicensee uses for its financial reporting obligations, consistently applied.

1.2 "**Affiliate**" means, as of any point in time and for so long as such relationship continues to exist with respect to any Person, any other Person that controls, is controlled by or is under common control with such Person. A Person will be regarded as in control of another Person if it (a) owns or controls, directly or indirectly, more than fifty percent (50%) of the equity securities of the subject Person entitled to vote in the election of directors (or, in the case of a Person that is not a corporation, for the election of the corresponding managing authority), or (b) possesses, directly or indirectly, the power to direct or cause the direction of the management or policies of such Person (whether through ownership of securities or other ownership interests, by contract or otherwise).

1.3 "**Agreement**" has the meaning set forth in the Preamble.

1.4 "**Alliance Manager**" has the meaning set forth in Section 7.4.

1.5 "**Allocation Methodology**" has the meaning set forth in Exhibit B.

1.6 "**Allowable Expenses**" has the meaning set forth in Exhibit B.

1.7 "**Applicable Law**" means all applicable laws, statutes, rules, regulations and other pronouncements having the effect of law of any federal, national, multinational, state, provincial, county, city or other political subdivision, agency or other body, domestic or foreign, including any applicable rules, regulations, guidelines, or other requirements of the Regulatory Authorities that may be in effect from time to time, including the United States Federal Food, Drug, and Cosmetic Act, as amended, GCP, GLP and GMP, anti-bribery laws, such as the United States Anti-Kickback Statute, Foreign Corrupt Practices Act and UK Bribery Act, as well as all applicable data protection and privacy laws, rules and regulations, including the United States Department of Health and Human Services privacy rules under the Health Insurance Portability and Accountability Act, as amended, and the Health Information Technology for Economic and Clinical Health Act and the EU General Data Protection Regulation 2016/679 of the European Parliament and of the Council of 27 April 2016 on the protection of natural persons with regard to the processing of personal data and on the free movement of such data, and repealing Directive 95/46/EC, along with other country-level data protection laws, as may be applicable.

1.8 "**Approval Application**" means a BLA or similar application or submission for a pharmaceutical product to a Regulatory Authority in a country or group of countries to obtain Marketing Approval for such pharmaceutical product in that country or group of countries, including any amendment thereof.

1.9 "**Approval Milestone Event**" has the meaning set forth in Section 9.2.2.

1.10 "**Approval Milestone Payment**" has the meaning set forth in Section 9.2.2.

1.11 "**Audited Party**" has the meaning set forth in Section 9.10.

1.12 "**Auditing Party**" has the meaning set forth in Section 9.10.

1.13 "**Balancing Payment**" has the meaning set forth in Exhibit B.

1.14 "**Bankrupt Party**" has the meaning set forth in Section 8.5.

1.15 "**Bankruptcy Code**" has the meaning set forth in Section 8.5.

1.16 "**BLA**" means a Biologics License Application filed with the FDA pursuant to 42 U.S.C. § 262 (as may be amended or replaced), or any other equivalent application or regulatory mechanism or authorization filed with the applicable regulatory authority of a country in the Territory other than the United States.

1.17 "**Blocking Third Party Intellectual Property**" means, with respect to a Licensed Product in any country, [**].

1.18 "**Blocking Third Party Intellectual Property Costs**" means [**].

1.19 "**Breaching Party**" means the Party that is believed by the other Party to be in material breach of this Agreement.

1.20 "**Business Day**" means a day, other than (a) a Saturday or Sunday, on which banks in each of Boston, Massachusetts, and San Francisco, California are open for commercial banking business or (b) a day during Ultragenyx's summer shutdown (as of the Effective Date, typically in early August of each Calendar Year) or Ultragenyx's winter shutdown (as of the Effective Date, typically December 25 through January 1 of each Calendar Year).

1.21 "**Calendar Quarter**" means the respective periods of three (3) consecutive calendar months ending on March 31, June 30, September 30 or December 31, during the Term, or the applicable part thereof during the first or last calendar quarter of the Term.

1.22 "**Calendar Year**" means any year commencing on January 1 and ending on December 31, or the applicable part thereof during the first or last year of the Term.

1.23 "**Calendar Year Net Sales**" means, on a Licensed Product-by-Licensed Product basis, the total Net Sales by Selling Parties in the Territory of such Licensed Product in a particular Calendar Year.

1.24 "**CDA**" has the meaning set forth in Section 1.35.

1.25 "**Change of Control**" means, with respect to a Party, (a) a merger or consolidation of such Party with a Third Party that results in the voting securities of such Party outstanding immediately prior thereto, or any securities into which such voting securities have been converted or exchanged, ceasing to represent more than fifty percent (50%) of the combined voting power of the surviving entity or the parent of the surviving entity immediately after such merger or consolidation, (b) a transaction or series of related transactions in which a Third Party, together with its Affiliates, becomes the beneficial owner of more than fifty percent (50%) of the combined voting power of the outstanding securities of such Party, or (c) the sale or other transfer to a Third Party of all or substantially all of such Party's business to which the subject matter of this Agreement relates.

1.26 "**Clinical Trial**" means a clinical study in humans to obtain information regarding a product, including information relating to the safety, tolerability, pharmacological activity, pharmacokinetics, dose ranging or efficacy of such product.

1.27 "**Combination Product**" has the meaning set forth in Section 1.118.

1.28 "**Commercial Milestone Event**" has the meaning set forth in Section 9.2.3.

1.29 "**Commercial Milestone Payment**" has the meaning set forth in Section 9.2.3.

1.30 "**Commercialize**" or "**Commercializing**" means, in respect of a Licensed Product, to (a) market, advertise, promote, detail, distribute, offer for sale, sell, have sold, import, have imported, export, have exported or otherwise exploit, (b) conduct activities, other than Development and Manufacturing, in preparation for the foregoing activities, including obtaining Price Approval, (c) conduct post-Marketing Approval commitments or studies (including Post-Marketing Clinical Trials), or (d) conduct other activities in connection with the foregoing activities, including activities related to medical affairs matters, sales force matters, and pharmacovigilance matters. Cognates of the word "Commercialize" will have correlative meanings.

1.31 "**Commercially Reasonable Efforts**" means (a) with respect to Ultragenyx's efforts, [**]; and (b) with respect to Solid's efforts, [**].

1.32 "**Committee**" means each of the Joint Steering Committee, Joint Finance Committee and each Subcommittee.

1.33 "**Competing Product**" means, on a Licensed Product-by-Licensed Product basis, an AAV based gene therapy product that is (a) Controlled by Solid or any of its Affiliates or its or their licensees, (b) directed at the same Indication as the Licensed Product, (c) for which Solid or any of its Affiliates or its or their licensees has Initiated clinical Development or commenced Commercialization, and (d) for which Solid has not abandoned or terminated such clinical Development or Commercialization as of the date of the Solid Exercise Notice.

1.34 "**Competitive Infringement**" means, on a country-by-country basis, where the actual or suspected research, development, manufacture or commercialization by any Third Party of any biological or pharmaceutical product comprising any AAV8 or another clade E AAV variant pharmaceutical product that expresses Solid's MD5 nNOS binding domain form of microdystrophin, and is (a) Covered by a Valid Claim of any Licensed Patent or Joint Patent and (b) may reasonably be expected to result in a loss of Net Sales of a Licensed Product (regardless of whether such Licensed Product has been launched). For clarity, filing of a BLA with respect to a Licensed Product as the reference product by any Third Party will be deemed to be Competitive Infringement.

1.35 "**Confidential Information**" means, with respect to each Party, all Know-How or other information, including proprietary information (whether or not patentable) regarding or embodying such Party's technology, products, business information or objectives, that is communicated in any way or form by or on behalf of the Disclosing Party to the Receiving Party or its permitted recipients, pursuant to this Agreement or that certain Confidentiality Agreement between the Parties dated [**], as amended or restated from time to time (the "**CDA**"), whether or not such Know-How or other information is identified as confidential at the time of disclosure. Notwithstanding the foregoing, Confidential Information does not include any Know-How or information that: (a) was already known by the Receiving Party (other than under an obligation of confidentiality to the Disclosing Party) at the time of disclosure by or on behalf of the Disclosing Party; (b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (c) became generally available to the public or otherwise part of the public domain after its disclosure to the Receiving Party, other than through any act or omission of the Receiving Party in breach of its obligations under this Agreement; (d) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the

Disclosing Party not to disclose such information to the Receiving Party; or (e) was independently discovered or developed by or on behalf of the Receiving Party without the use of any Confidential Information belonging to the Disclosing Party. Confidential Information disclosed to the Receiving Party hereunder will not be deemed to fall within the foregoing exceptions merely because broader or related information falls within such exceptions, nor will combinations of elements or principles be considered to fall within the foregoing exceptions merely because individual elements of such combinations fall within such exceptions. Without limiting the foregoing, and notwithstanding clauses (a), (d) and (e) of the preceding sentence: (i) the terms of this Agreement will be considered Confidential Information of both Parties, with both Parties deemed to be the Receiving Party of such Confidential Information and (ii) any Know-How that is subject to a Party's ownership rights under this Agreement will be deemed to be the Confidential Information of such Party and the other Party will be deemed to be the Receiving Party of such Know-How.

1.36 "**Control**" or "**Controlled**" means, with respect to a Party or its Affiliate, and any Know-How or Patent, possession of the ability by such Party or its Affiliate (whether by sole or joint ownership, license or otherwise), other than pursuant to this Agreement, to grant, without violating the terms of any agreement with a Third Party, a license, access or other right in, to or under such Know-How or Patent. Notwithstanding anything in this Agreement to the contrary, a Party and its Affiliates will be deemed to not Control any Know-How or Patents that are owned or controlled by a Third Party described in the definition of "Change of Control," or such Third Party's Affiliates (other than an Affiliate of such Party prior to the Change of Control), (a) prior to the closing of such Change of Control, except to the extent that any such Know-How or Patents were created, conceived, discovered or generated by such Third Party prior to such Change of Control using or incorporating such Party's or its pre-existing Affiliate's Know-How or Patents, or (b) after such Change of Control to the extent that such Know-How or Patents are created, conceived, discovered or generated by such Third Party or its Affiliates (other than such Party or its pre-existing Affiliates) after such Change of Control without using or incorporating such Party's or its pre-existing Affiliate's Know-How or Patents.

1.37 "**Cost/Income Share**" has the meaning set forth in Section 3.3.6.

1.38 "**Cost/Income Sharing Terms**" has the meaning set forth in Section 3.3.6.

1.39 "**Cover**," "**Covering**" or "**Covers**" means (a) as to a product and a Patent, that, in the absence of a license granted under, or ownership of, such Patent, the making, using, selling, offering for sale or importation of such product would infringe such Patent or, as to a pending claim included in such Patent, the making, using, selling, offering for sale or importation of such product would infringe such Patent if such pending claim were to issue in an issued patent without modification, (b) as to Know-How and a Patent, that, in the absence of a license granted under, or ownership of, such Patent, the use or practice of such Know-How would infringe such Patent or, as to a pending claim included in such Patent, the use or practice of such Know-How would infringe such Patent if such pending claim were to issue in an issued patent without modification and (c) as to a compound, product or technology and Know-How, that the Exploitation of such compound, product or technology incorporates, uses, employs, embodies, or practices such Know-How.

1.40 "**Data Package**" means the data package described on Schedule 1.40.

1.41 "**Development**" means, with respect to a Licensed Product, all (a) non-clinical and pre-clinical discovery, research and development activities and optimization completed prior to filing an IND with respect to such Licensed Product, including animal and toxicology studies, and (b) clinical and non-clinical research and development activities conducted after filing of an IND with respect to such Licensed Product, including toxicology, pharmacology test method development and stability testing, process development, formulation development, delivery system development, quality assurance and quality control development, statistical analysis, Clinical Trials (other than Post-Marketing Clinical Trials), regulatory affairs, pharmacovigilance, Clinical Trial regulatory activities and obtaining and maintaining Marketing Approval. Cognates of the word "Development" will have correlative meanings.

1.42 "**Development Milestone Event**" has the meaning set forth in Section 9.2.1.

1.43 "**Development Milestone Payment**" has the meaning set forth in Section 9.2.1.

1.44 "**Development Option**" has the meaning set forth in Section 3.1.

1.45 "**Development Option Period**" has the meaning set forth in Section 3.3.4.

1.46 "**Development Share Product**" has the meaning set forth in Section 3.3.1.

1.47 "**Disclosing Party**" has the meaning set forth in Section 14.1.

1.48 "**Dispute**" has the meaning set forth in Section 15.10.

1.49 "**Distribution Costs**" has the meaning set forth in Exhibit B.

1.50 "**Effective Date**" has the meaning set forth in the Preamble.

1.51 "**EMA**" means the European Medicines Agency and any successor entity thereto.

1.52 "**European Commission**" means the European Commission or any successor entity that is responsible for granting Marketing Approvals authorizing the sale of pharmaceuticals in the European Union.

1.53 "**European Union**" or "**EU**" means the European Union and all its then-current member countries but including in any case the Major European Countries regardless of whether they are then-current member countries.

1.54 "**Exclusions**" has the meaning set forth in Exhibit B.

1.55 "**Existing In-License Agreements**" has the meaning set forth in Section 11.2(f).

1.56 "**Expert**" means a Person with no less than [**] years of experience in the pharmaceutical and life sciences industries, with expertise in determining financial matters similar to the FTE Rate, but

excluding any current or former employee or consultant of either Party. Such person will be fluent in the English language.

1.57 "**Exploit**" means 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. Cognates of the word "**Exploit**" will have correlative meanings.

1.58 "**FD&C Act**" means the United States Federal Food, Drug, and Cosmetic Act, as amended, and the rules and regulations promulgated thereunder.

1.59 "**FDA**" means the United States Food and Drug Administration and any successor entity thereto.

1.60 "**Field**" means the diagnosis, treatment, cure, mitigation or prevention of Duchenne Muscular Dystrophy and other disease indications resulting from a lack of functional dystrophin, including Becker Muscular Dystrophy.

1.61 "**Final Balancing Report**" has the meaning set forth in Exhibit B.

1.62 "**First Achievement of Clinical POC**" means the receipt by Ultragenyx of final tables, listings and figures from a Clinical Trial that is supportive of, and subsequently results in, Ultragenyx deciding to Initiate the first Registrational Study for a Licensed Product contemplated by the Option Territory Development Plan. The date of First Achievement of Clinical POC will be the date Ultragenyx formally decides to Initiate such Registrational Study.

1.63 "**First Commercial Sale**" means with respect to a Licensed Product, the first sale of such Licensed Product by a Selling Party to a Third Party resulting in Net Sales in a particular country after any required Marketing Approval (excluding Price Approval) for the Licensed Product has been obtained in such country; provided that the following will not constitute a First Commercial Sale: (a) any sale of a Licensed Product to an Affiliate or Sublicensee; (b) any sale of a Licensed Product for use in Clinical Trials or other Development activities; or (c) the disposal or transfer of a Licensed Product for a *bona fide* charitable purpose; or (d) compassionate use, "named patient sales", expanded access sales or right to try sales.

1.64 "**Force Majeure**" means a condition, the occurrence and continuation of which is beyond the reasonable control of a Party, including an act of God, governmental acts or restrictions, war, civil commotion, labor strike or lock-out, epidemic, pandemic, flood, failure or default of public utilities or common carriers, or destruction of production facilities or materials by fire, earthquake, storm or like catastrophe.

1.65 "**FTE**" means [**] hours of work per annum, which number of hours will be pro-rated based on the number of days when used for periods of less than twelve (12) months, devoted to or in support

of activities hereunder that is carried out by one or more qualified scientific or technical employees (excluding Third Party contractors) of a Party or its Affiliates. Notwithstanding the foregoing, [**].

1.66 "**FTE Committee**" has the meaning set forth in Section 7.3.3(a).

1.67 "**FTE Costs**" means, for any period, the FTE Rate multiplied by the number of FTEs who perform a specified activity under this Agreement. FTEs will be pro-rated as necessary.

1.68 "**FTE Rate**" means $[**] per FTE through [**], and after such time, upon the request of a Party, the JFC will propose, and the JSC will agree on, such agreement not to be unreasonably withheld, conditioned or delayed, an updated FTE Rate for the relevant Calendar Year. The FTE Rate includes [**] (the "**Indirect Allocations**"). The FTE Rate will in no case include [**].

1.69 "**FTE Rate Dispute**" has the meaning set forth in Section 7.3.3.

1.70 "**FTE Report**" has the meaning set forth in Section 7.3.3(b).

1.71 "**Funding Failure**" has the meaning set forth in Section 3.3.7.

1.72 "**GAAP**" means United States generally accepted accounting principles, consistently applied.

1.73 "**GCP**" means good clinical practices, which are the then-current standards for Clinical Trials for pharmaceuticals, as set forth in the FD&C Act and associated regulations (including 21 C.F.R. Parts 50, 54, 56 and 312), ICH Guideline E6, or other Applicable Law, and such standards of good clinical practice as are required by the Regulatory Authorities of the European Union and other organizations and governmental authorities in countries for which the applicable Licensed Product is intended to be Developed.

1.74 "**GLP**" means the then-current good laboratory practice standards promulgated or endorsed by the FDA as defined in 21 C.F.R. Part 58, or the successor thereto, or comparable regulatory standards in jurisdictions outside of the United States as they may be updated from time to time.

1.75 "**GMP**" means the then-current Good Manufacturing Practices as specified in Applicable Law, including the United States Code of Federal Regulations, applicable ICH Guidelines, or equivalent laws, rules or regulations of an applicable Regulatory Authority at the time of manufacture.

1.76 "**Governmental Authority**" means any court, agency, department, authority or other instrumentality of any national, state, county, city or other political subdivision, including any relevant Regulatory Authority.

1.77 "**HEOR Costs**" has the meaning set forth in Exhibit B.

1.78 "**ICH"** means the International Council on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use.

1.79 "**Income Option**" has the meaning set forth in Section 3.1.

1.80 "**Income Option Period**" has the meaning set forth in Section 3.3.6.

1.81 "**Income Share Product**" has the meaning set forth in Section 3.3.1.

1.82 "**IND**" means any Investigational New Drug application (including any amendment or supplement thereto) filed with the FDA pursuant to Part 312 of Title 21 of the U.S. Code of Federal Regulations, including any amendments thereto or if applicable, a comparable application or submission filed with a Regulatory Authority outside the U.S. for the investigation of any product in any other country or group of countries (such as a Clinical Trial Application in the EU).

1.83 "**Indemnified Party**" has the meaning set forth in Section 12.1.3.

1.84 "**Indemnifying Party**" has the meaning set forth in Section 12.1.3.

1.85 "**Indication**" means a specific disease or medical condition in humans.

1.86 "**Indirect Allocations**" has the meaning set forth in Section 1.68.

1.87 "**Infringement**" has the meaning set forth in Section 10.5(a)(ii).

1.88 "**Infringement Action**" has the meaning set forth in Section 10.5(b)(ii).

1.89 "**Initial Balancing Report**" has the meaning set forth in Exhibit B.

1.90 "**Initiate**" means, with respect to any Clinical Trial, dosing of the first human subject in such Clinical Trial. Cognates of the word "Initiate" will have correlative meanings.

1.91 "**In-License Agreements**" means any in-license agreements pursuant to which Solid acquires rights to Licensed Technology owned by a Third Party, including the Existing In-License Agreements.

1.92 "**Institutions**" means [**] and [**].

1.93 "**Investor Agreement**" means that certain Investor Agreement entered into between Ultragenyx and Solid on the Effective Date.

1.94 "**JFC**" has the meaning set forth in Section 7.2.1.

1.95 "**Joint Know-How**" means all Know-How created, conceived, discovered or generated jointly by or on behalf of the Parties in the performance of activities under this Agreement.

1.96 "**Joint Patents**" means all Patents that Cover any Joint Know-How.

1.97 "**Joint Steering Committee**" or "**JSC**" has the meaning set forth in Section 7.1.1.

1.98 "**Joint Technology**" means the Joint Know-How and Joint Patents.

1.99 "**Know-How**" means all proprietary scientific information, data, results, pre-clinical and clinical protocols and data from studies and Clinical Trials, feedback from Regulatory Authorities, chemical structures, chemical sequences, materials, information, inventions, knowledge, know-how, formulas, trade secrets, techniques, methods, processes, procedures, technology, practices, knowledge and developments, whether or not patentable; provided that Know-How does not include Patents.

1.100 "**LCM Development**" has the meaning set forth in Exhibit B.

1.101 "**LCM Development Costs**" has the meaning set forth in Exhibit B.

1.102 "**Liability**" has the meaning set forth in Section 12.1.1(a).

1.103 "**Licensed Know-How**" means all Solid Background Know-How and Solid Foreground Know-How.

1.104 "**Licensed Patents**" means all Solid Background Patents and Solid Foreground Patents. The Licensed Patents include the Patents listed on Schedule 1.104.

1.105 "**Licensed Product**" means any AAV8 or another clade E AAV variant pharmaceutical product that expresses Solid's MD5 nNOS binding domain form of microdystrophin. As used herein, "**MD5**" refers to a specific engineered 5-repeat microdystrophin protein that contains, from N- to C-terminus, the N-terminal actin binding domain, Hinge region 1 (HI), spectrin-like repeats Rl, R16, R17, R23, and R24, Hinge region 4 (H4), and the C-terminal dystroglycan binding domain of the human full-length dystrophin protein. The protein sequence of MD5 and the related dystrophin minigene are described in US 10,479,821 & WO2016/115543.

1.106 "**Licensed Technology**" means the Licensed Patents and Licensed Know-How.

1.107 "**Major European Countries**" means [**].

1.108 "**Major Latin American Countries**" means [**].

1.109 "**Major Market Countries**" means [**].

1.110 "**Manufacture**" or "**Manufactured**" or "**Manufacturing**" means activities directed to making, having made, producing, manufacturing, processing, formulating, filling, finishing, packaging, labeling, quality control testing and quality assurance release, shipping or storage of a Licensed Product by or on behalf of a Selling Party.

1.111 "**Manufacturing Costs**" has the meaning set forth in Exhibit B.

1.112 "**Marketing Approval**" means, with respect to a product in a particular country or jurisdiction, all approvals (including approvals resulting from any priority review, breakthrough therapy designation, accelerated approval or fast track designation, application or submission), licenses, registrations or authorizations necessary for the Commercialization of such product in such country or jurisdiction, including, (a) with respect to the United States, approval of an Approval Application for such product by the FDA and with respect to the European Union, approval of an Approval Application for such product by the European Commission or the applicable Regulatory Authority in any particular country in the EU, (b) where applicable, Price Approval in such country or jurisdiction, (c) where applicable, pre- and post-approval marketing authorizations (including any prerequisite Manufacturing approval or authorization related thereto), and (d) where applicable, labeling approval.

1.113 "**MD5"** has the meaning set forth in Section 1.105.

1.114 "**Medical Affairs Costs**" has the meaning set forth in Exhibit B.

1.115 "**Milestone Events**" has the meaning set forth in Section 9.2.3.

1.116 "**Milestone Payments**" has the meaning set forth in Section 9.2.3.

1.117 "**Net Income**" **and** "**Net Losses**" has the meaning set forth in Exhibit B.

1.118 "**Net Sales**" means, with respect to the Licensed Products, the gross amount invoiced for all sales of such Licensed Products by Ultragenyx and its Affiliates and Sublicensees (each, a "**Selling Party**") to a Third Party (other than another Selling Party), less the following deductions actually incurred or paid or otherwise accrued, allowed, reserved or allocated in accordance with Ultragenyx's Accounting Standards:

1.118.1 normal and customary trade, cash and quantity discounts, allowances and credits for such Licensed Products;

1.118.2 fees paid, reserves, and allowances to distributors and discounts (including cash, quantity and patient program discounts), charge-back payments, rebates and similar payments granted to customers, wholesalers, distributors, buying groups, retailers, health care insurance carriers, pharmacy benefit management companies, health maintenance organizations, managed health care organizations or to federal, state and local governments, their agencies, and purchasers and reimbursers or to trade customers;

1.118.3 credits or allowances actually granted for price adjustments (including retroactive price adjustments), damaged goods, spoiled product, claims, recalls, rejections or returns of such Licensed Products, including such Licensed Products returned in connection with withdrawals;

1.118.4 freight out, postage, customs charges, shipping and insurance charges for delivery of such Licensed Products; and

1.118.5 taxes or duties levied on, absorbed or otherwise imposed on the sale of such Licensed Products, including value-added taxes, or other governmental charges otherwise imposed upon the billed amount, as adjusted for rebates and refunds, to the extent not paid by the Third Party, but excluding any income taxes.

The following will not give rise to Net Sales: sales and other transfer of Licensed Products between Selling Parties, any dispositions of any Licensed Products for pre-clinical or clinical testing required in connection with obtaining Marketing Approval of any Licensed Products, and any dispositions or use of any Licensed Products under compassionate use, patient assistance, named patient use, or other similar programs or studies where the Licensed Product is supplied without charge or below manufacturing cost. Net Sales will be calculated in accordance with Accounting Standards so as to arrive at "net sales" under the relevant Accounting Standard as reported by such Selling Party, as applicable, in such Person's financial statements.

In the event that a Licensed Product is sold in any country in the form of a Combination Product, Net Sales of such Combination Product will be adjusted by multiplying actual Net Sales of such Combination Product in such country calculated pursuant to the foregoing definition of "Net Sales" by the fraction A/(A+B), where A is the average net selling price in such country of the relevant Licensed Product if sold separately in such country and B is the average net selling price in such country of, as applicable, each product that contains the Other Items contained in such Combination Product if sold separately in such country; provided that the invoice price in a country for (A) each Licensed Product without the Other Items and (B) each product that contains solely the Other Items included in the Combination Product will, in each case ((A) and (B)), to the extent feasible be for a quantity comparable to that used in such Combination Product and of substantially the same class, purity and potency or functionality, as applicable. If either such Licensed Product or the relevant Other Item(s) is not sold separately (including in the case of the sale of a combination therapy that contains the Licensed Product but is not sold separately) in a particular country, then the adjustment to Net Sales will be determined by the Parties in good faith to reasonably reflect the fair market value of the contribution of such Licensed Product or product in such Combination Product to the total fair market value of such Combination Product.

In the case of pharmacy incentive programs, hospital performance incentive programs, chargebacks, disease management programs, similar programs or discounts on portfolio product offerings, all rebates, discounts and other forms of reimbursements will be allocated among products on the basis on which such rebates, discounts and other forms of reimbursements were actually granted or, if such basis cannot be determined, in accordance with Ultragenyx's, its Affiliates' or its or their Sublicensees' existing allocation method; provided that any such allocation will be done in accordance with Applicable Law, including any price reporting laws, rules and regulations and such allocation method will not disproportionately reduce Net Sales in favor of net sales of other products.

For the purposes of calculating Net Sales, all Net Sales will be converted into Dollars pursuant to Section 9.7.2.

Subject to the above, Net Sales will be calculated in accordance with the standard internal policies and procedures of Ultragenyx, its Affiliates or its or their Sublicensees, which will be in accordance with applicable Accounting Standards. Notwithstanding anything herein to the contrary, in the event that a Selling Party will receive payment for a Licensed Product on an installment basis, the Parties will apply the

pro-rata amount of the installment received to Net Sales with respect to such Licensed Product; provided that for calculations of Net Sales for purposes of Milestone Payments, Net Sales will be calculated in a manner consistent with Accounting Standards and as if such payment was made in a lump sum and not on an installment basis.

As used herein, "**Combination Product**" means any Licensed Product in combination with one or more Other Items either when (A) priced and sold in a single package containing such multiple products or (B) packaged separately but sold together for a single price or where a discount, rebate or other amount is provided in exchange for (or otherwise conditioned upon) the purchase of such Other Item, in each case ((A) and (B)), including all dosage forms, formulations, presentations, and package configurations. Drug delivery vehicles, adjuvants and excipients will not be deemed to be "active ingredients," except in the case where such delivery vehicle, adjuvant or excipient is recognized by the FDA as an active ingredient in accordance with 21 C.F.R. 210.3(b)(7). "**Other Item**" means any therapeutically active pharmaceutical ingredient other than the therapeutically active pharmaceutical ingredient(s) of the Licensed Product.

1.119 "**Non-Breaching Party**" means the Party that believes the other Party is in material breach of this Agreement.

1.120 "**Option Exercise Deadline**" has the meaning set forth in Section 3.3.1.

1.121 "**Option Product**" means a Licensed Product with respect to which Solid has exercised the Development Option or Income Option. For clarity, Development Share Products and Income Share Products are Option Products. If the corresponding Development Option Period or Income Option Period expires or terminates, such Option Product will no longer be an Option Product.

1.122 "**Option Territory**" means the United States and EU.

1.123 "**Option Territory Commercialization Activities**" has the meaning set forth in Section 4.2.1.

1.124 "**Option Territory Commercialization Budget**" has the meaning set forth in Section 4.2.1.

1.125 **"Option Territory Commercialization Plan**" has the meaning set forth in Section 4.2.1.

1.126 "**Option Territory Development Activities**" has the meaning set forth in Section 3.3.5(a).

1.127 "**Option Territory Development Budget**" on a Licensed Product-by-Licensed Product basis, the [**] budget of anticipated Option Territory Development Costs as further detailed in Section 3.3.5(a).

1.128 "**Option Territory Development Costs**" means, on an Option Product-by-Option Product basis, [**].

1.129 "**Option Territory Development Plan**" has the meaning set forth in Section 3.3.5(a).

1.130 "**Opt-Out**" has the meaning set forth in Section 3.3.8.

1.131 "**Other Items**" has the meaning set forth in Section 1.118.

1.132 "**Out-of-Pocket Costs**" means, with respect to a Party, costs and expenses paid by such Party or its Affiliates to Third Parties (or payable to Third Parties and accrued in accordance with Accounting Standards), other than employees of such Party or its Affiliates.

1.133 "**Overlapping Infringement Action**" has the meaning set forth in Section 10.5(b)(i).

1.134 "**Party**" or "**Parties**" has the meaning set forth in the Preamble.

1.135 "**Patent and Trademark Costs**" has the meaning set forth in Exhibit B.

1.136 "**Patent and Trademark Recoveries**" has the meaning set forth in Exhibit B.

1.137 "**Patent Challenge**" has the meaning set forth in Section 13.2.5.

1.138 "**Patents**" means the rights and interests in and to issued patents and pending patent applications in any country, jurisdiction or region (including inventor's certificates and utility models), including all provisionals, non-provisionals, substitutions, continuations, continuations-in-part, divisionals, renewals and all patents granted thereon, and all reissues, reexaminations, extensions, confirmations, revalidations, registrations and patents of addition thereof, including patent term extensions and supplementary protection certificates, international patent applications filed under the Patent Cooperation Treaty (PCT) and any foreign equivalents to any of the foregoing.

1.139 "**Patient Assistance Program Costs**" has the meaning set forth in Exhibit B.

1.140 "**Person**" means an individual, sole proprietorship, partnership, limited partnership, limited liability partnership, corporation, limited liability company, business trust, joint stock company, trust, incorporated association, joint venture or similar entity or organization, including a government or political subdivision or department or agency of a government.

1.141 "**Post-Marketing Clinical Trial**" means any Clinical Trial of a product initiated after receipt of Marketing Approval for such product in a country or territory, that is not required by the Regulatory Authority in such country or territory as a condition to or to maintain the Marketing Approval for such product in such country or territory.

1.142 "**Price Approval**" means, in any country where a Governmental Authority authorizes reimbursement for, or approves or determines pricing for, pharmaceutical products, receipt (or, if required to make such authorization, approval or determination effective, publication) of any government approval, agreement, determination or decision establishing such reimbursement authorization or price approval or determination.

1.143 "**Proceeding**" means any action, suit, litigation, arbitration, proceeding (including any civil, criminal, administrative, investigative or appellate proceeding), prosecution, contest, hearing, inquiry, inquest, audit, examination or investigation that is, has been or may in the future be commenced, brought, conducted or heard at law or in equity or before any Governmental Authority, excluding all administrative proceedings before any patent office.

1.144 "**Product Reversion**" has the meaning set forth in Section 9.4.

1.145 "**Prosecution and Maintenance**" or "**Prosecute and Maintain**" means, with regard to a Patent, the preparing, filing, prosecuting and maintenance of such Patent, as well as handling re-examinations and reissues with respect to such Patent, together with the conduct of interferences, derivation proceedings, pre- and post-grant opposition proceedings, post-grant patent proceedings (such as inter partes review and post grant review) and other similar proceedings with respect to the particular Patent. For clarification, "**Prosecution and Maintenance**" or "**Prosecute and Maintain**" will not include any enforcement actions taken with respect to a Patent.

1.146 "**[**]**" has the meaning set forth in [**].

1.147 "**[**]**" has the meaning set forth in [**].

1.148 "**Receiving Party**" has the meaning set forth in Section 14.1.

1.149 "**Registrational Study**" means a human clinical study that is intended to establish that a Licensed Product is safe and efficacious for its intended use in the target population, and to determine any warnings, precautions, and adverse reactions that are associated with such pharmaceutical product in the dosage range to be prescribed, which clinical study is a registration trial intended to support Marketing Approval for such Licensed Product.

1.150 "**Regulatory Authority**" means, with respect to a country in the Territory, any national (*e.g.*, the FDA), supra-national (*e.g.*, the European Commission, the Council of the European Union, or the EMA), regional, state or local regulatory agency, department, bureau, board, commission, council or other Governmental Authority that holds responsibility for development, commercialization or manufacturing of, or the granting of Marketing Approval for a pharmaceutical product in such country or region.

1.151 "**Regulatory Exclusivity**" means, with respect to a Licensed Product in a country, any data exclusivity rights, market exclusivity rights, or other exclusive right, other than a Patent, granted, conferred or afforded by any Regulatory Authority in such country or otherwise under Applicable Law with respect to such Licensed Product in such country, which either confers exclusive marketing rights with respect to a product or prevents another party or the applicable Regulatory Authority from using or otherwise relying on the data supporting the approval of the Marketing Approval for a product without the prior written authorization of the Marketing Approval holder, as applicable, such as new chemical entity exclusivity, exclusivity associated with new Clinical Trials necessary for approval of a change to the label (*e.g.*, new Indication or use), orphan drug exclusivity, non-patent-related pediatric exclusivity, or any other applicable marketing or data exclusivity, including any such periods under national implementations in the EU of Article 10 of Directive 2001/83/EC, Article 14(11) of Parliament and Council Regulation (EC) No

726/2004, Parliament and Council Regulation (EC) No 141/2000 on orphan medicines, Parliament and Council Regulation (EC) No 1901/2006 on medicinal products for pediatric use and all international equivalents.

1.152 "**Regulatory Expenses**" has the meaning set forth in Exhibit B.

1.153 "**Regulatory Filings**" means, with respect to a Licensed Product, collectively: (a) all INDs, Approval Applications, establishment license applications, Drug Master Files, applications for designation (including as an "Orphan Product(s)" under the Orphan Drug Act, for "Fast Track" status under Section 506 of the FD&C Act (21 U.S.C. § 356) or for a Special Protocol Assessment under Section 505(b)(4)(B) and (C) of the FD&C Act (21 U.S.C. § 355(b)(4)(B))) and all other similar filings (including counterparts of any of the foregoing in any country or region in the Territory); (b) any applications for Marketing Approval and other applications, filings, dossiers or similar documents submitted to a Regulatory Authority in any country for the purpose of obtaining Marketing Approval from that Regulatory Authority; (c) any Patent-related filings with any Regulatory Authority; (d) all supplements and amendments to any of the foregoing and submissions made related to any of the foregoing; (e) all documents referenced in the complete regulatory chronology for each Marketing Approval; (f) confirmed meeting requests and meeting minutes; (g) foreign equivalents of any of the foregoing; and (h) all data and other information contained in, and correspondence with any Regulatory Authority relating to, any of the foregoing.

1.154 "**Royalty Term**" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the period commencing on the date of the First Commercial Sale of such Licensed Product in such country and ending upon the latest of: (a) the tenth (10^{th}) anniversary of the First Commercial Sale of such Licensed Product in such country; (b) the date on which [**]; or (c) expiration of all forms of Regulatory Exclusivity in such country with respect to such Licensed Product.

1.155 "**Sales and Marketing Costs**" has the meaning set forth in Exhibit B.

1.156 "**Selling Party**" has the meaning set forth in Section 1.118.

1.157 "**Share Purchase Agreement**" means that certain Share Purchase Agreement entered into between Ultragenyx and Solid on the Effective Date.

1.158 "**Solid**" has the meaning set forth in the Preamble.

1.159 "**Solid Background Know-How**" means all Know-How that is Controlled by Solid or any of its Affiliates prior to the Effective Date or comes into Solid's Control during the Term of this Agreement independent of activities under this Agreement and is necessary or reasonably useful for the Exploitation of a Licensed Product.

1.160 "**Solid Background Patents**" means all Patents that are Controlled by Solid or any of its Affiliates and Cover Solid Background Know-How.

1.161 "**Solid Budget**" has the meaning set forth in Section 2.1.2.

1.162 "**Solid Development Plan**" has the meaning set forth in Section 2.1.2.

1.163 "**Solid Exercise Effective Date**" has the meaning set forth in Section 3.3.2.

1.164 "**Solid Exercise Notice**" has the meaning set forth in Section 3.3.1.

1.165 "**Solid Foreground Know-How**" means all Know-How that is created, conceived, discovered or generated solely by or on behalf of Solid or any of its Affiliates in the performance of activities under this Agreement.

1.166 "**Solid Foreground Patents**" means all Patents that are Controlled by Solid or any of its Affiliates and Cover Solid Foreground Know-How.

1.167 "**Solid Indemnified Party**" has the meaning set forth in Section 12.1.1(a)(ii).

1.168 "**Solid Option**" has the meaning set forth in Section 3.1.

1.169 "**Standard Cost**" has the meaning set forth in Exhibit B.

1.170 "**Subcommittee**" means any subcommittee formed by the JSC in accordance with Section 7.1.4.

1.171 "**Subcontractor**" means a consultant, subcontractor, contract researcher, contract manufacturer, academic researcher or other vendor engaged by a Party to conduct activities on behalf of such Party or its Affiliate under this Agreement.

1.172 "**Sublicense**" means, directly or indirectly, to sublicense, grant any other right with respect to, or agree not to assert, the rights granted to Ultragenyx hereunder. When used as a noun, "Sublicense" means any agreement to Sublicense.

1.173 "**Sublicense Revenues**" has the meaning set forth in Exhibit B.

1.174 "**Sublicensee**" means a Third Party to whom Ultragenyx (or a Sublicensee or Affiliate) sublicenses any of the rights granted to Ultragenyx hereunder during the Term.

1.175 "**Tax Authority**" has the meaning set forth in Section 9.9.3.

1.176 "**Term**" has the meaning set forth in Section 13.1.

1.177 "**Territory**" means worldwide.

1.178 "**Third Party**" means any Person other than Ultragenyx, Solid or their respective Affiliates.

1.179 "**Third Party Infringement Claim**" has the meaning set forth in Section 10.6.

1.180 "**Third Party Payments**" has the meaning set forth in Exhibit B.

1.181 "**Ultragenyx**" has the meaning set forth in the Preamble.

1.182 "**Ultragenyx Background Know-How**" means all Know-How that is Controlled by Ultragenyx prior to the Effective Date or comes into Ultragenyx's Control during the Term of this Agreement independent of activities under this Agreement.

1.183 "**Ultragenyx Background Patents**" means all Patents that are Controlled by Ultragenyx and Cover Ultragenyx Background Know-How.

1.184 "**Ultragenyx Foreground Know-How**" means all Know-How that is created, conceived, discovered or generated solely by or on behalf of Ultragenyx in the performance of activities under this Agreement.

1.185 "**Ultragenyx Foreground Patents**" means all Patents that are Controlled by Ultragenyx and Cover Ultragenyx Foreground Know-How.

1.186 "**Ultragenyx Indemnified Party**" has the meaning set forth in Section 12.1.2.

1.187 "**Ultragenyx Know-How**" means all the Ultragenyx Background Know-How and Ultragenyx Foreground Know-How.

1.188 "**Ultragenyx Patents**" means all the Ultragenyx Background Patents and Ultragenyx Foreground Patents.

1.189 "**Ultragenyx Technology**" means the Ultragenyx Background Know-How, Ultragenyx Background Patents, Ultragenyx Foreground Know-How and Ultragenyx Foreground Patents .

1.190 "**United States**" or "**U.S.**" means the United States of America and its territories, possessions and districts.

1.191 "**[**]**" means [**].

1.192 "**[**] Agreement**" means the [**] Agreement, dated [**], by and between Solid and [**].

1.193 "**[**]**" means [**].

1.194 "**[**] Agreement**" means the [**] Agreement, dated as of [**], by and between Solid and [**].

1.195 "**Valid Claim**" means a claim (a) of any issued, unexpired United States or foreign Patent, which has not, in the country of issuance, been irrevocably donated to the public, disclaimed, held invalid or unenforceable by a court of competent jurisdiction in an unappealed or unappealable decision, or (b) of any United States or foreign patent application, which has not, in the country in question, been finally

cancelled, withdrawn, or abandoned; provided that, for purposes of this clause (b), notwithstanding the foregoing, on a country-by-country basis, a patent application pending for more than [**] will not be considered to have any Valid Claim for purposes of this Agreement unless and until a patent that meets the criteria set forth in clause (a) above with respect to such application issues.

ARTICLE 2
DEVELOPMENT

2.1 **Development Activities**.

2.1.1 Subject to the terms and conditions of this Agreement, Ultragenyx (directly, or through its Affiliates, Sublicensees or contractors) will use Commercially Reasonable Efforts to Develop at least one (1) Licensed Product. Notwithstanding the foregoing, Ultragenyx will not be deemed to be in breach of its obligations under this Section 2.1 to the extent it is prevented from or delayed in using Commercially Reasonable Efforts to Develop a Licensed Product as a result of the acts or omissions of Solid. Without limiting the Parties' rights and obligations under Article 7, Ultragenyx will determine the Development activities to be conducted with respect to the Licensed Products, including the prioritization of the Development activities and allocation of resources among the Development activities.

2.1.2 Within [**] after the Effective Date, the JSC will agree upon a plan of Development activities to be conducted by Solid with respect to a Licensed Product and a budget therefor (each such budget, a "**Solid Budget**", and each such plan, inclusive of the corresponding budget, a "**Solid Development Plan**"). Each Solid Development Plan agreed upon will be reflected in the minutes of one or more meetings of the JSC. Upon agreement on a Solid Development Plan, Solid will conduct activities in accordance with such plan. By way of example, a Solid Development Plan may require that Solid conduct [**]. Either Party may propose amendments to a Solid Development Plan at any time. The JSC will review and discuss such proposed amendments and each such amendment to a Solid Development Plan, including to a Solid Budget therefor, will be effective only if approved by the JSC. In the event of an inconsistency between this Agreement and a Solid Development Plan, the terms of this Agreement will prevail.

2.2 **Development Records and Reporting**.

2.2.1 **Records**. Each Party will maintain complete and accurate records of all work conducted by or on behalf of such Party in furtherance of the Development of Licensed Products. Such records will be maintained in sufficient detail and in good scientific manner appropriate for patent and regulatory purposes and in accordance with Applicable Laws.

2.2.2 **Reporting**. Prior to the First Commercial Sale of the first Licensed Product, at each [**] meeting of the JSC, Ultragenyx will provide a written report or presentation describing in reasonable detail Ultragenyx's activities and progress related to the Development of Licensed Products. For so long as Solid is conducting any activities under any Solid Development Plan, at each [**] meeting of the JSC Solid will provide a written report or presentation describing in reasonable detail Solid's activities and progress under the relevant Solid Development Plan.

2.3 **Development Costs**.

2.3.1 On a Licensed Product-by-Licensed Product basis, unless and until Solid exercises the Development Option for a given Licensed Product, Ultragenyx will (a) be solely responsible for all costs and expenses incurred by Ultragenyx in connection with the Development of Licensed Products and (b) reimburse Solid for its FTE Costs and Out-Of-Pocket Costs actually incurred by Solid or its Affiliates for the Development activities conducted under a Solid Development Plan (without markup) in accordance with Section 9.6, subject to the limits set forth in the corresponding Solid Budget. In no event will Ultragenyx be obligated to make any payments to Solid in excess of the corresponding budget for Development activities approved by the JSC.

2.3.2 On a Licensed Product-by-Licensed Product basis, if Solid exercises the Development Option for a given Licensed Product, then during the applicable Development Option Period, (a) Exhibit A will apply with respect to the Option Territory Development Costs with respect to such Licensed Product and (b) Ultragenyx will be solely responsible for all costs and expenses other than such Option Territory Development Costs incurred in connection with the Development of Licensed Products the Field.

2.4 **Subcontracting**. Solid may utilize the services of Subcontractors to perform its activities under the Solid Development Plan, provided that (a) Solid requires that such Subcontractor perform its obligations in a manner consistent with the terms of this Agreement and (b) Solid will remain at all times fully liable for its responsibilities under this Agreement. Each agreement entered into between Solid and a Subcontractor pursuant to this Section 2.4 will (i) include confidentiality and non-use provisions that are substantially similar to those set forth in Article 14 (but of duration customary in confidentiality agreements entered into for a similar purpose) and (ii) [**]. In each agreement between Solid and a Subcontractor that relates solely to Licensed Products, Solid will use Commercially Reasonable Efforts to require that such agreement is freely assignable to Ultragenyx. Solid will be solely responsible for direction of and communications with any such Subcontractor.

ARTICLE 3
SOLID OPTION.

3.1 **Generally**. Subject to the remainder of this Article 3, on a Licensed Product-by-Licensed Product basis, Ultragenyx hereby grants to Solid an exclusive option, exercisable in Solid's sole discretion one (1) time per Licensed Product, to fund thirty percent (30%) of the Option Territory Development Costs for such Licensed Product (the "**Development Option**") and share the Net Income and Net Losses of Commercializing such Licensed Product in the Field in the Option Territory in accordance with the Cost/Income Share (the "**Income Option**" and, collectively with the Development Option, the "**Solid Option**"). The application of the terms of this Article 3 with respect to a given Licensed Product have no effect on another Licensed Product.

3.2 **Data Package**. Within [**] after the date of First Achievement of Clinical POC, Ultragenyx will provide to Solid a Data Package with respect to the relevant Licensed Product. Solid will use the Data Package for each applicable Licensed Product solely to determine whether to exercise the corresponding Development Option or Income Option with respect to such Licensed Product.

3.3 **Option Exercise**.

3.3.1 On a Licensed Product-by-Licensed Product basis, Solid will have the right, in its sole discretion, to exercise the (a) Development Option or (b) Development Option and Income Option, in each case ((a) and (b)), for a given Licensed Product by delivering to Ultragenyx written notice of exercise (each such notice, the "**Solid Exercise Notice**") within [**] after Solid's receipt of the Data Package with respect to such Licensed Product (the end of such [**] period, the "**Option Exercise Deadline**"). In the event Solid exercises the Development Option or the Development Option and Income Option for a given Licensed Product, Solid will include in the applicable Solid Exercise Notice (i) reasonable documentation demonstrating that [**], and (ii) a certification that Solid does not have a Competing Product as of the date of such Solid Exercise Notice. For clarity, on a Licensed Product-by-Licensed Product basis, (1) in no event may Solid exercise the Income Option without also exercising the corresponding Development Option, (2) Solid may exercise the Development Option without also exercising the corresponding Income Option, (3) if Solid or any of its Affiliates has a Competing Product at the time of option exercise with respect to a Licensed Product, Solid may exercise only the Development Option as to such Licensed Product and the Income Option as to such Licensed Product will be null and void; and if in the case of the foregoing clause (2) or this clause (3) Solid exercises the Development Option, the relevant Option Product will be a "**Development Share Product**" and (4) any Option Product that is not a Development Share Product will be an "**Income Share Product**". The Solid Exercise Notice will expressly state whether Solid is exercising the Development Option and the Income Option and whether Solid or any of its Affiliates has a Competing Product on the date of such notice. If, during the relevant Development Option Period or Income Option Period, Solid or any of its Affiliates acquires a Competing Product or Initiates clinical Development or commences Commercialization of a Competing Product, then (y) such Income Share Product will automatically become a Development Share Product as of the first day of the first Calendar Quarter immediately following the Calendar Quarter in which Solid or its Affiliate acquired such Competing Product or commenced such activities and (z) Solid will give Ultragenyx written notice of the same within [**] of commencing such activities. In no event will a Development Share Product become an Income Share Product.

3.3.2 On a Licensed Product-by-Licensed Product basis, if Solid provides a Solid Exercise Notice for a given Licensed Product in accordance with Section 3.3.1, then (a) Solid will have exercised the Development Option or Income Option, as applicable, with respect to such Licensed Product, and (b) the date of Ultragenyx's receipt of such Solid Exercise Notice will be the "**Solid Exercise Effective Date**" with respect to such Licensed Product.

3.3.3 On a Licensed Product-by-Licensed Product basis, if Solid fails to provide a Solid Exercise Notice in accordance with Section 3.3.1 with respect to a Licensed Product prior to the Option Exercise Deadline for such Licensed Product, the Development Option and Income Option will expire and be of no further force or effect with respect to such Licensed Product.

3.3.4 Notwithstanding anything herein to the contrary, on a Licensed Product-by-Licensed Product basis, if Solid exercises the Development Option, then Exhibit A will apply with respect to the Option Territory Development Costs with respect to such Option Product (the period of time commencing on the Solid Exercise Effective Date with respect to the corresponding Option Product and

ending upon the completion of the Option Territory Development Activities set forth in the applicable Option Territory Development Plan (the "**Development Option Period**")).

3.3.5 During the Development Option Period, on an Option Product-by-Option Product basis:

(a) By no later than [**] of each Calendar Year, Ultragenyx will submit to the JSC a plan for Development activities to be undertaken by or on behalf of Ultragenyx with respect to the relevant Option Product [**] (each such plan, a "**Option Territory Development Plan**" and the activities described therein, the "**Option Territory Development Activities**"); provided that Ultragenyx will submit a preliminary Option Territory Development Plan by [**] of each Calendar Year. Each Option Territory Development Plan will include an updated detailed Option Territory Development Budget for [**], and estimated Option Territory Development Costs for the subsequent [**]. The Option Territory Development Costs are for information and planning purposes only. If the activities under the Option Territory Development Plan and costs described in the Option Territory Development Budget for the [**] thereof exceed [**] percent ([**]%) of the Option Territory Development Budget for [**], each Party will continue to share such costs in accordance with Exhibit A, provided that [**]. Each Option Territory Development Budget, and each update thereto, will be prepared by Ultragenyx based on Ultragenyx's good faith estimation of the probable Option Territory Development Activities to be conducted during the relevant time period. Ultragenyx's obligations under this Section 3.3.5(a) will terminate on an Option Product-by-Option Product basis on the last day of the relevant Development Option Period.

(b) Either Party may, through its representatives on the JSC, propose amendments to any Option Territory Development Plan until such time as no further Development activities are occurring or expected to occur under such Option Territory Development Plan.

3.3.6 The terms set forth on Exhibit B (the "**Cost/Income Sharing Terms**") will apply on an Income Share Product-by-Income Share Product basis with respect to each Income Share Product. Pursuant to the Cost/Income Sharing Terms, Ultragenyx will be entitled to and bear seventy percent (70%) of Net Income and Net Losses and Solid will be entitled to and bear thirty percent (30%) of all Net Income and Net Losses incurred in each Calendar Quarter with respect to such Licensed Product being sold in the Option Territory by or on behalf of a Selling Party, pursuant to the terms of Exhibit B (the "**Cost/Income Share**"). On an Option Product-by-Option Product basis, the Cost/Income Share will commence on the Solid Exercise Effective Date with respect to the corresponding Income Option and immediately terminate on the earliest to occur of [**] (such period of time, the "**Income Option Period**"). If the Income Option Period for an Income Share Product terminates pursuant to clause (a) or (b) in the immediately preceding sentence, then the Royalty Term will apply with respect to such Licensed Product with such Income Share Product thereafter treated as a Development Share Product or a Licensed Product that is not an Option Product, respectively.

3.3.7 If Solid fails to pay undisputed amounts owed for Option Territory Development Costs or undisputed amounts owed under the Cost/Income Share within [**] after receiving notice of such

failure (each, a "**Funding Failure**"), then, subject to any applicable cure period set forth herein, Ultragenyx will be entitled, in its sole discretion, upon written notice to Solid to [**].

3.3.8 On an Option Product-by-Option Product basis and in Solid's sole discretion, following the Solid Exercise Effective Date with respect to an Option Product, Solid may elect to convert the Option Product to a Licensed Product such that Solid may receive milestones and royalties with respect thereto as if no Solid Option were exercised by Solid with respect to such Option Product and such conversion will be irreversible (each such election, an "**Opt-Out**"). The Opt-Out will require [**] of notice and will be effective on the first day of the Calendar Quarter following notice to Ultragenyx of such Opt-Out.

ARTICLE 4
COMMERCIALIZATION

4.1 **Commercialization Activities**. Subject to the terms and conditions of this Agreement, Ultragenyx (directly, or through its Affiliates, Sublicensees or contractors) will use Commercially Reasonable Efforts to Commercialize at least one (1) Licensed Product in each of the Major Market Countries [**] for which it has obtained or expects to obtain Marketing Approval for such Licensed Product, at its sole cost and expense or subject to the Cost/Income Sharing Terms, as applicable. Subject to the foregoing sentence, Ultragenyx will be solely responsible for and will have sole discretion with respect to, Commercializing the Licensed Products in the Field in the Territory, including development and implementation of any promotional or branding strategy and materials, handling all returns, recalls, order processing, booking sales, invoicing and collections, inventory and receivables, and price matters.

4.2 **Commercialization Plans**. During the Income Option Period, on an Income Share Product-by-Income Share Product basis:

4.2.1 By no later than [**] of each Calendar Year, Ultragenyx will submit to the JSC a plan for Commercialization activities to be undertaken by or on behalf of Ultragenyx with respect to the relevant Income Share Product in the Option Territory during the [**] (each such plan, a "**Option Territory Commercialization Plan**" and the activities described therein, the "**Option Territory Commercialization Activities**"); provided that Ultragenyx will submit a preliminary Option Territory Commercialization Plan by [**] of each Calendar Year. Each Option Territory Commercialization Plan will include a [**] budget of estimated costs for Option Territory Commercialization Activities (the "**Option Territory Commercialization Budget**") and, if requested by Solid in writing, [**]. If the activities under the Option Territory Commercialization Plan exceed the corresponding Option Territory Commercialization Budget, each Party will continue to share the costs therefor in accordance with Exhibit B; provided, however, that [**]. The Option Territory Commercialization Budget will be for information and planning purposes only. Each Option Territory Commercialization Budget, and each update thereto, will be prepared by Ultragenyx based on Ultragenyx's good faith estimation of the probable Option Territory Commercialization Activities to be conducted during the relevant time period. Ultragenyx's obligations under this Section 4.2.1 will terminate on the last day of the relevant Income Option Period.

4.2.2 Either Party may, through its representatives on the JSC, propose amendments to any Option Territory Commercialization Plan until such time as no further Commercialization activities are

occurring or expected to occur under such Option Territory Commercialization Plan. Notwithstanding the foregoing, Ultragenyx will have sole control over branding for each Licensed Product, including selecting trademarks therefor.

4.3 **First Commercial Sale**. Ultragenyx will provide Solid with written notice of the First Commercial Sale of each Licensed Product in the each of the Major Market Countries [**] within [**] after such event. Following receipt of a request from Solid with respect to the occurrence of the First Commercial Sale of a given Licensed Product in any country in the Territory other than the Major Market Countries [**], Ultragenyx will promptly (and in any event within [**] after receipt of Solid's written request) notify Solid of the countries in the Territory other than the Major Market Countries [**] in which the First Commercial Sale of such Licensed Product has occurred and the date of the First Commercial Sale in each applicable country.

ARTICLE 5
REGULATORY MATTERS

5.1 **Regulatory Activities**.

5.1.1 Subject to the terms and conditions of this Agreement, Ultragenyx (directly, or through its Affiliates, Sublicensees or contractors) will use Commercially Reasonable Efforts to obtain Marketing Approval of at least one (1) Licensed Product from the FDA in the U.S., from the European Commission in the EU [**], at its sole cost and expense or subject to the Cost/Income Sharing Terms, as applicable. Subject to the foregoing sentence, Ultragenyx will be solely responsible for and will have sole discretion with respect to, all regulatory matters with respect to Licensed Products. Ultragenyx will be the sole point of contact with Regulatory Authorities with respect to the Licensed Products.

5.1.2 Notwithstanding the foregoing, if Ultragenyx desires that Solid conduct Development activities in support of regulatory matters, the Parties will discuss the same through the JSC. The JSC and Solid will seek to agree upon a Solid Development Plan, or amendment to an existing Solid Development Plan, that will reflect such activities to be conducted by Solid and a Solid Budget in accordance with Section 2.1.2 (*mutatis mutandis*). By way of example, an amended Solid Development Plan may require that Solid conduct [**].

5.2 **Communications with Regulatory Authorities; Regulatory Filings**. Ultragenyx will conduct all interactions, communications and meetings with Regulatory Authorities with respect to activities of Ultragenyx, its Affiliates and Sublicensees with respect to the Licensed Products. Solely during the Development Option Period or Income Option Period for a particular Option Product, before Ultragenyx submits any briefing book or response to a request by a Regulatory Authority, in each case, with respect to an Option Product in the Option Territory with respect to which neither Solid nor any of its Affiliates has a Competing Product, Ultragenyx will provide Solid with a reasonable opportunity to review and comment on such briefing book or response and will consider in good faith Solid's reasonable and timely requests and suggestions regarding such briefing book or response; provided that Ultragenyx's obligations in this sentence will expire [**]. Ultragenyx, or its Affiliates or Sublicensees, will have the sole and exclusive right to file and hold all Regulatory Filings, and to apply for and maintain all Marketing Approvals for all Licensed Products in the name of Ultragenyx or any of its Affiliates or Sublicensees. Notwithstanding the

foregoing, if, following receipt of Regulatory Approval with respect to an Option Product with respect to which neither Solid nor any of its Affiliates has a Competing Product, Ultragenyx receives notice from any Regulatory Authority in the Option Territory related to any (i) new or modified post-marketing study commitments, (ii) the emergence of new safety signals (including a Drug Safety Communication by the FDA or equivalent of the foregoing by the European Commission), (iii) Dear Dr. Letter, (iv) product labeling changes (*e.g.*, the imposition of black box warning), or (v) new requirement for a Risk Evaluation and Mitigation Strategy (REMS) or changes to an existing REMS, Ultragenyx shall notify Solid and provide Solid with a reasonable opportunity to review and comment on Ultragenyx's response to such notice from the applicable Regulatory Authority and Ultragenyx will consider in good faith Solid's reasonable and timely requests and suggestions regarding such response.

ARTICLE 6
MANUFACTURING

6.1 **Manufacturing Activities**. Subject to the terms and conditions of this Agreement, Ultragenyx (directly, or through its Affiliates, Sublicensees or contractors) will be solely responsible for Manufacturing and supplying Licensed Products at its sole cost and expense or subject to Cost/Income Sharing Terms. Without limiting the foregoing sentence, Ultragenyx will be solely responsible for and will have sole discretion with respect to, all manufacturing matters related to Licensed Products, including supply chain matters and quality control matters.

ARTICLE 7
GOVERNANCE

7.1 **Joint Steering Committee**.

7.1.1 **Formation; Responsibilities**. Within [**] after the Effective Date, the Parties will establish a joint steering committee (the "**Joint Steering Committee**" or "**JSC**") to direct and oversee the Parties' activities under this Agreement. The JSC will hold its first meeting within [**] after the Effective Date. Without limiting the foregoing, the JSC will have the responsibility and authority to:

(a) encourage and facilitate ongoing communication and cooperation between the Parties with respect to the Exploitation of Licensed Products in the Field in the Territory;

(b) review, discuss and approve any Solid Development Plan and Solid Budget or proposed amendment thereto;

(c) review and approve each Option Territory Development Plan and Option Territory Development Budget;

(d) solely during the applicable Development Option Period, review and approve each Party's proposed updates and amendments to each Option Territory Development Plan;

(e) review and approve each Option Territory Commercialization Plan and Option Territory Commercialization Budget;

(f) solely during the applicable Income Option Period, review and approve each Party's proposed updates and amendments to each Option Territory Commercialization Plan;

(g) approve changes to the FTE Rate following discussion thereof by the JFC;

(h) review and discuss reports provided pursuant to Section 2.2.2;

(i) discuss and resolve any disputes related to the Exploitation of Licensed Products;

(j) discuss proposed grants of Sublicenses pursuant to Section 8.3.1;

(k) review and discuss any potential in-license or acquisition of Third Party Patents or Know How pursuant to Section 8.6;

(l) review, discuss and approve any decisions or disputes submitted by a Subcommittee to the JSC;

(m) establish Subcommittees; and

(n) perform such other functions as are set forth in this Agreement, or as the Parties may mutually agree in writing, except where in conflict with any provision of this Agreement.

7.1.2 **Membership; Meetings**. The JSC will be composed of an equal number of representatives appointed by each of Solid and Ultragenyx, such number to initially be [**]. Each Party will have collectively one vote, regardless of the number or representatives from each Party. The Parties may from time to time change the size of the JSC. Each Party may replace its JSC representatives at any time upon written notice to the other Party. The JSC will be co-chaired by one designated representative of each Party. The co-chairpersons will not have any greater authority than any other representative on the committee. Each Party may designate the same individual as a representative on more than one Committee, and such individual may be an employee or consultant of such Party or any of its Affiliates. Each Party will be responsible for all costs and expenses incurred by it in participating in the JSC and any Subcommittees. The JSC will hold meetings at such times as the JSC will determine, but in no event will such meetings of the JSC be held less frequently than once every [**], or with such other frequency as unanimously agreed by the Parties' JSC members. The JSC may meet in person or by audio or video conference as the Parties may mutually agree. Other representatives of the Parties, their Affiliates and Third Parties involved in the Exploitation of the Licensed Products may attend such meetings of the JSC as nonvoting observers. The JSC may upon agreement meet on an ad hoc basis between regularly scheduled meetings in order to address and resolve time-sensitive issues within its purview that may arise from time to time. No action taken at a meeting of the JSC will be effective unless a representative of each Party is present or participating. Neither Party will unreasonably withhold attendance of at least one representative of such Party at any meeting of a committee for which reasonable advance notice was provided. Each Party will be responsible for its own personnel and travel costs relating to its JSC members' participation in the JSC.

7.1.3 **Agenda; Minutes**. The Alliance Managers will be responsible for: (a) preparing JSC meeting agendas reasonably in advance of JSC meetings, which JSC meeting agendas will include all agenda items reasonably requested by any JSC member for inclusion therein; (b) sending invitations and a JSC meeting agenda along with appropriate information for such agenda to all members of the JSC at least [**] before the next scheduled meeting of the JSC; and (c) preparing and circulating draft minutes within a reasonable time after each meeting of the JSC setting forth, among other things, a description of the discussions at the meeting and a list of any actions, decisions, or determinations approved by the JSC. Unless agreed otherwise by the JSC, minutes of all JSC meetings will be deemed finalized [**] after the draft minutes have been circulated if there are no other edits or pending clarifications brought up by any JSC member, if not unanimously agreed by the JSC beforehand.

7.1.4 **Subcommittees of the JSC**. From time to time, the JSC may establish Subcommittees, as it deems necessary or advisable to further the purposes of this Agreement, including any responsibilities assigned to the JSC under this Agreement; provided, however, that (a) the JSC will not delegate its decision-making authority and (b) no Subcommittee will have any power to amend, modify or waive compliance with this Agreement. Each Subcommittee will consist of an equal number of representatives of each Party (unless otherwise agreed by the Parties) and meet with such frequency as the JSC determines is appropriate from time to time. The purpose, scope and procedures of any such Subcommittee will be mutually agreed by the Parties via the JSC. All decisions of each Subcommittee will be made by unanimous decision, with each Party's designated Subcommittee members having collectively one (1) vote in all decisions. If, with respect to a matter that is subject to a Subcommittee's decision-making authority, the Subcommittee cannot reach unanimity, the matter will be referred to the JSC for resolution.

7.2 **Joint Finance Committee**.

7.2.1 **Formation; Responsibilities**. Within [**] after the first Solid Exercise Effective Date (if any), the Parties will establish a joint finance committee (the "**JFC**") to coordinate the financial reporting by the Parties with respect to the funding of Option Territory Development Costs for Option Products and, if applicable, implementation of the Cost/Income Sharing Terms. Without limiting the foregoing, the JFC will have the responsibility and authority to:

(a) reconcile financial and accounting matters between the Parties;

(b) initiate and execute an effective and efficient revenue and cost sharing process (cross-charges);

(c) discuss proposed changes to the FTE Rate;

(d) cooperate to ensure that the Option Territory Commercialization Budget (including a template for converting gross amounts to net amounts) agreed to for a Calendar Year (or any other given period) can be interpreted for the purposes of both Parties' internal financial and audit reporting requirements, including each Party's fiscal year reporting;

(e) discuss matters related to the calculation, implementation and reporting for the Parties' sharing of Option Territory Development Costs, Allowable Expenses, Net Income or Net Losses; and

(f) perform such other functions as are set forth in this Agreement, or as the JSC may delegate to the JFC, except where in conflict with any provision of this Agreement.

7.2.2 **Membership; Meetings**. The JFC will be composed of an equal number of representatives appointed by each of Solid and Ultragenyx, such number to initially be [**]. Each Party will have collectively one vote, regardless of the number or representatives from each Party. The Parties may from time to time change the size of the JFC. Each Party may replace its JFC representatives at any time upon written notice to the other Party. The JFC will be co-chaired by one designated representative of each Party. The co-chairpersons will not have any greater authority than any other representative on the committee. Each Party may designate the same individual as a representative on more than one Committee, and such individual may be an employee or consultant of such Party or any of its Affiliates. Each Party will be responsible for all costs and expenses incurred by it in participating in the JFC. The JFC will hold meetings at such times as the JFC will determine, but in no event will such meetings of the JFC be held less frequently than once every [**] following its formation, or with such other frequency as unanimously agreed by the Parties' JFC members. The JFC may meet in person or by audio or video conference as the Parties may mutually agree. Other representatives of the Parties, their Affiliates and Third Parties involved in the financial matters contemplated by this Agreement may attend such meetings of the JFC as nonvoting observers. The JFC may upon agreement meet on an ad hoc basis between regularly scheduled meetings in order to address and resolve time-sensitive issues within its purview that may arise from time to time. No action taken at a meeting of the JFC will be effective unless a representative of each Party is present or participating. Neither Party will unreasonably withhold attendance of at least one representative of such Party at any meeting of a committee for which reasonable advance notice was provided. Each Party will be responsible for its own personnel and travel costs relating to its JFC members' participation in the JFC. The JFC will automatically disband following the expiration of all payment obligations with respect to each Income Share Product.

7.2.3 **Agenda; Minutes**. The Alliance Managers will be responsible for: (a) preparing JFC meeting agendas reasonably in advance of JFC meetings, which JFC meeting agendas will include all agenda items reasonably requested by any JFC member for inclusion therein; (b) sending invitations and a JFC meeting agenda along with appropriate information for such agenda to all members of the JFC at least [**] before the next scheduled meeting of the JFC; and (c) preparing and circulating draft minutes within a reasonable time after each meeting of the JFC setting forth, among other things, a description of the discussions at the meeting and a list of any actions, decisions, or determinations approved by the JFC. Unless agreed otherwise by the JFC, minutes of all JFC meetings will be deemed finalized [**] after the draft minutes have been circulated if there are no other edits or pending clarifications brought up by any JFC member, if not unanimously agreed by the JFC beforehand.

7.3 **Decision-Making; Limitations on JSC**.

7.3.1 With respect to decisions of all Committees other than the JSC, the representatives of each Party will have collectively one (1) vote on behalf of such Party. For each meeting of such

Committee, at least one (1) representative of each Party will constitute a quorum and each Party will use Commercially Reasonable Efforts to have its representative(s) participate in each Committee meeting. Action on any matter may be taken at a meeting, by teleconference, videoconference or by written agreement. The members of each such Committee will act in good faith to cooperate with one another and seek agreement and consensus with respect to issues to be decided by such Committee. If a Committee other than the JSC is unable to resolve a matter by consensus during a period of [**], then such matter(s) will be escalated to the JSC.

7.3.2 With respect to decisions of the JSC, the members of the JSC will act in good faith to cooperate with one another and seek agreement and consensus with respect to issues to be decided by the JSC. Decisions of the JSC will be made by unanimous vote, with each Party's designated JSC members having collectively one (1) vote in all decisions. The presence of at least one (1) JSC member representing each Party will constitute a quorum in order for decisions to be made. The JSC will have only such powers as are specifically delegated to it in this Article 7, and such powers will be subject to the terms and conditions set forth herein. Without limiting the generality of the foregoing, the JSC will have no power to amend this Agreement or take any action which (i) imposes a material performance obligation on a Party without such Party's consent or (ii), under the terms of this Agreement, requires the consent or agreement of either or both of the Parties, without having received such consent or agreement. Subject to the foregoing, in the event that the JSC is unable to reach a unanimous decision on a matter that is within its decision-making authority within [**] after it has met and attempted to reach such decision, then resolution of such matter will be escalated to the Parties' respective Chief Executive Officers, who will confer in good faith on the resolution of such matter for a [**] period promptly after being made aware of the failure to reach a unanimous decision on such matter. If such matter is not resolved within such [**] period by the Chief Executive Officers, then (A) Solid will have final decision-making authority on all matters relating to any Solid Development Plan and any Solid Budget, provided that in no event may Solid exercise such final decision-making authority to increase the scope of the Solid Development Plan or make corresponding increases to the Solid Budget if Ultragenyx opposes such increases, and (B) Ultragenyx will have final decision-making authority on all other matters relating to the Exploitation of Licensed Products in the Field in the Territory, including financial matters; provided that, in all cases ((A) and (B)), no decision by either Party may be in conflict with any of the terms of this Agreement and a decision by the Chief Executive Officers or a final decision by a Party in accordance with this sentence will be deemed a decision by the JSC; provided, further, that if any dispute related to the FTE Rate is unresolved after escalation to the Chief Executive Officers pursuant to this Section 7.3.2, such dispute will be resolved in accordance with Section 7.3.3. For all other disputes, a Party may institute dispute resolution procedures pursuant to Section 15.10. For the avoidance of doubt, any action of the JSC will be consistent with the terms of this Agreement.

7.3.3 Any dispute related to whether to change the FTE Rate or the appropriate amount of the revised FTE Rate (an "**FTE Rate Dispute**") will be resolved by the following procedure:

(a) Each Party will select one (1) individual who would qualify as an Expert and those two (2) individuals will select one (1) individual who would qualify as an Expert and who will be chairman of a committee of the three (3) Experts (the "**FTE Committee**"), each with a single vote. Any decision of the FTE Committee will be determined by majority vote of the three (3) Experts.

(b) Within [**] following the formation of the FTE Committee, each Party will provide the FTE Committee with (i) a proposal regarding the resolution of the FTE Rate Dispute, (ii) a written memorandum in support of its position and (iii) any documentary evidence it wishes to provide in support thereof (each, an "**FTE Report**") and the FTE Committee will promptly provide each Party's FTE Report to the other Party after it receives it from each Party.

(c) Within [**] after a Party submits its FTE Report, the other Party will have the right to respond thereto. The response and any material in support thereof will be provided to the FTE Committee and the other Party.

(d) The FTE Committee will have the right to meet with the Parties as necessary to inform the FTE Committee's determination and to perform independent research and analysis. Within [**] after the receipt by the FTE Committee of both Parties' responses (or expiration of the [**] period if any Party fails to submit a response), then the FTE Committee will deliver its decision regarding the FTE Rate Dispute in writing; provided that the FTE Committee will select one of the resolutions proposed by the Parties. The determination of the FTE Committee will be binding on the Parties. The Parties will share equally in the costs of the FTE Committee. The FTE Committee may not decide on issues outside the scope mandated under terms of this Agreement.

7.4 **Alliance Managers**. Promptly after the Effective Date, each Party will appoint an individual to act as the alliance manager for such Party (each, an "**Alliance Manager**") (who may be a member of the JSC or other Committee). Each Alliance Manager will be permitted to attend meetings of any Committee as a nonvoting observer (if not a member), subject to the confidentiality provisions of Article 14. The Alliance Managers will be the point of contact for the Parties regarding the activities contemplated by this Agreement and will facilitate communication regarding all activities hereunder. The Alliance Managers will lead the communications between the Parties and will be responsible for following up on decisions made by the Committees. The name and contact information for such Alliance Manager, as well as any replacement(s) chosen by Solid or Ultragenyx, in their sole discretion, from time to time, will be promptly provided to the other Party in writing in accordance with Section 15.4.

ARTICLE 8
LICENSE GRANTS; TECHNOLOGY TRANSFER

8.1 **License Grant to Ultragenyx**.

8.1.1 Subject to the terms of this Agreement, Solid hereby grants to Ultragenyx and its Affiliates an exclusive (even as to Solid), royalty-bearing (sub)license, including the right to grant Sublicenses in accordance with Section 8.3, under the Licensed Technology and Joint Technology to Exploit Licensed Products in the Field in the Territory. Such license grant to Ultragenyx and its Affiliates includes the right to use Subcontractors. For the avoidance of doubt, the foregoing license grant to Ultragenyx does not include the right under the Licensed Technology to use, and Ultragenyx shall not Exploit, vectors that co-express Solid's MD5 nNOS binding domain form of microdystrophin and one or more additional coding sequences selected from [**]. Solid retains all rights under the Licensed Technology and Joint Technology to Exploit Licensed Products in the Field in the Territory to the extent necessary for Solid to perform its obligations under this Agreement.

8.1.2 **Existing In-License Agreements**.

(a) Ultragenyx acknowledges and agrees that the rights, licenses and sublicenses granted by Solid to Ultragenyx in this Agreement (including any rights to sublicense) are subject to the terms of the Existing In-License Agreements and the rights granted to the Institutions thereunder, the scope of the licenses granted to Solid and its Affiliates thereunder and the rights retained by such Institutions.

(b) Ultragenyx hereby acknowledges that, as set forth in the [**] Agreement, [**] reserves the right to make, use or otherwise practice the Licensed Patents owned by [**] for non-commercial research purposes and to grant to non-profit, academic, educational, or governmental institutions a nonexclusive, royalty-free license and right to make, use or otherwise practice such Licensed Patents for non-commercial research purposes (as defined in the [**] Agreement). Ultragenyx further hereby acknowledges that [**] also reserves the right to transfer tangible research materials and intangible materials incorporating the Licensed Patents owned by [**] to other non-profit, academic, educational, or governmental institutions for such non-commercial research purposes (as defined in the [**] Agreement). Ultragenyx hereby agrees that, notwithstanding any other provision of this Agreement, Ultragenyx has no right to enforce such Licensed Patents owned by [**] against [**] or any nonprofit, academic, educational, or governmental institution with respect to such use or practice for non-commercial research purposes (as defined in the [**] Agreement). Ultragenyx understands that the Licensed Patents owned [**] were developed under a funding agreement with the U.S. government and that the U.S. government may have certain rights relative thereto. Thus, notwithstanding anything hereunder, any and all licenses and other rights granted hereunder are limited by and subject to the rights and requirements of the U.S. government which may arise out of its sponsorship of the research which led to the conception or reduction to practice of the Licensed Patents owned by [**]. The U.S. government is entitled, as a right, under the provisions of 35 U.S.C. §§ 200-212 and applicable regulations of Title 37 of the Code of Federal Regulations: (i) to a nonexclusive, nontransferable, irrevocable, paid-up license to practice or have practiced for or on the behalf of the U.S. government any of such Licensed Patents owned by [**] throughout the world and (ii) to exercise march in rights on Licensed Patents. This Agreement is explicitly made subject to the U.S. government's rights under such U.S. government funding agreement and any applicable law or regulation. If there is a conflict between the U.S. government funding agreement, applicable law or regulation and this Agreement, the terms of the U.S. government funding agreement, applicable law or regulation shall prevail. Ultragenyx agrees to take any actions necessary to enable [**] to satisfy its obligations with the U.S. government relating to the Licensed Patents owned by [**]. Ultragenyx agrees, during the period of exclusivity of its license under the Licensed Patents owned by [**] in the United States, that any Licensed Product covered by a Valid Claim of the Licensed Patents owned by [**] that is produced for sale in the United States will be manufactured substantially in the United States as required by 35 U.S.C. § 204.

(c) Ultragenyx hereby acknowledges that as set forth in the [**] Agreement, [**] reserved all rights not expressly granted to Solid under the [**] Agreement. Ultragenyx further hereby acknowledges that [**] (i) retained for itself an irrevocable, nonexclusive license to make, have made, and use products, processes, and other subject matter covered by the Licensed Patents owned by [**] in the Field of Use (as defied therein) for academic research, medical, instructional, or any other academic

purpose, (ii) retained the right to require Solid to grant sublicenses to responsible applicants in the Field of Use (as defined therein) under the Licensed Patents owned by [**] on terms that are reasonable under the circumstances; or, if Solid fails to grant a license, to grant the license itself, subject to the limitations set forth in the [**] Agreement.

8.2 **License Grant to Solid**. Subject to the terms and conditions of this Agreement, Ultragenyx hereby grants to Solid a non-exclusive license (without the right to sublicense) under the Ultragenyx Technology solely to perform Solid's obligations under this Agreement.

8.3 **Sublicensing**.

8.3.1 Prior to granting a Sublicense of the license granted in Section 8.1, Ultragenyx will inform Solid of its intent to grant a Sublicense, the Parties will discuss the same through the JSC and Ultragenyx will reasonably consider Solid's input with respect to the grant of any such Sublicense.

8.3.2 Each such Sublicense will be consistent with, the terms of this Agreement and any applicable In-License Agreement and will require such Sublicensee to comply with all applicable terms of this Agreement and any applicable In-License Agreement. Ultragenyx will remain responsible for the performance of its Sublicensees. Within [**] after entering into a Sublicense with a Sublicensee, Ultragenyx will provide Solid with a copy of such Sublicense (which copy may be redacted to remove provisions which are not necessary to monitor compliance with this Section 8.3). For the avoidance of doubt, Ultragenyx will have no obligation to provide Solid with any copy of any Subcontractor agreement; provided, however, that each Subcontractor agreement will contain a requirement that the Subcontractor comply with commercially reasonable obligations of confidentiality and non-use provisions with respect to Solid's Confidential Information. Each Sublicense will contain the following provisions: [**].

8.3.3 Notwithstanding the foregoing, unless and until the receipt by Solid of a written agreement from an Institution to permit further sublicensing (as applicable) with respect to the Licensed Patents that are the subject of the relevant Existing In-License Agreement, Ultragenyx shall not have the right to grant any Sublicense (other than to Affiliates of such Party and other than as may be agreed in writing by the applicable Institution(s), in each case subject to all restrictions on the granting of Sublicenses herein) under such Licensed Patents. In the event and to the extent that an agreement from an Institution permitting further sublicensing to a Third Party is not obtained, then, upon Ultragenyx's request, Solid shall promptly grant, without further consideration, a direct license to the applicable Third Party as Ultragenyx directs, as and to the extent permitted under Solid's obligations to the applicable Institution, and provided that such direct license is within the scope of Ultragenyx's licenses granted under Section 8.1, and provided, further, [**]. Solid will keep Ultragenyx informed regarding the negotiation of a license agreement under this Section 8.3.3 and will consider in good faith Ultragenyx's reasonable and timely requests and suggestions regarding such agreement. Solid will provide to Ultragenyx an unredacted copy of each license agreement entered into pursuant to this Section 8.3.3 within [**] following the execution thereof.

8.4 **No Implied Licenses**. Except as expressly provided in this Agreement, no Party will be deemed by estoppel, implication or otherwise to have granted the other Party any licenses or other right with respect to any intellectual property.

8.5 **Bankruptcy**. All rights and licenses granted under or pursuant to this Agreement to Ultragenyx are and will otherwise be deemed to be, for purposes of Section 365(n) of the Bankruptcy Code, licenses of rights to "intellectual property" as defined under Section 101 of the Bankruptcy Code. The Parties agree that Ultragenyx and its Affiliates and Sublicensees will retain and may fully exercise all of its rights and elections under the Bankruptcy Code and any foreign equivalent thereto. The Parties further agree that if (a) a bankruptcy proceeding by or against Solid or its Affiliate (the "**Bankrupt Party**") is commenced under the Bankruptcy Code, (b) this Agreement is rejected as provided in the Bankruptcy Code, and (c) Ultragenyx or its Affiliate elects to retain its rights hereunder as provided in Section 365(n) of the Bankruptcy Code, Ultragenyx will be entitled to a complete duplicate of, and complete access to (as Ultragenyx deems appropriate), all such intellectual property and all embodiments of such intellectual property. Upon such occurrence, such intellectual property and all embodiments of such intellectual property will be promptly delivered to Ultragenyx. The Bankrupt Party (in any capacity, including debtor-in-possession) and its successors and assigns (including any trustee) agrees not to interfere with the exercise by Ultragenyx of its rights and licenses to such intellectual property and such embodiments of intellectual property in accordance with this Agreement, and agrees to assist Ultragenyx and its Affiliates in obtaining such intellectual property and such embodiments of intellectual property in the possession or control of Third Parties. The foregoing provisions are without prejudice to any rights Ultragenyx may have arising under the Bankruptcy Code or other Applicable Law. As used herein, "**Bankruptcy Code**" means Title 11, United States Code, as from time to time in effect.

8.6 **In-License Agreements**. On a Licensed Product-by-Licensed Product basis, a Party may notify the JSC that the Exploitation of Licensed Products may require or benefit from a grant of rights under additional Patents or Know-How of Third Parties, whether by license or acquisition. If a Party notifies the JSC pursuant to the foregoing sentence, the JSC will discuss obtaining a license to or acquiring such additional Patents or Know-How. In the event that Solid chooses to obtain such license or conduct such acquisition, then Solid will keep Ultragenyx informed of the progress and substance of the negotiations through the JSC and will consider in good faith any comments provided by Ultragenyx. If any such license obtained by Solid is exclusive, it must be sublicensable through multiple tiers, including to Ultragenyx, and include standby rights substantially similar to those set forth in Section 13.3.1(d) (*mutatis mutandis*). Promptly following the execution of any such license agreement or acquisition agreement, Solid will provide Ultragenyx through the JSC with a copy of the applicable contract with such Third Party. Within [**] following receipt of such contract, Ultragenyx will decide, in its sole discretion, whether or not to accept such Patents or Know-How as Licensed Technology licensed under this Agreement and provide Solid written notice of such decision. [**].

8.7 **Technology Transfer**. Promptly following the Effective Date, Solid will, and will cause its Affiliates and contractors to, reasonably cooperate with Ultragenyx to facilitate the technology transfer of the materials set forth on Schedule 8.7 for use by and on behalf of Ultragenyx in the Exploitation of Licensed Products. Such technology transfer may include providing Ultragenyx, and any designated contract researcher or manufacturer of Ultragenyx, with such assistance as may be necessary or useful to transfer and implement the processes, methods and techniques used by or on behalf of Solid in the practice of the Licensed Technology as of the Effective Date. Such cooperation will include providing Ultragenyx with reasonable access by teleconference or in-person at Solid's and Solid's Affiliates', and its and their contractors', facilities to appropriate personnel from Solid and its Affiliates, and its and their contractors, to provide Ultragenyx with a reasonable level of technical assistance and consultation in connection with

the transfer of Licensed Technology. Solid covenants to Ultragenyx that, to Solid's knowledge, all information and data transmitted to Ultragenyx pursuant to this Section 8.7 will be true, correct and complete in all material respects as of the date of transmission.

ARTICLE 9
FINANCIAL PROVISIONS

9.1 **Equity Investment**. On the Effective Date, the Parties will enter into the Share Purchase Agreement and Investor Agreement.

9.2 **Milestone Payments**.

9.2.1 **Development Milestones**. On a Licensed Product-by-Licensed Product basis, Ultragenyx will pay to Solid the milestone payments set forth in this Section 9.2.1 (each a "**Development Milestone Payment**") upon the achievement of the relevant milestone event (each a "**Development Milestone Event**") with respect to a Licensed Product that is not an Option Product, subject to the limitations set forth in this Section 9.2.1:

Milestone Number	Development Milestone Event	Development Milestone Payment
1	[**]	[**]
2	[**]	[**]

On a Licensed Product-by-Licensed Product basis, (i) each of the Development Milestone Payments is payable only the first time the corresponding Development Milestone Event is achieved by a given Licensed Product that is not an Option Product and (ii) no Development Milestone Payment will be payable for subsequent or repeated achievements of the same Development Milestone Event with respect to the same Licensed Product. In no event will a Development Milestone Payment be payable with respect to achievement of a Development Milestone Event by an Option Product. In no event will the Development Milestone Payments with respect to any one Licensed Product exceed Twenty-Five Million Dollars ($25,000,000).

On a Licensed Product-by-Licensed Product basis, if a Licensed Product is not required to undergo the first Development Milestone Event prior to undergoing the second Development Milestone Event, the first Development Milestone Event will be deemed to have been achieved upon the achievement by such Licensed Product of the second Development Milestone Event. Payment for any such skipped first Development Milestone Event that is owed in accordance with the provisions of the foregoing sentence with respect to a given Licensed Product will be due concurrently with the payment for the second Development Milestone Event with respect to such Licensed Product.

Neither Development Milestone Payment will be due or payable with respect to the achievement of the corresponding Development Milestone Event by an Option Product anywhere in the Territory during the corresponding Development Option Period or Income Option Period even if a Product Reversion occurs

with respect to such Option Product. For the avoidance of doubt, if a Product Reversion occurs with respect to an Option Product, then the Development Milestone Payments will be due or payable with respect to the achievement of the corresponding Development Milestone Events by such Licensed Product solely if any such Development Milestone Event is achieved with respect to a Licensed Product following the date on which such Licensed Product ceases to be an Option Product.

9.2.2 **Approval Milestones**. On a Licensed Product-by-Licensed Product basis, Ultragenyx will pay to Solid the milestone payments set forth in this Section 9.2.2 (each an "**Approval Milestone Payment**") upon the achievement of the relevant milestone event (each an "**Approval Milestone Event**") with respect to a Licensed Product that is not an Option Product, subject to the limitations set forth in this Section 9.2.2:

Milestone Number	**Approval Milestone Event**	**Approval Milestone Payment**
1	[**]	[**]
2	[**]	[**]
3	[**]	[**]

On a Licensed Product-by-Licensed Product basis, except with respect to Option Products, each of the Approval Milestone Payments is payable only the first time the corresponding Approval Milestone Event is achieved by a Licensed Product and no Approval Milestone Payment will be payable for subsequent or repeated achievements of the same Approval Milestone Event with respect to the same Licensed Product. In no event will the Approval Milestone Payments with respect to any one Licensed Product exceed Sixty-Five Million Dollars ($65,000,000).

No Approval Milestone Payment will be due or payable with respect to the achievement of the corresponding Approval Milestone Event by an Option Product anywhere in the Territory during the corresponding Development Option Period or Income Option Period even if a Product Reversion occurs with respect to such Option Product. For the avoidance of doubt, if a Product Reversion occurs with respect to an Option Product, then the Approval Milestone Payments will be due or payable with respect to the achievement of the corresponding Approval Milestone Events by such Licensed Product solely if any such Approval Milestone Event is achieved with respect to a Licensed Product following the date on which such Licensed Product ceases to be an Option Product.

9.2.3 **Commercial Milestones**. On a Licensed Product-by-Licensed Product basis, except with respect to Income Share Products, Ultragenyx will pay Solid the milestone payments set forth in this Section 9.2.3 (each a "**Commercial Milestone Payment**", and, together with the Development Milestone Payments and Approval Milestone Payments, the "**Milestone Payments**") upon the first achievement of the relevant milestone event (each, a "**Commercial Milestone Event**", and, together with the Development Milestone Events and Approval Milestone Events, the "**Milestone Events**") with respect to a particular Licensed Product in a Calendar Year, subject to the limitations set forth in this Section 9.2.3.

Milestone Number	**Commercial Milestone Event**	**Commercial Milestone Payment**

1	First occurrence of Calendar Year Net Sales in the Territory for a particular Licensed Product equal to or in excess of $[**]	[**]
2	First occurrence of Calendar Year Net Sales in the Territory for a particular Licensed Product equal to or in excess of $[**]	[**]
3	First occurrence of Calendar Year Net Sales in the Territory for a particular Licensed Product equal to or in excess of $[**]	[**]
4	First occurrence of Calendar Year Net Sales in the Territory for a particular Licensed Product equal to or in excess of $[**]	[**]

Each of the Commercial Milestone Payments is payable only the first time the corresponding Commercial Milestone Event is achieved with respect to a particular Licensed Product that is not an Income Share Product and no such Commercial Milestone Payment will be payable for subsequent or repeated achievements of the same Commercial Milestone Event with respect to the same Licensed Product. In no event will a Commercial Milestone Payment be due with respect to an Income Share Product during the corresponding Development Option Period or Income Option Period even if a Product Reversion occurs with respect to an Option Product. In no event will the Commercial Milestone Payments associated with a particular Licensed Product exceed One Hundred Sixty-Five Million Dollars ($165,000,000).

On a Licensed Product-by-Licensed Product basis (but excluding Income Share Products), the Commercial Milestone Payments in Section 9.2.3 are additive, such that if more than one Commercial Milestone Event specified in Section 9.2.3 is achieved in the same Calendar Year with respect to a particular Licensed Product, then each corresponding Commercial Milestone Payment for each such event will be payable.

9.2.4 **Notice; Payment**. Each Milestone Payment will be deemed earned upon achievement of the corresponding Milestone Event, and Ultragenyx will provide Solid with written notice and payment following the achievement of each of the Milestone Events set forth in Sections 9.2.1, 9.2.2, and 9.2.3, such written notice and payment to be provided (a) with respect to any Milestone Event under Section 9.2.1, or 9.2.2 , within [**] after such achievement and (b) with respect to any Milestone Event under Section 9.2.3, on or prior to the date of delivery of the royalty report under Section 9.3.7 for the Calendar Quarter in which such Milestone Event is first achieved.

9.3 **Royalties**.

9.3.1 **Royalty Rates**. Subject to the remainder of this Section 9.3, on a Licensed Product-by-Licensed Product and country-by-country basis, Ultragenyx will pay Solid royalties based on the aggregate Net Sales of the applicable Licensed Product sold by a Selling Party during a Calendar Year at the rates set forth in the applicable table below; provided that in no event will royalties be due with respect to Net Sales of an Income Share Product in the Option Territory even if a Product Reversion occurs with respect to such Income Share Product. The obligation to pay royalties will be imposed only once with respect to the same unit of a Licensed Product.

Calendar Year Net Sales (in U.S. Dollars) for such Licensed Product	**Royalty Rates as a Percentage (%) of Net Sales with respect to** **a Licensed Product that is not an Option Product**	**Royalty Rates as a Percentage (%) of Net Sales with respect to a Development Share Product**
Portion of Calendar Year Net Sales up to and including $[**]	[**]%	[**]%
Portion of Calendar Year Net Sales that exceeds $[**] up to and including $[**]	[**]%	[**]%
Portion of Calendar Year Net Sales that exceeds $[**]	[**]%	[**]%

Calendar Year Net Sales (in U.S. Dollars) for such Licensed Product that is an Income Share Product outside the Option Territory	**Royalty Rates as a Percentage (%) of Net Sales with respect to an Income Share Product**
Portion of Calendar Year Net Sales up to and including $[**]	[**]%
Portion of Calendar Year Net Sales that exceeds $[**] up to and including $[**]	[**]%
Portion of Calendar Year Net Sales that exceeds $[**]	[**]%

On a Licensed Product-by-Licensed Product basis, Net Sales of an Income Share Product in the Option Territory will not be counted for purposes of determining which royalty tier applies to the Net Sales of such Income Share Product outside the Option Territory.

Further, the applicable royalty rate set forth in the applicable table above will apply only to that portion of the Net Sales of a given Licensed Product during a given Calendar Year that falls within the indicated range. By way of example and without limitation of this Section 9.3.1, if Calendar Year Net Sales by the Selling Parties of a given Licensed Product that is not an Option Product were [**] Dollars ($[**]) for a given Calendar Year, then the royalties payable with respect to such Calendar Year Net Sales for such Licensed Product for such Calendar Year, subject to adjustment as set forth in this Section 9.3.1, would be: [**].

9.3.2 **Royalty Term**. Ultragenyx will pay royalties to Solid under this Section 9.3 on a Licensed Product-by-Licensed Product, and a country-by-country basis during the Royalty Term for such Licensed Product in such country. Upon the expiration of the Royalty Term for a given Licensed Product in a given country, the license granted to Ultragenyx under Section 8.1 will become fully-paid, perpetual, irrevocable and royalty free with respect to such Licensed Product.

9.3.3 **Reduction for Lack of Patent Coverage**. If during any period within the applicable Royalty Term for a country, (a) no Valid Claim of any Licensed Patent exists that Covers the Exploitation of such Licensed Product in such country and all forms of Regulatory Exclusivity with respect to such Licensed Product in such country have expired, then the royalty rate applied to Net Sales of such Licensed Product in such country will be reduced by [**] percent ([**]%) for purposes of calculating the royalty owed under Section 9.3.1 for the remainder of the Royalty Term for such Licensed Product in such country, or (b) no Valid Claim of any Licensed Patent exists that Covers the Exploitation of such Licensed Product in such country but Regulatory Exclusivity has not expired with respect to such Licensed Product in such country, then the royalty rate applied to Net Sales of such Licensed Product in such country will be reduced by [**] percent ([**]%) for purposes of calculating the royalty owed under Section 9.3.1 until the sooner of clause (a) of this Section 9.3.3 applies or the remainder of the Royalty Term for such Licensed Product in such country.

9.3.4 **Third Party Licenses**. On a country-by-country basis, Ultragenyx may deduct from the royalties payable to Solid under Section 9.3 (a) subject to Section 9.3.4(b), [**] percent ([**]%) of any Blocking Third Party Intellectual Property Costs incurred in a given country as to a Licensed Product (other than Income Share Products in the Option Territory), [**], and paid by Ultragenyx prior to or during such Calendar Quarter; provided, however, that in no event will the royalties that would otherwise be payable to Solid with respect to Net Sales of Licensed Products, after any applicable reduction to such Net Sales under this Section 9.3.4(a) be reduced by more than [**] percent ([**]%) in any given [**] provided, that, subject to Section 9.3.5, Ultragenyx will be entitled [**].

9.3.5 **Limit on Deductions**. On a Licensed Product-by-Licensed Product basis, in no event will the cumulative effect of the adjustments in Section 9.3.3 and Section 9.3.4 reduce the royalties payable to Solid under Section 9.3.1 to less than [**] percent ([**]%) of the amounts that would otherwise have been payable under Section 9.3.1 with respect to the applicable Licensed Product in the applicable Calendar Quarter. For the avoidance of doubt, the limit set forth in this Section 9.3.5 does not apply to reductions pursuant to Section 9.3.4(b).

9.3.6 **Income Share Products**. Notwithstanding anything to the contrary in this Section 9.3, on an Income Share Product-by-Income Share Product basis, (a) the Parties will share costs and profits in the Option Territory with respect to such Income Share Product in accordance with the applicable Cost/Income Sharing Terms and (b) the terms of Sections 9.3.1 through Section 9.3.5 and 9.3.7 will apply to sales of such Income Share Product outside of the Option Territory.

9.3.7 **Royalty Reports**. Following the First Commercial Sale of a Licensed Product for which a royalty is due and continuing for the remainder of the Royalty Term for such Licensed Product, within [**] after the end of each Calendar Quarter, Ultragenyx will provide Solid a good faith estimate of royalties that will be due for such Calendar Quarter, and within [**] after the end of each Calendar Quarter, Ultragenyx will deliver a report to Solid specifying on a Licensed Product-by-Licensed Product basis: [**]. Ultragenyx's reporting obligations with respect to Net Sales of Income Share Products in the Option Territory are set forth in Exhibit B. All royalty payments due under Section 9.3 for each Calendar Quarter will be due and payable within [**] after the end of each Calendar Quarter. If requested by Solid in writing [**] and prior to receipt of the report for the final Calendar Quarter of such Calendar Year, Ultragenyx shall include in such report, on a Licensed Product-by-Licensed Product basis for each Licensed Product for

which a royalty was paid or payable in the U.S. or EU in such Calendar Year, the deductions (by deduction category) from gross amounts billed or invoiced taken in calculating Net Sales in the U.S. and EU.

9.4 **Option Product to Licensed Product; Income Share Product to Development Share Product**. In the event an Option Product reverts to a Licensed Product in accordance with the terms and conditions of this Agreement as if no Solid Option was exercised by Solid with respect to such Licensed Product or an Income Share Product becomes a Development Share Product in accordance with the terms and conditions of this Agreement (each, a "**Product Reversion**"), the applicable provisions of this Article 9 will apply with respect to such Licensed Product or Development Share Product as of the first day of the first Calendar Quarter following the Calendar Quarter in which such Licensed Product no longer qualifies as an Option Product or such Income Share Product no longer qualifies as an Income Share Product.

9.5 **Invoicing for Additional Amounts**. With respect to any amounts owed under this Agreement by one Party to the other Party for which no other invoicing or payment procedure is specified elsewhere in this Agreement, within [**] after the end of each Calendar Quarter, the applicable Party will provide an invoice, together with reasonable supporting documentation, to the other Party for such amounts owed. The owing Party will pay any undisputed amounts within [**] of receipt of the invoice, and any disputed amounts owed by the owing Party will be paid within [**] of resolution of the Dispute.

9.6 **Development Reimbursement**. Ultragenyx will reimburse Solid for its FTE Costs and Out-of-Pocket Costs actually incurred by Solid or its Affiliates for the Development activities conducted under Section 2.1.2 in accordance with the Solid Development Plan. Solid will provide a good faith estimate of any costs for which reimbursement is due under this Section 9.6 within [**] after each Calendar Quarter. Any payments to be made to Solid by Ultragenyx pursuant to this Section 9.6 will be made [**] pursuant to final invoices submitted by Solid to Ultragenyx within [**] following the end of the applicable Calendar Quarter for which such costs have been incurred and will not exceed any corresponding amounts set forth in the Solid Budget. Each such invoice will be accompanied by reasonable supporting documentation evidencing the expenses incurred for such Development activities (such expenses to be itemized) during such Calendar Quarter. Undisputed payments will be due within [**] following the end of the applicable Calendar Quarter.

9.7 **Payment Terms**.

9.7.1 **Currency; Payment Method**. All payments under this Agreement are expressed in U.S. Dollars and will be paid in U.S. Dollars, by wire transfer or Automated Clearing House (ACH) payment to an account designated by the Party receiving payment (which account such Party may update from time to time in writing).

9.7.2 **Exchange**. For any currency conversion from the currency of one country in which the Licensed Products are sold into U.S. Dollars (or another currency if applicable) required in determining the amount of Net Sales or any royalties or revenue share due hereunder, such conversion will be equal to the average exchange rate, over the applicable Calendar Quarter, calculated at the conversion rate as reported by OANDA (www.oanda.com), or an equivalent or similar resource as agreed by the Parties, on the last Business Day of the Calendar Quarter in which the applicable Net Sales were made.

9.8 **General Right to Reconcile Payments**. Each Party will have the right to offset any amount owed by the other Party to such Party under or in connection with this Agreement which obligation is not being contested by the other Party in good faith, including in connection with any Funding Failure, any other breach or any indemnification obligation of the other Party, against any payments owed by one Party to the other Party under this Agreement. Such offsets will be in addition to any other rights or remedies available under this Agreement and Applicable Law.

9.9 **Withholding Tax; Tax Treatment**.

9.9.1 In the event any withholding, value added or other tax (including any tax based on income to Solid as well as any penalties, interest and surcharges) is required to be withheld from payments by Ultragenyx or is required to be paid by a Party pursuant to this Agreement under Applicable Laws, notwithstanding anything to the contrary herein, (a) Ultragenyx will promptly notify Solid of such requirement and make such withholding or make such payment to the applicable Governmental Authority out of such payments by Ultragenyx pursuant to this Agreement, (b) any amounts so required to be withheld will be remitted by Ultragenyx on a timely basis to the appropriate Governmental Authority, (c) any amounts withheld, deducted or paid to any Governmental Authority pursuant to this Section 9.10 will be treated as paid to Solid for the purposes of this Agreement and (d) at Solid's request, Ultragenyx will provide Solid reasonable evidence of the remittance for the purposes of this Agreement; provided, however, that the Parties will reasonably cooperate to eliminate or minimize any such withholding taxes, including by providing any reasonable documentation to reduce or eliminate such withholding and making payments from entities domiciled in the United States.

9.9.2 To the extent permitted by Applicable Law, Ultragenyx and Solid agree to treat, for U.S. federal income and other applicable tax purposes: (a) the Milestone Payments as royalties, (b) the royalties paid pursuant to Section 9.3.1 as royalties, (c) payments relating to any Development Share Product or Income Share Product or otherwise attributable to the Solid Option as royalties and (d) this Agreement, including the exercise of the Solid Option, as not giving rise to a partnership between any of Ultragenyx, Solid and any of their Affiliates. Ultragenyx and Solid agree to file all applicable tax returns in a manner consistent with this Section 9.9.2, unless otherwise required by Applicable Law.

9.9.3 If the Internal Revenue Service (or any state or local tax authority, each a "**Tax Authority**") asserts that a partnership exists (a "tax partnership") or existed at any time in respect of this Agreement, the exercise of the Solid Option or otherwise, the following costs will be borne by the Parties in accordance with the Cost/Income Share: (a) any taxes (including any interest and penalties) imposed by the Tax Authority directly relating to the assertion that a tax partnership exists or has existed and that would not otherwise be levied in the absence of such tax partnership and (b) any professional fees relating to the characterization of a tax partnership, including but not limited to professional fees relating to the maintenance of capital accounts and the filing of tax returns. For the avoidance of doubt, this Section 9.9.3 will not govern any taxes that would be imposed in the absence of any tax partnership. If a Tax Authority asserts that a partnership exists or has existed at any time, Ultragenyx or its Affiliates will have the authority to conduct and undertake the defense of such tax audit or other tax controversy, except to the extent that such tax audit or other tax controversy could reasonably be expected to materially adversely impact Solid, in which case Ultragenyx and Solid will have joint authority regarding such tax audit or other tax controversy.

9.9.4 If a Tax Authority were to assert that this Agreement in any way gives rise to a tax partnership, Ultragenyx and Solid agree that Ultragenyx (or its designee) will be the "partnership representative" of such partnership within the meaning of Section 6223 of the Internal Revenue Code of 1986, as amended.

9.10 **Records; Audits**. Ultragenyx will keep and maintain accurate and complete records regarding (i) Net Sales during the [**] preceding Calendar Years, including deductions (by deduction category) from gross amounts billed or invoiced taken in calculating Net Sales, (ii) Option Territory Development Costs incurred by Ultragenyx during the [**] preceding Calendar Years in sufficient detail to confirm the accuracy of any payments required under this Agreement, and (iii) Net Income or Net Loss (including Ultragenyx's Allowable Expenses) during the [**] preceding Calendar Years. Solid will keep accurate and complete records regarding all FTE Costs and Out-of-Pocket Costs incurred in connection with the Development activities performed by or on behalf of Solid and Solid's Allowable Expenses in sufficient detail to confirm the accuracy of any payments required under this Agreement, covering the [**] preceding Calendar Years. Upon [**] prior written notice from the other Party (the "**Auditing Party**"), the Party required to maintain such records (as applicable, the "**Audited Party**") will permit an independent certified public accounting firm of internationally recognized standing, selected by the Auditing Party and reasonably acceptable to the Audited Party, to examine the relevant books and records of the Audited Party and its Affiliates, as may be reasonably necessary to verify the royalty reports submitted by Ultragenyx in accordance with Section 9.3.7, the Option Territory Development Costs reported by Ultragenyx, the Net Income or Net Loss calculation for Income Share Products, or the FTE Costs and Out-of-Pocket Costs reported by Solid in accordance with Section 9.6, as applicable. An examination by the Auditing Party under this Section 9.10 will occur not more than [**] and will be limited to the pertinent books and records for any Calendar Year ending not more than [**] before the date of the request. No records will be audited more than [**]. The accounting firm will be provided access to such books and records at the Audited Party's facility or facilities where such books and records are normally kept and such examination will be conducted during the Audited Party's normal business hours. The Audited Party may require the accounting firm to sign a customary non-disclosure agreement before providing the accounting firm access to its facilities or records. Upon completion of the audit, the accounting firm will provide both Parties a written report disclosing whether the reports, Option Territory Development Costs or calculation of Net Income or Net Loss submitted by Ultragenyx or the FTE Costs, Out-of-Pocket Costs and Allowable Expenses submitted by Solid, as applicable, are correct or incorrect and the specific details concerning any discrepancies. No other information will be provided to the Auditing Party. If the report or information submitted by the Audited Party results in an underpayment or overpayment, (a) the Party owing the underpaid or overpaid amount will promptly pay the amount of such underpayment to the other Party, and (b) any such overpayment will be creditable against future payments to the other Party hereunder. The costs and fees of any audit conducted by the Auditing Party under this Section 9.10 will be borne by the Auditing Party, unless such audit reveals an underpayment of amounts owed to the Auditing Party of more than [**] percent ([**]%) of the amount that was owed by the Audited Party with respect to the relevant period, in which case, the Audited Party will reimburse the Auditing Party for the commercially reasonable expense incurred by the Auditing Party in connection with the audit.

9.11 **Late Payment**. Any payment under this Agreement that is not paid on or before the date such payment is due will bear interest, to the extent permitted by Applicable Law, at a rate equal to [**].

ARTICLE 10
INTELLECTUAL PROPERTY

10.1 **Ownership; Assignment**.

10.1.1 **Ultragenyx Technology**. As between the Parties, Ultragenyx will own and retain all rights, title and interest in and to the Ultragenyx Technology.

10.1.2 **Licensed Technology**. As between the Parties, Solid will own and retain all rights, title and interest in and to the Licensed Technology, subject to any rights or licenses expressly granted by one Party to the other Party under this Agreement.

10.1.3 **Joint Technology**. As between the Parties, the Parties will jointly own, on an equal and undivided basis, any Joint Technology without any duty of accounting to the other Party, subject to any rights or licenses expressly granted by one Party to the other Party under this Agreement.

10.2 **Ownership**. For purposes of determining inventorship under this Section 10.1, inventorship will be determined in accordance with United States patent laws (regardless of where the applicable activities occurred).

10.3 **Prosecution and Maintenance of Patents**.

10.3.1 **Ultragenyx Patents**. Ultragenyx will control, be responsible for and have the sole right for (but not the obligation), at its own expense, all aspects of the Prosecution and Maintenance of the Ultragenyx Patents.

10.3.2 **Licensed Patents**.

(a) **Solid Background Patents**. Solid will have the first right, but not the obligation, at its own expense, to Prosecute and Maintain the Solid Background Patents. Subject to Section 10.3.2(f), Solid will keep Ultragenyx reasonably informed of all steps with regard to, and the status, of such Prosecution and Maintenance of the Solid Background Patents, including by providing to Ultragenyx the following within [**] following the sending or filing thereof by Solid or an Institution: (i) copies of all correspondence and material communications it sends to or receives from any patent office or agency relating to any such Solid Background Patent, (ii) a copy of applications as filed, together with notice of its filing date and serial number and (iii) copies of all notices received from an Institution related to the Prosecution or Maintenance of any Solid Background Patent.

(b) **Solid Foreground Patents**. Solid will have the first right, but not the obligation, at its own expense, to Prosecute and Maintain the Solid Foreground Patents. Subject to Section 10.3.2(f), Solid will keep Ultragenyx reasonably informed of all steps with regard to, and the status of, such Prosecution and Maintenance of the Solid Foreground Patents, including by providing Ultragenyx with (i) copies of all correspondence and material communications it sends to or receives from any patent office or agency relating to any such Solid Foreground Patent, (ii) a draft copy of all applications sufficiently in advance of filing to permit reasonable review and comment by Ultragenyx in accordance with the remainder

of this Section 10.3.2(b) and (iii) a copy of applications as filed, together with notice of its filing date and serial number. Before Solid submits any material filing, including a new patent application, or response to such patent authorities with respect to any of the Solid Foreground Patents, Solid will provide Ultragenyx with a reasonable opportunity to review and comment on such filing or response and will consider in good faith Ultragenyx's reasonable and timely requests and suggestions regarding the Prosecution and Maintenance of such Solid Foreground Patents.

(c) **Step-In Right**. If Solid elects not to continue to Prosecute or Maintain a Licensed Patent pursuant to Section 10.3.2(a) or Section 10.3.2(b), as applicable, then Solid will give Ultragenyx notice thereof within a reasonable period (but not less than [**]) prior to allowing such Licensed Patent to lapse or become abandoned or unenforceable or forfeiting any rights in such defense, and Ultragenyx will have the right (but not the obligation), at its own expense, to assume responsibility for directing the Prosecution and Maintenance of such Licensed Patents in the applicable country or region and paying any required fees to maintain such Licensed Patent in such country or region. Upon transfer of Solid's responsibility for directing the Prosecution and Maintenance of any of the Licensed Patents to Ultragenyx under this Section 10.3.2(c), Solid will promptly deliver to Ultragenyx copies of all necessary files related to the Licensed Patents with respect to which responsibility has been transferred and will take all actions and execute all documents reasonably necessary for Ultragenyx to assume such prosecution, maintenance or defense.

(d) **Cooperation**. Subject to Section 10.3.2(f), each Party will, and will cause its Affiliates to, reasonably cooperate, with the other Party with respect to the Prosecution and Maintenance of Licensed Patents pursuant to this Section 10.3.2, including with respect to obtaining patent term restoration, supplemental protection certificates or their equivalents.

(e) **Patent Term Extension**. Subject to the remainder of this Section 10.3.2(e) and Section 10.3.2(f), Ultragenyx will be solely responsible to make the determination as to whether to seek patent term extensions, including supplementary protection certificates and any other extensions that are now or become available in the future, regarding any Joint Patent or Licensed Patent that is not a Solid Background Patent with respect to any Licensed Product. Ultragenyx may seek patent term extensions, including supplementary protection certificates and any other extensions that are now or become available in the future, regarding (i) a Solid Background Patent with respect to any Licensed Product only with Solid's prior written consent and (ii) any Joint Patent or Licensed Patent that is not a Solid Background Patent at Ultragenyx's election. Solid shall reasonably consider, and in good faith, Ultragenyx's input in determining whether to grant such consent with respect to the patent term extension of a Solid Background Patent. In no event will Solid grant a Third Party a right to seek patent term extension, including a supplementary protection certificate or any other extension, with respect to any Joint Patent such that a Third Party would be able to seek patent term extension with respect to such Patent prior to Ultragenyx seeking such extension. Solid will, and will cause its Affiliates to, at Ultragenyx's request and sole cost, assist and cooperate with Ultragenyx in any such filing or application with respect to a Licensed Patent that is available for patent term extension and, in the case of a Solid Background Patent, for which Solid has provided consent.

(f) **Existing In-License Agreements**. Ultragenyx acknowledges that, pursuant to the Existing In-License Agreements, [**] and [**] control Prosecution and Maintenance of certain Licensed Patents, with Solid having certain rights to review. Ultragenyx acknowledges and agrees

that (i) the rights and obligations under this Section 10.3.2 are subject to the rights of Solid's licensors under the Existing In-License Agreements and (ii) Solid's obligations under this Agreement only apply to the extent of Solid's rights with respect to participation in Prosecuting and Maintaining the Licensed Patents under the Existing In-License Agreements.

10.3.3 **Joint Patents**.

(a) Ultragenyx will have the first right, but not the obligation, at its own expense, to Prosecute and Maintain the Joint Patents. Ultragenyx will keep Solid reasonably informed of all steps with regard to, and the status of, such Prosecution and Maintenance of the Joint Patents, including by providing Solid with (i) copies of all correspondence and material communications it sends to or receives from any patent office or agency relating to any such Joint Patent, (ii) a draft copy of all applications sufficiently in advance of filing to permit reasonable review and comment by Solid in accordance with the remainder of this Section 10.3.3 and (iii) a copy of applications as filed, together with notice of its filing date and serial number. Before Ultragenyx submits any material filing, including a new patent application, or response to such patent authorities with respect to any of the Joint Patents, Ultragenyx will provide Solid with a reasonable opportunity to review and comment on such filing or response and will consider in good faith Solid's reasonable and timely requests and suggestions regarding the Prosecution and Maintenance of such Joint Patents.

(b) **Step-In Right**. If Ultragenyx elects not to continue to Prosecute or Maintain a Joint Patent pursuant to Section 10.3.3(b), then Ultragenyx will give Solid notice thereof within a reasonable period (but not less than [**]) prior to allowing such Joint Patent to lapse or become abandoned or unenforceable or forfeiting any rights in such defense, and Solid will have the right (but not the obligation), at its own expense, to assume responsibility for directing the Prosecution and Maintenance of such Joint Patents in the applicable country or region and paying any required fees to maintain such Joint Patent in such country or region. Upon transfer of Ultragenyx's responsibility for directing the Prosecution and Maintenance of any of the Joint Patents to Solid under this Section 10.3.3(b), Ultragenyx will promptly deliver to Solid copies of all necessary files related to the Joint Patents with respect to which responsibility has been transferred and will take all actions and execute all documents reasonably necessary for Solid to assume such prosecution, maintenance or defense.

10.4 **Entity Status**. Promptly following the Effective Date, Solid will provide each counterparty to an Existing In-License Agreement written notice indicating that the entity status of the applicable Licensed Patents changed to "large entity" and will cooperate with each such counterparty to change such entity status with each relevant patent and trademark office in the Territory.

10.5 **Third Party Infringement**.

(a) **Notice**.

(i) Each Party will promptly notify the other in writing of any (A) apparent, threatened or actual infringement by a Third Party of any Licensed Patent or Joint Patent or (B) unauthorized use or misappropriation by a Third Party of any Licensed Know-How or Joint Know-How, in

each case ((A) and (B)), of which it becomes aware, and will provide the other Party with all evidence in such Party's possession or control concerning such infringement or unauthorized use or misappropriation.

(ii) Solid will promptly notify Ultragenyx in writing of any (A) apparent, threatened or actual infringement by a Third Party of any Ultragenyx Patent or (B) unauthorized use or misappropriation by a Third Party of any Ultragenyx Know-How, in each case ((A) and (B)), of which it becomes aware, and will provide Ultragenyx with all evidence in such Solid's possession or control supporting concerning such infringement or unauthorized use or misappropriation (each apparent, threatened, or actual infringement or unauthorized use or misappropriation described in Section 10.5(a)(i) or 10.5(a)(ii), an "**Infringement**").

(b) **First Right with Respect to Competitive Infringement**.

(i) As between the Parties, Solid will have the first right, but not the obligation, using counsel of its choosing and at its sole expense, to institute any cause of action, suit, demand or proceeding alleging Infringement solely of the Licensed Patents by a Third Party with respect to Competitive Infringement that is also competitive with a Solid product (an "**Overlapping Infringement Action**"). Ultragenyx will have the right, at its own expense, to be represented in any such Overlapping Infringement Action by counsel of its own choice. Solid will promptly notify and keep Ultragenyx apprised in writing in a timely manner of any such Overlapping Infringement Action and any material filings in such Overlapping Infringement Action and will consider timely comments received from Ultragenyx with respect to any material filings proposed to be made in such Overlapping Infringement Action; provided, that, if Solid fails to commence a suit to enforce the Licensed Patents against such Overlapping Infringement Action (or to settle or otherwise secure the abatement of such Overlapping Infringement Action) within (i) [**] after its receipt or delivery of notice under Section 10.5(a)(i) or (ii) [**] before the time limit, if any, set forth in the Applicable Laws for the filing of such actions in order to avoid any loss of rights, whichever comes first, or ceases to diligently pursue an Overlapping Infringement Action, Ultragenyx will have the right, subject to Section 10.5(f), but not the obligation, at its own expense to institute an Overlapping Infringement Action against the applicable Third Party infringer(s) with respect to such Competitive Infringement, and Solid will have the right, at its own expense, to be represented in any such action by counsel of its own choice.

(ii) Subject to Section 10.5(f), as between the Parties, Ultragenyx will have the first right, but not the obligation, using counsel of its choosing and at its sole expense, to institute any cause of action, suit, demand or proceeding alleging Infringement of the Licensed Patents or Joint Patents by a Third Party with respect to Competitive Infringement that is not an Overlapping Infringement Action (an "**Infringement Action**"). Solid will have the right, at its own expense, to be represented in any such Infringement Action by counsel of its own choice. Ultragenyx will promptly notify and keep Solid apprised in writing in a timely manner of any such Infringement Action and any material filings in such Infringement Action and will consider timely comments received from Solid with respect to any material filings proposed to be made in such Infringement Action; provided, that, if Ultragenyx fails to commence a suit to enforce the Licensed Patents or Joint Patents, as applicable, against such Infringement Action (or to settle or otherwise secure the abatement of such Infringement Action) within (i) [**] after its receipt or delivery of notice under Section 10.5(a)(i) or (ii) [**] before the time limit, if any, set forth in the Applicable Laws for the filing of such actions in order to avoid any loss of rights, whichever comes first, or ceases to

diligently pursue an Infringement Action, Solid will have the right, but not the obligation, at its own expense to institute an Infringement Action against the applicable Third Party infringer(s) with respect to such Competitive Infringement, and Ultragenyx will have the right, at its own expense, to be represented in any such action by counsel of its own choice provided that if Ultragenyx notifies Solid that it is electing in good faith not to institute any Infringement Action to enforce the Licensed Patents or Joint Patents against such Competitive Infringement for strategic reasons intended to maintain the commercial value of the relevant Licensed Patent or Joint Patent, as applicable, and any Licensed Product Covered thereby and there are otherwise no Solid programs subject to such Infringement Action, Solid will neither have the right to commence nor control any Proceeding to enforce the Licensed Patents or Joint Patents against such Competitive Infringement.

(iii) Any settlements, damages or monetary awards recovered by either Party pursuant to any Overlapping Infringement Action or Infringement Action, as applicable, will, after (a) reimbursing the Parties for their reasonable out-of-pocket expenses in making such recovery (which amounts will be allocated pro rata if insufficient to cover the totality of such expenses) and (b) paying the Institutions the amount of such recovery to which the Institutions are entitled pursuant to the Existing In-License Agreements, as applicable, be paid [**].

(c) **Non-Competitive Infringement**.

(i) Solid Sole Right with Respect to Solid Background Patents. As between the Parties, Solid will have the sole right, but not the obligation, using counsel of its choosing and at its sole expense, to institute an Infringement Action and otherwise to enforce the Solid Background Patents in matters that are not Competitive Infringement, and to retain all settlements, damages or monetary awards recovered in connection with any such Infringement Action with respect to the Solid Background Patents. Prior to Solid instituting an Infringement Action pursuant to this Section 10.5(c)(i), Solid will give Ultragenyx at least [**] written notice of the same and will reasonably consider comments from Ultragenyx with respect to the same. Further, if Solid has not already instituted an Infringement Action pursuant to this Section 10.5(c)(i) at least [**] before the time limit, if any, set forth in the Applicable Laws for instituting such an Infringement Action, Solid will endeavor to notify Ultragenyx in writing of such time limit and will reasonably consider comments from Ultragenyx with respect to instituting, or not instituting, any such Infringement Action.

(ii) Solid First Right with Respect to Solid Foreground Patents. As between the Parties, Solid will have the first right, but not the obligation, using counsel of its choosing and at its sole expense, to institute an Infringement Action and otherwise to enforce the Solid Foreground Patents in matters that are not Competitive Infringement. In the event Solid institutes an Infringement Action pursuant to this Section 10.5(c)(i)(i), Solid will promptly notify Ultragenyx and will reasonably consider comments from Ultragenyx with respect to any material filings proposed to be made by Solid or its Affiliates; provided, that, if Solid fails to commence a suit to enforce the Solid Foreground Patents against such Infringement Action (or to settle or otherwise secure the abatement of such Infringement Action) within (i) [**] after its receipt or delivery of notice under Section 10.5(a)(i) or (ii) [**] before the time limit, if any, set forth in the Applicable Laws for the filing of such actions in order to avoid any loss of rights, whichever comes first, or ceases to diligently pursue such an Infringement Action, Ultragenyx will have the right, subject to Section 10.5(f), but not the obligation, at its own expense to institute an

Infringement Action to enforce the Solid Foreground Patents against the applicable Third Party infringer(s), and Solid will have the right, at its own expense, to be represented in any such action by counsel of its own choice.

(d) **Cooperation**. In any action brought pursuant to Section 10.5(b) or Section 10.5(c), each Party will, and will cause its Affiliates to, reasonably cooperate with each other, in good faith, relative to the other Party's efforts to protect the Licensed Patents and will join such suit as a party, if requested by the other Party. Furthermore, the Party commencing any Infringement Action pursuant to Section 10.5(b) or Section 10.5(c) will consider in good faith all reasonable and timely comments from the other Party on any proposed arguments asserted or to be asserted in litigation related to the enforcement or defense of any such Licensed Patents in an Infringement Action. Neither Party will have the right to settle any patent infringement litigation with respect to any Licensed Patent under this Section 10.5(d) in a manner that diminishes the rights or interests of the other Party (including any settlement that disclaims, limits the scope of, admits the invalidity or unenforceability of any such Licensed Patent) without the consent of such other Party (which will not be unreasonably withheld, conditioned or delayed).

(e) **Ultragenyx Technology**. As between the Parties, Ultragenyx will have the sole right, but not the obligation, using counsel of its choosing and at its sole expense, to institute an action, suit, demand or proceeding alleging Infringement of the Ultragenyx Patents.

(f) **Existing In-License Agreements**. Ultragenyx acknowledges that, pursuant to the Existing In-License Agreements, the Institutions have certain rights with respect to enforcement of certain of the Licensed Patents, including with respect to recoveries in respect of enforcement of certain of the Licensed Patents. Ultragenyx acknowledges and agrees that (i) the rights and obligations under this Section 10.5 are subject to the rights of Solid's licensors under the Existing In-License Agreements and (ii) Solid's obligations under this Agreement only apply to the extent of Solid's rights with respect to participation in enforcing the Licensed Patents under the Existing In-License Agreements.

10.6 **Claimed Infringement**. Each Party will promptly notify the other Party if a Third Party brings any action, suit, demand or proceeding, including any declaratory judgment action, alleging Patent infringement by Solid or Ultragenyx or any of their respective Affiliates or any Sublicensee with respect to the Exploitation of any Licensed Product (a "**Third Party Infringement Claim**"). Ultragenyx will have the sole right, but not the obligation, to control the defense and response to any such Third Party Infringement Claim with respect to activities of Ultragenyx, its Affiliates or Sublicensees, at Ultragenyx's sole cost and expense. Solid will have the first right, but not the obligation, to control the defense and response to any such Third Party Infringement Claim with respect to activities of Solid or its Affiliates, at Solid's sole cost and expense. In the event that Solid does not exercise its right to control, defend and respond to a Third Party Infringement Claim under this Section 10.6, Ultragenyx may, at its sole cost and expense, defend and respond to any such Third Party Infringement Claim with respect to activities of Solid or its Affiliates. Upon the request of the Party controlling the response to the Third Party Infringement Claim, the other Party will reasonably cooperate with the controlling Party in the reasonable defense of such Third Party Infringement Claim. The other Party will have the right to consult with the controlling

Party concerning any Third Party Infringement Claim and to participate in and be represented by independent counsel in any associated litigation. The Party defending a Third Party Infringement Claim under this Section 10.6 will (a) consult with the other Party as to the strategy for the prosecution of such defense, (b) consider in good faith any comments from the other Party with respect thereto and (c) keep the other Party informed of any material steps taken and provide copies of all material documents filed, in connection with such defense. The Party controlling the defense against a Third Party Infringement Claim will have the right to settle such Third Party Infringement Claim on terms deemed reasonably appropriate by such Party, provided, that, (i) any such settlement that adversely affect the rights or interests of the other Party will be subject to the other Party's prior written consent, not to be unreasonably withheld, (ii) such settlement does not impose any restriction or obligation (including the payment of any monetary damages) on or admit fault of the other Party, (iii) does not affect the scope, ownership, validity, enforceability or enforcement of any Licensed Patents, (iv) does not include the grant of any license, covenant or other rights to any Third Party that would conflict with or reduce the scope of the subject matter included under the licenses granted to the other Party under this Agreement, and (v) releases the other Party and its Affiliates and sublicensees from liability with respect to the same Third Party Infringement Claims to the same extent as the settling Party (but, for clarity, need not release the other Party from any liability for an infringement claim relating to any product or activities outside of this Agreement).

10.7 **Common Interest**. All information exchanged between the Parties regarding the Prosecution and Maintenance, and enforcement and defense, of any Patents under this Article 10 will be deemed Confidential Information of the Disclosing Party. In addition, the Parties acknowledge and agree that, with regard to such Prosecution and Maintenance, the interests of the Parties as collaborators and licensor and licensee are to obtain the strongest patent protection possible, and as such, are aligned and are legal in nature. The Parties agree and acknowledge that they have not waived, and nothing in this Agreement constitutes a waiver of, any legal privilege concerning the Patents under this Article 10, including privilege under the common interest doctrine and similar or related doctrines. Notwithstanding anything to the contrary contained herein, to the extent a Party has a good faith belief that any information required to be disclosed by such Party to the other Party under this Article 10 is protected by attorney-client privilege or any other applicable legal privilege or immunity, such Party will not be required to disclose such information, and the Parties will in good faith cooperate to agree upon a procedure (including entering into a specific common interest agreement, disclosing such information on a "for counsel eyes only" basis or similar procedure) under which such information may be disclosed without waiving or breaching such privilege or immunity.

10.8 **Recordation**. The Parties agree to collaborate in good faith and sign a short form license agreement regarding the subject matter of this Agreement for recordation purposes. The Parties further agree that, notwithstanding the signing of such short form license agreement, this Agreement will remain in full force and effect and that in the event there is any inconsistencies between this Agreement and the short form license agreement, this Agreement will control.

10.9 **Settlement**. Notwithstanding anything to the contrary under this Article 10, neither Party may enter a settlement, consent judgment or other voluntary final disposition of a suit under this Article 10 that disclaims, limits the scope of, admits the invalidity or unenforceability of, or grants a license, covenant not to sue or similar immunity under, or take any other action in a manner that materially diminishes the rights or interest of a Patent or Know-How Controlled by the other Party or its Affiliates without first

obtaining the written consent of the Party that Controls the relevant Patent or Know-How, such consent not to be unreasonably withheld, delayed or conditioned.

10.10 **Other Infringement**.

10.10.1 **Patents Solely Owned by Ultragenyx**. Ultragenyx will retain all rights to pursue an infringement of any Patent solely owned by Ultragenyx, and Ultragenyx will retain all recoveries with respect thereto.

10.10.2 **Other Patents Owned by Solid**. Solid will retain all rights to pursue an infringement of any Patent solely owned by Solid that is not a Licensed Patent, and Solid will retain all recoveries with respect thereto.

10.11 **Joint Research Agreements**. The Parties intend that this Agreement is and will be understood to be a "joint research agreement" (as that term is defined in 35 U.S.C. § 100(h) and used in 35 U.S.C. § 102(c)), entered into for the purposes of Exploiting Licensed Products. The Parties will coordinate their activities with respect to any submissions, filings or other activities in support thereof.

10.12 **Marking**. Prior to the issuance of Licensed Patents, Ultragenyx agrees to mark Licensed Products (or their containers or labels) sold by Ultragenyx with the words "Patent Pending" and following the issuance of one or more of Licensed Patents, with the words "Patent No. " or in such a manner as to conform with the patent laws and practice of the country of manufacture, sale, or importation.

ARTICLE 11
REPRESENTATIONS AND WARRANTIES

11.1 **Representations and Warranties of Ultragenyx**. Ultragenyx hereby represents and warrants to Solid, as of the Effective Date, that:

(a) Ultragenyx is duly organized, validly existing and in good standing under the Applicable Laws of the jurisdiction of its incorporation or organization;

(b) Ultragenyx (i) has the requisite power and authority and the legal right to enter into this Agreement and to perform its obligations hereunder and (ii) has taken all requisite action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

(c) This Agreement has been duly executed and delivered on behalf of Ultragenyx, and constitutes a legal, valid and binding obligation, enforceable against Ultragenyx in accordance with the terms hereof, except to the extent that enforcement of the rights and remedies created hereby is subject to (i) bankruptcy, insolvency, reorganization, moratorium and other similar laws of general application affecting the rights and remedies of creditors, or (ii) laws governing specific performance, injunctive relief and other equitable remedies;

(d) The execution, delivery and performance of this Agreement by Ultragenyx will not constitute a default under or conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it is bound, or violate any Applicable Law of any governmental body or administrative or other agency having jurisdiction over Ultragenyx; and

(e) Ultragenyx has obtained all necessary consents, approvals and authorizations of all Governmental Authorities and other Persons or entities required to be obtained by it as of the Effective Date in connection with the execution and delivery of this Agreement.

11.2 **Representations and Warranties of Solid**. Solid hereby represents and warrants to Ultragenyx, as of the Effective Date, that:

(a) Solid is duly organized, validly existing and in good standing under the Applicable Laws of the jurisdiction of its incorporation or organization;

(b) Solid (i) has the requisite power and authority and the legal right to enter into this Agreement and to perform its obligations hereunder and (ii) has taken all requisite action on its part to authorize the execution and delivery of this Agreement and the performance of its obligations hereunder;

(c) This Agreement has been duly executed and delivered on behalf of Solid, and constitutes a legal, valid and binding obligation, enforceable against Solid in accordance with the terms hereof, except to the extent that enforcement of the rights and remedies created hereby is subject to (i) bankruptcy, insolvency, reorganization, moratorium and other similar laws of general application affecting the rights and remedies of creditors, or (ii) laws governing specific performance, injunctive relief and other equitable remedies;

(d) The execution, delivery and performance of this Agreement by Solid will not constitute a default under or conflict with any agreement, instrument or understanding, oral or written, to which it is a party or by which it is bound, or violate any Applicable Law of any governmental body or administrative or other agency having jurisdiction over Solid;

(e) Solid has obtained all necessary consents, approvals and authorizations of all Governmental Authorities and other Persons or entities required to be obtained by it as of the Effective Date in connection with the execution and delivery of this Agreement;

(f) (i) the in-license agreements set forth on Schedule 11.2(f) (solely in the form such agreements exist as of the Effective Date, the "**Existing In-License Agreements**") are all of the in-license agreements pursuant to which Solid acquired rights to Licensed Technology owned by a Third Party and all other Licensed Technology is owned solely by Solid or its Affiliate; (ii) a true, complete and correct copy of each Existing In-License Agreement has been provided or made available to Ultragenyx prior to the Effective Date and each Existing In-License Agreement is in full force and effect and has not been amended, modified or waived, except as otherwise disclosed to Ultragenyx in writing; (iii) all Licensed Technology owned, or, to the knowledge of Solid, in-licensed by Solid, is free and clear of any liens, charges and encumbrances; (iv) no license granted by any Third Party to Solid or its Affiliates, or by Solid

or its Affiliates to any Third Party, conflicts with the rights and licenses granted to Ultragenyx hereunder and (v) no written notice of breach, default, or termination has been received or given under any Existing In-License Agreement, and to the knowledge of Solid, there is no act or omission by Solid or its Affiliates that would provide a right to terminate any such agreement;

(g) Solid (i) has sufficient legal or beneficial title and ownership of, or sufficient license rights under, the Licensed Technology to grant the licenses thereto granted pursuant to this Agreement, including sublicense rights under the Existing In-License Agreements to the extent permitted by this Agreement, (ii) is entitled to grant all rights, options and licenses under the Licensed Technology that it purports to grant or that are anticipated to be granted to Ultragenyx under this Agreement and (iii) Solid has obtained all consents or waivers from the Institutions with respect to this Agreement that are required to enter into this Agreement;

(h) Schedule 1.104 sets forth a true, correct and complete list of all Licensed Patents owned by Solid as of the Effective Date and, to Solid's knowledge, the Licensed Patents licensed or sublicensed to Solid pursuant to the Existing In-License Agreements. Except as set forth on Schedule 1.104 each such Licensed Patent is owned solely by Solid. To the extent any Licensed Patent is not owned by Solid, to Solid's knowledge Schedule 1.104 identifies the licensor or sublicensor from which the Licensed Patent is licensed;

(i) All Licensed Patents that are owned by Solid have been Prosecuted and Maintained from the respective patent offices in accordance with Applicable Law. To Solid's knowledge, all Licensed Patents that are not owned by Solid or any of its Affiliates have been Prosecuted and Maintained from the respective patent offices in accordance with Applicable Law. Neither Solid nor any of its Affiliates has received any written claims, nor to Solid's knowledge, is there any ongoing claim, or threatened claim, investigation, demand, proceeding or other legal action by any Third Party or Regulatory Authority with respect to the Licensed Technology, including (i) challenging the scope, validity or enforceability of any issued Licensed Patents, (ii) asserting the misuse or non-infringement of any of the Licensed Technology, or (iii) challenging Solid's Control of any of the Licensed Technology;

(j) With respect to the Licensed Patents owned by Solid or any of its Affiliates that exist as of the Effective Date, (i) Solid or its Affiliate has obtained valid and enforceable assignments from the inventors of all inventorship rights relating to such Patents, and all such assignments of inventorship rights relating to such Patents have been properly executed and recorded in the relevant U.S. and foreign patent offices, (ii) to Solid's knowledge, no current officer, employee, agent, advisor, consultant or representative of Solid or any of its Affiliates is in violation of any term of any such assignment or other agreement with Solid or such Affiliate regarding the protection of any Licensed Technology, and (iii) to Solid's knowledge, no Person who claims to be an inventor of an invention claimed in a Licensed Patent is not identified as an inventor of such invention in the filed Patent documents for such Licensed Patent;

(k) (i) All employees of Solid or its Affiliates performing activities on behalf of Solid or its Affiliates with respect to any Licensed Technology are subject to a present obligation to assign to Solid or its Affiliate all right, title and interest in and to any inventions developed by them in the conduct of such activities, whether or not patentable; and (ii) all Subcontractors of Solid or its Affiliates

performing activities on behalf of Solid or its Affiliates with respect to any Licensed Technology are subject to a written contract that satisfies the requirements of Section 2.4;

(l) With respect to the Licensed Patents that exist as of the Effective Date that have not been abandoned in the ordinary course of patent prosecution and are owned by Solid or any of its Affiliates, (i) Solid and its Affiliates have complied with all applicable requirements of the applicable Governmental Authority (including any duties of candor), in connection with the Prosecution and Maintenance of such Licensed Patents, (ii) the pending applications included in Licensed Patents that are owned by Solid or any of its Affiliates are being diligently prosecuted in the respective patent offices in which Solid has chosen to file in accordance with Applicable Law, (iii) to Solid's knowledge and to the extent required by Applicable Law, Solid or its Affiliate has presented all relevant references, documents and information of which it and the inventors are aware to the relevant patent examiner at the relevant patent office, (iv) it has timely paid all filing, maintenance, annuity, and renewal and other fees payable with respect to any such Licensed Patents; and (v) such Licensed Patents remain in full force and effect;

(m) Solid and its Affiliates have taken commercially reasonable measures consistent with industry practices to protect the secrecy, confidentiality and value of all Licensed Know-How that exists as of the Effective Date that constitutes confidential information or trade secrets under Applicable Law (including requiring all employees, consultants and independent contractors to execute binding and enforceable agreements requiring all such employees, consultants and independent contractors to maintain the confidentiality of such Licensed Know-How) and to Solid's knowledge there have not been any breaches by any party to such confidentiality agreements;

(n) To Solid's knowledge, (i) all information and data related to the Licensed Patents that exists as of the Effective Date that is material to the Exploitation of Licensed Products has been included in the electronic data room made available to Ultragenyx by Solid reasonably in advance of the Effective Date, and (ii) such information contained in such data room is true, correct and complete (subject to any redactions to such information or data) in all material respects as of the date a corresponding request for information was made by Ultragenyx;

(o) Other than with respect to Licensed Technology subject to an Existing In-License Agreement, no inventions Covered by the Licensed Technology (i) were conceived, discovered, developed or otherwise made in connection with any research activities funded, in whole or in part, by the federal government of the United States or any agency thereof; (ii) are a "subject invention" as that term is described in 35 U.S.C. §201(e); (iii) are otherwise subject to the provisions of the Patent and Trademark Law Amendments Act of 1980, as amended, codified at 35 U.S.C. §§200-212, as amended, or any regulations promulgated pursuant thereto, including in 37 C.F.R. Part 401; or (iv) are the subject of any licenses, options or other rights of any Governmental Authority, within or outside the United States;

(p) There are no judgments or settlements against or owed by Solid or any of its Affiliates, or, to Solid's knowledge, pending or threatened claims or litigation, in each case, in

connection with the Licensed Technology that exists as of the Effective Date or relating to the transactions contemplated by this Agreement;

(q) To Solid's knowledge, the use, practice or application by Ultragenyx (or its Affiliates or Sublicensees) of Solid's MD5 nNOS binding domain form of microdystrophin in the form provided by Solid to Ultragenyx pursuant to the technology transfer provisions of this Agreement does not infringe any Valid Claim of any issued and unexpired Patents, or otherwise violate the intellectual property rights, of a Third Party;

(r) Neither Solid nor any of its Affiliates has misappropriated any trade secret or other Know-How of a Third Party in development of the Licensed Technology; and

(s) Neither Solid nor any of its Affiliates has employed, nor engaged directly or indirectly, in any capacity in connection with the Licensed Technology any Person who has either been debarred or disqualified by the FDA (or a similar foreign authority), is the subject to of a conviction described in Section 306 of the FD&C Act or is subject to any similar sanction.

11.3 **Ultragenyx Covenants**. Ultragenyx hereby covenants to Solid that, except as otherwise expressly permitted under this Agreement:

(a) Ultragenyx will, and will require its Affiliates and Subcontractors to comply with all Applicable Law in its and their conduct of activities pursuant to this Agreement, including where appropriate GMP, GCP and GLP (or similar standards);

(b) all employees of Ultragenyx or its Affiliates working under this Agreement will be under appropriate confidentiality provisions at least as protective as those contained in this Agreement (but of duration customary in agreements entered into for a similar purpose);

(c) where this Agreement refers to an action or obligation to be undertaken by Ultragenyx's Affiliates, Ultragenyx will cause such Affiliates to undertake such obligations or other actions, and Ultragenyx will be responsible and liable for any acts or omissions by its Affiliates;

(d) Ultragenyx will not, and will cause its Affiliates not to, engage directly or indirectly, in any capacity in connection with this Agreement any Person who has either been debarred or disqualified by the FDA (or a similar foreign authority), is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any similar sanction; and

(e) Ultragenyx will inform Solid in writing promptly if it learns that it or any of its Affiliates or Subcontractors engages or did engage directly or indirectly, in any capacity in connection with this Agreement any Person who is either debarred or disqualified by the FDA (or similar foreign authorities), is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any such similar sanction, or if any action, suit, claim, investigation or legal or administrative proceeding is pending or, to Ultragenyx's knowledge, is threatened, relating to the debarment, disqualification or conviction of Ultragenyx, any of its Affiliates or Subcontractors or any such Person engaged directly or indirectly in connection with this Agreement.

(f) Ultragenyx agrees to comply with all applicable federal, state, and local laws and regulations. In particular, Ultragenyx will comply with all applicable U.S. laws dealing with the export of any items under the Export Administration Act (EAA), including its Export Administration Regulations (EAR). Ultragenyx further understands that the U.S. export laws and regulations include (but are not limited to): (1) EAR item-specific requirements; (2) EAR ultimate destination-specific requirements; (3) EAR end user-specific requirements; (4) EAR end use-specific requirements; (5) Foreign Corrupt Practices Act; and (6) anti-boycott laws and regulations. Ultragenyx will comply with all then-current applicable export laws and regulations of the U.S. Government (and other applicable U.S. laws and regulations) pertaining to the Licensed Products (including any associated products, items, articles, computer software, media, services, and other information). Ultragenyx will not, directly or indirectly, export (including any deemed export), nor re-export (including any deemed re-export) the Licensed Product (including any associated products, items, articles, computer software, media, services, and other information) in violation of U.S. export laws and regulations or other applicable U.S. laws and regulations. Ultragenyx will include an appropriate provision in its agreements with its Sublicensees requiring that such parties comply with all then-current applicable U.S. export laws and regulations and other applicable U.S. laws and regulations. Ultragenyx's obligations to comply with U.S. export control laws and regulations are independent of and survive the termination of this Agreement.

11.4 **Solid Covenants**. Solid hereby covenants to Ultragenyx that, except as otherwise expressly permitted under this Agreement:

(a) Solid will, and will require its Affiliates and Subcontractors to, comply with all Applicable Law in its and their conduct of activities pursuant to this Agreement, including where appropriate GMP, GCP and GLP (or similar standards);

(b) all employees of Solid or its Affiliates or Subcontractors working under this Agreement will be under appropriate confidentiality provisions at least as protective as those contained in this Agreement (but of duration customary in confidentiality agreements entered into for a similar purpose);

(c) Solid will not, and will cause its Affiliates not to (i) license, sell, assign or otherwise transfer to any Person any Licensed Technology or Joint Technology (or agree to do any of the foregoing) or (ii) incur or permit to exist, with respect to any Licensed Technology or Joint Technology, any lien, encumbrance, charge, security interest, mortgage, liability, grant of license or option to Third Parties or other restriction (including in connection with any indebtedness), in each case, that would conflict with, limit, impair or restrict the rights and licenses (or sublicenses, as the case may be) granted to Ultragenyx hereunder; provided that nothing herein will restrict Solid from effectuating an assignment in accordance with Section 15.1 and, for the avoidance of doubt, nothing herein will restrict Solid or its Affiliates from Exploiting, or granting any Third Party the right to Exploit, (x) vectors that co-express Solid's MD5 nNOS binding domain form of microdystrophin and one or more additional coding sequences selected from [**] or (y) any product that is not a Licensed Product;

(d) Solid will not, during the Term, enter into any material agreements or contracts that would be inconsistent with Solid's obligations or conflict with, limit, impair or restrict Ultragenyx's rights under this Agreement;

(e) during the Term, (i) all employees of Solid or its Affiliates performing activities on behalf of Solid or its Affiliates will be subject to a present obligation to assign to Solid or its Affiliate all right, title and interest in and to any inventions developed by them in the conduct of such activities, whether or not patentable; and (ii) all Subcontractors of Solid or its Affiliates performing activities on behalf of Solid or its Affiliates will be subject to a written contract that satisfies the requirements of Section 2.4;

(f) where this Agreement refers to an action or obligation to be undertaken by Solid's Affiliates, Solid will cause such Affiliates to undertake such obligations or other actions, and Solid will be responsible and liable for any acts or omissions by its Affiliates;

(g) Solid will not, and will cause its Affiliates and Subcontractors not to, engage directly or indirectly, in any capacity in connection with this Agreement any Person who has either been debarred or disqualified by the FDA (or a similar foreign authority), is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any similar sanction;

(h) Solid will inform Ultragenyx in writing promptly if it learns that it or any of its Affiliates or Subcontractors engages or did engage directly or indirectly, in any capacity in connection with this Agreement any Person who is either debarred or disqualified by the FDA (or similar foreign authorities), is the subject of a conviction described in Section 306 of the FD&C Act or is subject to any such similar sanction, or if any action, suit, claim, investigation or legal or administrative proceeding is pending or, to Solid's knowledge, is threatened, relating to the debarment, disqualification or conviction of Solid, any of its Affiliates or Subcontractors or any such Person engaged directly or indirectly in connection with this Agreement; and

(i) Solid will enforce (including in connection with any counterparty's breach of any representations or warranties thereunder) Solid's rights and benefits and the obligations of the respective counterparties under each In-License Agreement, and will inform Ultragenyx of any action it may take under an In-License Agreement to the extent such action may conflict with or otherwise adversely affect, or would reasonably be expected to conflict or adversely affect, the rights or licenses granted to Ultragenyx hereunder; provided that, without limiting the foregoing, Solid will (i) fulfill all of its material obligations, including its payment obligations, under each In-License Agreement; (ii) not amend or waive or take any action or omit to taking any action that would alter, diminish or adversely affect any of Ultragenyx's rights under an In-License Agreement, including breach, be in default or terminate such agreement or increase any of Ultragenyx's obligations under such agreement; (iii) promptly, but no later than [**] after Solid learns of any material breach, default under, termination or amendment by any party of any In-License Agreement, notify Ultragenyx of such event and, to the extent not prohibited by an In-License Agreement, grant Ultragenyx the right (but not the obligation) to cure such event; and (iv) will use Commercially Reasonable Efforts to facilitate Ultragenyx entering into a side letter with each counterparty to each In-License Agreement regarding the applicable counterparty granting to Ultragenyx a "standby" license that will survive the termination of the applicable In-License Agreement; provided that Solid is not

required to pay any money or grant any concessions to any counterparty to an In-License Agreement in order to induce such counterparty to grant Ultragenyx the "standby" license described in this clause (iv).

11.5 **Disclaimer**. WITHOUT LIMITING THE RESPECTIVE RIGHTS AND OBLIGATIONS OF THE PARTIES EXPRESSLY SET FORTH HEREIN, EACH PARTY AND [**] SPECIFICALLY DISCLAIMS ANY GUARANTEE THAT THE ACTIVITIES CONDUCTED HEREUNDER OR ANY LICENSED PRODUCT WILL BE SUCCESSFUL, IN WHOLE OR IN PART. EXCEPT AS OTHERWISE EXPRESSLY PROVIDED IN THIS AGREEMENT, NEITHER PARTY NOR [**] MAKES ANY REPRESENTATIONS OR EXTENDS ANY WARRANTY OF ANY KIND, EITHER EXPRESS OR IMPLIED (AND EACH PARTY HEREBY EXPRESSLY DISCLAIMS ANY AND ALL REPRESENTATIONS AND WARRANTIES NOT EXPRESSLY PROVIDED IN THIS AGREEMENT), INCLUDING WITH RESPECT TO ANY PATENTS OR KNOW-HOW, OR MATERIALS, INCLUDING WARRANTIES OF VALIDITY OR ENFORCEABILITY OF ANY PATENTS, TITLE, QUALITY, COMPLETENESS, ACCURACY, MERCHANTABILITY, FITNESS FOR A PARTICULAR USE OR PURPOSE, PERFORMANCE, AND NONINFRINGEMENT OF ANY THIRD PARTY PATENTS OR OTHER INTELLECTUAL PROPERTY RIGHTS.

ARTICLE 12
INDEMNIFICATION; INSURANCE; LIMITATIONS

12.1 **Indemnification**.

12.1.1 **Indemnification by Ultragenyx**. Ultragenyx will indemnify, defend and hold harmless:

(a) Solid, its Affiliates, and its and its Affiliates' employees, officers, directors and agents and their respective successors, heirs and assigns (each, a "**Solid Indemnified Party**") from and against any liability, loss, damage or expense (including reasonable attorneys' fees and expenses) (collectively, "**Liability**") arising out of any Third Party suit, investigation, claim or demand in connection with:

(i) the Exploitation of any Licensed Product by, on behalf of, or under the authority of, Ultragenyx or any of its Affiliates (other than by a Solid Indemnified Party);

(ii) the breach by Ultragenyx of any of its representations, warranties or covenants set forth in this Agreement; or

(iii) the negligence, recklessness or willful misconduct of Ultragenyx or any Ultragenyx Indemnified Party in connection with the performance of its obligations hereunder;

except, in each case ((i)–(iii)), to the extent such claims fall within the scope of Solid's indemnification obligations under Section 12.1.2 (or would have had the Third Party claim been made against Ultragenyx under this Agreement) as to which Liability each Party will indemnify the other to the extent of their respective liability.

(b) [**] and its regents, employees, and agents harmless from all suits, actions, claims, liabilities, demands, damages, losses, or expenses (including reasonable attorneys' and investigative expenses), relating to or arising out of the manufacture, use, lease, sale, or other disposition of a Licensed Product to the extent Covered by Licensed Technology in-licensed by Solid pursuant to the [**] Agreement, including, without limitation, personal injury, property damage, breach of contract and warranty and products-liability claims relating to a Licensed Product to the extent Covered by Licensed Technology in-licensed by Solid pursuant to the [**] Agreement and claims brought by a Sublicensee.

(c) [**] its current or former curators, officers, employees and affiliates, harmless from any claim, proceeding, suit, demand, expense, loss, penalty, judgment, or liability of any kind whatsoever, including costs, expenses and reasonable attorneys' fees, resulting from, related to, arising out of, or in connection with (1) the design, development, production, manufacture, shipping, use, importation, sale, advertisement, labeling, promotion, or patent marking of a Licensed Product to the extent Covered by Licensed Technology in-licensed by Solid pursuant to the [**] Agreement, or end users, including but not limited to (i) any infringement or misappropriation of a patent, copyright, trade secret or other intellectual property or proprietary right of any third party or (ii) any product liability claims, such as those involving the death of or injury to any person or persons or damage to property; or (2) any breach of any obligation, covenant, representation, or warranty by Ultragenyx its Affiliates hereunder; or (3) the production, use or sale of any product, process or service identified, characterized or otherwise developed with the aid of the Licensed Patents in-licensed by Solid pursuant to the [**] Agreement by Ultragenyx or its Affiliates or Sublicensees; or (4) a breach or violation of applicable law by Ultragenyx its Affiliates or Sublicensees; or (5) the exercise of its Ultragenyx's or its Affiliates' rights under this Agreement.

12.1.2 **Indemnification by Solid**. Solid will indemnify, defend and hold harmless Ultragenyx, its Affiliates and its and its Affiliates' employees, officers, directors and agents and their respective successors, heirs and assigns (each, an "**Ultragenyx Indemnified Party**") from and against any Liability arising out of any Third Party suit, investigation, claim or demand in connection with:

(a) the Exploitation of any Licensed Product by, on behalf of, or under the authority of, Solid or any of its Affiliates (other than by a Ultragenyx Indemnified Party);

(b) the breach by Solid of any of its representations, warranties or covenants set forth in this Agreement; or

(c) the negligence, recklessness or willful misconduct of Solid or any Solid Indemnified Party in connection with the performance of its obligations hereunder;

except, in each case ((a)–(c)), to the extent such claims fall within the scope of Ultragenyx's indemnification obligations under Section 12.1.1 (or would have had the Third Party claim been made against Solid under this Agreement) as to which Liability each Party will indemnify the other to the extent of their respective liability.

12.1.3 **Procedure**. Each Party will notify the other Party in writing if it becomes aware of a claim for which such Party may seek indemnification hereunder. If any Proceeding (including any governmental investigation) is instituted against a Party with respect to which indemnity may be sought

pursuant to Section 12.1.1 or 12.1.2, as applicable, such Party (the "**Indemnified Party**") will give prompt written notice of the indemnity claim to the other Party (the "**Indemnifying Party**") and provide the Indemnifying Party with a copy of any complaint, summons or other written notice that the Indemnified Party receives in connection with any such claim. An Indemnified Party's failure to deliver such written notice will relieve the Indemnifying Party of liability to the Indemnified Party under Section 12.1.1 or 12.1.2, as applicable, only to the extent such delay is prejudicial to the Indemnifying Party's ability to defend such claim and allow the Indemnifying Party to assume the defense of claim. Provided that the Indemnifying Party is not contesting the indemnity obligation, the Indemnified Party will permit the Indemnifying Party to control any litigation relating to such claim and the disposition of such claim by negotiated settlement or otherwise (subject to this Section 12.1) and any failure to contest such obligation prior to assuming control will be deemed to be an admission of the obligation to indemnify. The Indemnifying Party will act reasonably and in good faith with respect to all matters relating to such claim and will not settle or otherwise resolve such claim without the Indemnified Party's prior written consent, which will not be unreasonably withheld, conditioned or delayed; provided that such consent will not be required with respect to any settlement involving only the payment of monetary awards for which the Indemnifying Party will be fully responsible. The Indemnified Party will cooperate with the Indemnifying Party in the Indemnifying Party's defense of any claim for which indemnity is sought under this Agreement, at the Indemnifying Party's cost and expense.

12.2 **Insurance**. Solid and Ultragenyx will respectively, at their own cost and expense, obtain and maintain commercially reasonable insurance coverage from insurance carriers licensed to do business under the laws of the country, state, commonwealth, province, or territory in which such Party's obligations are provided, with insurers that carry a rating of at least an A- VII or better from A.M. Best and as are consistent with the requirements of the In-License Agreements (to the extent the technology licensed under such In-License Agreement(s) is Licensed Technology hereunder). Such insurance maintained by Ultragenyx will name each Institution as an additional insured. Each Party will furnish to the other Party evidence of such insurance upon request.

12.3 **No Duplication of Remedies**. To the extent any Party may have more than one remedy for any Liabilities incurred by it, it may pursue all available remedies, but in no event will any Indemnified Party be entitled to duplicate compensation with respect to any claims for any breach of, or inaccuracy in, any representation or warranty made by the other Party in this Agreement, or any breach or violation of any covenant, obligation, or agreement in the performance of this Agreement.

12.4 **Limitation of Consequential Damages**. Except for (a) claims of a Third Party that are subject to indemnification under this Article 12, (b) claims arising out of a Party's fraud or willful misconduct or (c) a Party's breach of Article 14, neither Party nor any of its Affiliates will be liable to the other Party or its Affiliates for any incidental, special, punitive or other indirect damages or lost or imputed profits or royalties, lost data or cost of procurement of substitute goods or services, which are not probable and reasonably foreseeable, whether liability is asserted in contract, tort (including negligence and strict product liability), indemnity or contribution, and irrespective of whether that Party or any representative of that Party has been advised of, or otherwise might have anticipated the possibility of, any such loss or damage.

12.5 **Ultragenyx Release**. With respect to the Licensed Technology in-licensed by Solid pursuant to the [**] Agreement, for itself and its employees, Ultragenyx hereby releases [**] and its regents, employees, and agents forever from any suits, actions, claims, liabilities, demands, damages, losses, or expenses (including reasonable attorneys' and investigative expenses) relating to or arising out of (i) the manufacture, use, lease, sale, or other disposition of a Licensed Product; or (ii) the assigning or sublicensing of Ultragenyx's rights under this Agreement.

ARTICLE 13
TERM; TERMINATION

13.1 **Term; Expiration**. This Agreement is effective as of the Effective Date and, unless earlier terminated pursuant to the other provisions of this Article 13, will expire, on a country-by-country and Licensed Product-by-Licensed Product basis, on the date of expiration of all payment obligations under this Agreement with respect to such Licensed Product in such country (such period, the "**Term**"). Upon the expiration of this Agreement with respect to a Licensed Product in a given country, the rights and licenses granted to Ultragenyx pursuant to Section 8.1 will become perpetual, irrevocable, fully paid-up and royalty free with respect to such Licensed Product in such country.

13.2 **Termination of the Agreement**.

13.2.1 **Ultragenyx's Termination for Convenience**. Ultragenyx may terminate this Agreement for convenience by providing written notice of its intent to terminate to Solid, in which case, such termination will be effective ninety (90) days after Solid's receipt of such written notice. During such ninety (90) day notice period, if requested by Solid, the Chief Executive Officers will confer in good faith with respect to Ultragenyx's termination of this Agreement and whether Ultragenyx will grant to Solid an exclusive license to the Ultragenyx Technology and Joint Technology then used in the Exploitation of Licensed Products to Exploit Licensed Products in the Field in the Territory.

13.2.2 **Termination for Material Breach**.

(a) **Ultragenyx's Right to Terminate for Material Breach**. If Ultragenyx believes that Solid is in material breach of this Agreement, Ultragenyx may deliver written notice of such material breach to Solid. If the breach is curable, Solid will have [**] following its receipt of such written notice to cure such breach. If Solid fails to cure, or fails to dispute, such breach within such [**] period, or the breach is not subject to cure, Ultragenyx may terminate this Agreement by providing written notice to Solid, in which case, this Agreement will terminate in its entirety on the date on which Solid receives such written notice; provided, however, that if (i) the relevant breach is curable, but not reasonably curable within [**], and (ii) Solid is making a *bona fide* effort to cure such breach, Ultragenyx's right to terminate this Agreement on account of such breach will be suspended for so long as Solid is continuing to make such *bona fide* effort to cure such breach and if such breach is successfully cured, Ultragenyx will no longer have the right to terminate this Agreement on account of such breach.

(b) **Solid's Right to Terminate for Material Breach**. If Solid believes that Ultragenyx is in material breach of this Agreement, Solid may deliver written notice of such material breach to Ultragenyx. If the breach is curable, Ultragenyx will have [**] (or in the case of any undisputed payment

obligation, [**]) following its receipt of such written notice to cure such breach. If Ultragenyx fails to cure, or fails to dispute, such breach within such [**] period or [**] period, as applicable, or the breach is not subject to cure, Solid may terminate this Agreement by providing written notice to Ultragenyx, in which case, this Agreement will terminate on the date on which Ultragenyx receives such written notice; provided, however, that if (i) the relevant breach (A) does not involve Ultragenyx's failure to make a payment when due and (B) is curable, but not reasonably curable within [**], and (ii) Ultragenyx is making a *bona fide* effort to cure such breach, Solid's right to terminate this Agreement on account of such breach will be suspended for so long as Ultragenyx is continuing to make such *bona fide* effort to cure such breach (but in no event longer than [**]) and if such breach is successfully cured, Solid will no longer have the right to terminate this Agreement on account of such breach.

13.2.3 **Disputes Regarding Material Breach**. Notwithstanding the foregoing, if the Breaching Party in Section 13.2.2 disputes in good faith the existence, materiality, or failure to cure of any breach, and provides written notice to the Non-Breaching Party of such dispute within the relevant cure period, the Non-Breaching Party will not have the right to terminate this Agreement in accordance with Section 13.2.2 unless and until the relevant dispute has been resolved pursuant to the dispute resolution provisions in Section 15.10. During the pendency of such dispute, all the terms of this Agreement will remain in effect and the Parties will continue to perform all of their respective obligations hereunder.

13.2.4 **Termination for Insolvency**. If, at any time during the Term, either Party makes an assignment for the benefit of creditors, appoints or suffers appointment of a receiver or trustee over all or substantially all of its property, files a petition under any bankruptcy or insolvency act or has any such petition filed against it that is not discharged within [**] after the filing thereof, the other Party may terminate this Agreement in its entirety by providing written notice of its intent to terminate this Agreement to such Party, in which case, this Agreement will terminate on the date on which such Party receives such written notice.

13.2.5 **Termination for Patent Challenge**. If Ultragenyx, any of its Affiliates or Sublicensees (a) solely with respect to Licensed Patents owned by [**], issues a press release, public announcement, news release alleging invalidity or unenforceability of any claim within such Licensed Patents; (b) asserts a claim or counterclaim in the courts or before the applicable governmental agency (e.g., the United States Patent Trial and Appeal Board) seeking to attack, invalidate or render unenforceable any claim within the Licensed Patents; or (c) assists a third party with either or both of the foregoing ((a) or (b)) (each of (a), (b) or (c) being a "**Patent Challenge**"), then (x) solely with respect to Licensed Patents owned by [**], Ultragenyx shall provide Solid and [**] with at least [**] notice prior to taking any such action, and (y) following [**] prior written notice thereof from Solid, unless Ultragenyx ceases such Patent Challenge, Solid may terminate this Agreement immediately upon written notice to Ultragenyx. To the extent required by an Existing In-License Agreement, [**]. Notwithstanding the foregoing, "**Patent Challenge**" does not include a response to a claim by Solid or any of its Affiliates that Ultragenyx or any of its Affiliates is engaging in patent infringement. Further, this Section 13.2.5 will not apply to any Patent Challenge involving a Third Party acquiree of Ultragenyx (i) if such Patent Challenge was initiated at least [**] before the signing of the definitive document(s) whereby such Third Party becomes an acquiree of Ultragenyx or (ii) if such Patent Challenge was initiated within any such [**] period, if Ultragenyx causes such Patent Challenge to be terminated or dismissed (or in the case of ex parte proceedings, multi-party

proceedings, or other Patent Challenges to be withdrawn, causes such acquiree to withdraw as a party from such Patent Challenge and to cease actively assisting any other party to such Patent Challenge).

13.3 **Consequences of Expiration or Termination of the Agreement**.

13.3.1 If this Agreement expires or is terminated, the following terms will apply:

(a) each Party's rights and obligations under this Agreement with respect to the Licensed Product(s) will automatically cease;

(b) each Party will take all actions required under Section 14.3;

(c) all Committees will automatically be dissolved; and

(d) solely in the case of termination (but not expiration) of the Agreement, any Sublicense of Ultragenyx will, at the Sublicensee's option, survive such termination on the conditions that (i) the relevant Sublicensee is not in material breach of any of its obligations under such Sublicense, (ii) the relevant Sublicensee pays to Solid any outstanding amounts due hereunder with respect to milestones achieved by such Sublicensee or sales of Licensed Products by such Sublicensee amounts and (iii) solely with respect to any Licensed Patents that are both owned by an Institution and subject to such Sublicense, solely to the extent permitted by the relevant Existing In-License Agreement. In order to effect this provision, at the request of the Sublicensee, Solid will enter into a direct license with the Sublicensee on terms that are substantially the same as the applicable terms (including economic terms) of this Agreement; provided that (A) Solid will not be required to undertake obligations in addition to those required by this Agreement, (B) Solid's right under such direct license will be consistent with its rights under this Agreement, taking into account the scope of the license granted under such direct license and (C) the license grant by Solid to such Sublicensee will only include the Licensed Technology and Joint Technology in existence as of the effective date of such termination. If the Sublicensee pays any amounts to Solid in accordance with the foregoing clause (ii), Solid will promptly inform Ultragenyx of the same and will not seek payment from Ultragenyx for the same milestones or sales.

13.3.2 Termination or expiration of this Agreement will be without prejudice to any rights or financial compensation that will have accrued to the benefit of a Party prior to such expiration or termination. Such expiration or termination will not relieve a Party from obligations that are expressly indicated to survive the termination or expiration of this Agreement.

13.3.3 The following provisions of this Agreement will survive the expiration or termination of this Agreement: Article 1, Article 12, Article 14, Article 15, Section 2.2.1 (but only for [**]), Section 8.5, Section 9.2.4 (solely with respect to accrued but unpaid amounts as of the effective date of expiration or termination), Section 9.3.7 (solely with respect to accrued but unpaid amounts as of the effective date of termination and reports with respect thereto), Section 9.5 (solely with respect to accrued but unpaid amounts as of the effective date of expiration or termination), Section 9.6 (solely with respect to accrued but unpaid amounts as of the effective date of expiration or termination), Section 9.7 (solely with

respect to accrued but unpaid amounts as of the effective date of expiration or termination), Section 9.9 (but only for [**]), Section 9.10, Section 9.11 (solely with respect to accrued but unpaid amounts as of the effective date of expiration or termination), Section 10.1, Section 10.2, Section 10.3.3, Section 10.5 (solely with respect to Joint Patents) Section 11.5, Section 12.1, Section 12.3, Section 12.4 and Section 13.3.

ARTICLE 14
CONFIDENTIALITY

14.1 **Confidentiality**. During the Term and for [**] thereafter, each Party (the "**Receiving Party**") receiving any Confidential Information of the other Party (the "**Disclosing Party**") hereunder will: (a) keep the Disclosing Party's Confidential Information confidential; (b) not publish, or allow to be published, and not otherwise disclose, or permit the disclosure of, the Disclosing Party's Confidential Information; and (c) not use, or permit to be used, the Disclosing Party's Confidential Information for any purpose, except, in each case, to the extent expressly permitted under this Agreement or otherwise agreed in writing. Without limiting the generality of the foregoing, to the extent that either Party provides the other Party any Confidential Information owned by any Third Party, the Receiving Party will handle such Confidential Information in accordance with the terms of this Article 14 applicable to a Receiving Party.

14.2 **Authorized Disclosure**. Notwithstanding Section 14.1, each Party may disclose the other Party's Confidential Information to the extent such disclosure is reasonably necessary to:

14.2.1 file or prosecute patent applications as contemplated by this Agreement;

14.2.2 prosecute or defend litigation;

14.2.3 allow its Affiliates and actual or potential Sublicensees and actual or potential Subcontractors, in each case, to exercise its rights or perform its obligations under this Agreement; provided that such disclosure is covered by terms of confidentiality at least as restrictive as those set forth herein (but of duration customary in confidentiality agreements entered into for a similar purpose);

14.2.4 subject to the remainder of this Section 14.2, share with its advisors (including financial advisors, attorneys and accountants), actual or potential acquisition partners, financing sources or investors and underwriters on a need to know basis; provided that such disclosure is covered by terms of confidentiality similar to those set forth herein (which may include professional ethical obligations); or

14.2.5 comply with Applicable Law (including to obtain and maintain Marketing Approvals for a Licensed Product); or

14.2.6 with respect to Solid, to the counterparty to an Existing In-License Agreement solely to comply with reporting obligations thereunder, subject to the confidentiality provisions of such Existing In-License Agreement;

provided that with respect to Section 14.2.1, 14.2.2, 14.2.5 or 14.2.6, the Receiving Party will notify the Disclosing Party of the Receiving Party's intent to make any disclosure pursuant thereto sufficiently prior to making such disclosure so as to allow the Disclosing Party adequate time to take whatever action it may deem appropriate to protect the confidentiality of the information to be disclosed.

14.3 **Expiration or Termination of this Agreement**. Following the expiration or termination of this Agreement, if requested by the Disclosing Party, at the Receiving Party's election, the Receiving Party will use diligent efforts to return or destroy, all data, files, records and other materials containing or comprising the Disclosing Party's Confidential Information, except to the extent such Confidential Information is necessary or reasonably useful to conduct surviving obligations or exercise surviving rights. Notwithstanding the foregoing, (a) the Receiving Party will be permitted to retain one copy of such data, files, records, and other materials for archival and legal compliance purposes and (b) the Receiving Party will not be required to return or destroy electronically stored information that is commercially impractical to access, segregate or destroy, including any electronic back-up tapes or other electronic back-up files that have been created solely by the Receiving Party's automatic or routine archiving and back-up procedures, to the extent created and retained in a manner consistent with its or their standard archiving and back-up procedures.

14.4 **SEC Filings and Other Disclosures**. Either Party may disclose the terms of this Agreement to the extent required to comply with Applicable Law, including the rules and regulations promulgated by the United States Securities and Exchange Commission or any equivalent governmental agency in any country in the Territory; provided that such Party will provide the other Party a reasonable opportunity to review such disclosure and reasonably consider the other Party's comments regarding confidential treatment sought for such disclosure.

14.5 **Public Announcements**. Upon execution of this Agreement, the Parties will issue a joint press release in substantially the form attached hereto as Exhibit C. In addition, either Party may issue press releases or public announcements relating to this Agreement subject to the other Party's prior review (such review period to not be longer than [**]), except that, (a) once a press release or other public statement has been released, made or approved in writing by both Parties, either Party may make subsequent public disclosure of the information contained in such press release or other written statement without the further review of the other Party and (b) either Party may issue a press release or public announcement as required by Applicable Law, including by the rules or regulations of the United States Securities and Exchange Commission or similar regulatory agency in a country other than the United States or of any stock exchange or listing entity; provided that in the case of (b), the issuing Party gives reasonable prior notice to the other Party of and the opportunity to comment on the press release or public announcement, and otherwise complies with this Article 14.

14.6 **Publications**.

14.6.1 **Publications Outside of any Development Option Period or Income Option Period**. During the Term and outside of any Development Option Period or Income Option Period (if any):

(a) None of Solid nor any of its Affiliates may make academic, scientific, medical or other publications or academic, scientific, medical or other public presentations related to the Licensed Technology or Joint Technology, in each case, as it relates to the Licensed Products, without Ultragenyx's prior consent, not to be unreasonably withheld, conditioned or delayed.

(b) Ultragenyx, its Affiliates and Sublicensees may make academic, scientific, medical or other publications or academic, scientific, medical or other public presentations related to the Licensed Technology or Joint Technology as it relates to the Licensed Products; provided that Ultragenyx first submits to Solid for review any such academic, scientific or medical publication or academic, scientific or medical public presentation. Solid will review such publication or presentation for purposes of (i) determining whether any portion of the proposed publication or presentation contains Solid's Confidential Information and (ii) preserving the value of the Licensed Technology or Joint Technology. Written copies of such proposed publication or presentation will be submitted to Solid no later than [**] before submission for publication or presentation. Solid will provide its comments with respect to such publications and presentations within [**] after its receipt of such written copy.

14.6.2 **Publications During a Development Option Period or Income Option Period**. During any Development Option Period or Income Option Period (if any):

(a) Either Party, its Affiliates and Sublicensees may make academic, scientific, medical or other publications or academic, scientific, medical or other public presentations related to the Licensed Technology or Joint Technology as it relates to the Licensed Products; provided that the disclosing Party first submits to the non-disclosing Party for review any such academic, scientific or medical publication or academic, scientific or medical public presentation related to the Licensed Technology, Joint Technology or a Licensed Product or any activities conducted pursuant to this Agreement with respect to the Licensed Technology or Joint Technology as it relates to a Licensed Product. The non-disclosing Party will review such publication or presentation for purposes of (i) determining whether any portion of the proposed publication or presentation contains the non-disclosing Party's Confidential Information and (ii) preserving the value of the Licensed Technology or Joint Technology. Written copies of such proposed publication or presentation will be submitted to the non-disclosing Party no later than [**] before submission for publication or presentation. The non-disclosing Party will provide its comments with respect to such publications and presentations within [**] after its receipt of such written copy. The review period may be extended for an additional [**] if the non-disclosing Party reasonably requests such extension, including, in the case of Solid, requests to permit Solid to prepare and file patent applications in accordance with Section 10.3.2. The non-disclosing Party may require that disclosing Party, its Affiliate or Sublicensee redact the non-disclosing Party's Confidential Information from any such proposed publication or presentation.

14.6.3 **Institution Publications**. Ultragenyx hereby acknowledges and agrees that the Institutions will have a right to publish any research results or technical data related to or arising out of the Licensed Patents licensed to Solid pursuant to the applicable Existing In-License Agreement in accordance with the applicable Institution's general policies and that this Agreement will not restrict, in any fashion, the Institutions' right to publish as provided in the applicable Existing In-License Agreement as provided to Ultragenyx prior to the Effective Date.

ARTICLE 15
MISCELLANEOUS

15.1 **Assignment**. Except as provided in this Section 15.1, this Agreement may not be assigned or otherwise transferred, nor may any right or obligation hereunder be assigned or transferred, by either Party without the prior written consent of the other Party. Notwithstanding the foregoing, either Party may, without the other Party's prior written consent, assign this Agreement and its rights and obligations hereunder in whole or in part to an Affiliate or to a Third Party that acquires, whether in connection with, merger, sale of assets, reorganization or otherwise, all or substantially all of the business of the assigning Party to which the subject matter of this Agreement relates. Any permitted successor or assignee of any rights or obligation under this Agreement must expressly assume performance thereof. An assignment to an Affiliate will terminate, and all rights so assigned will revert to the assigning Party, if and when such Affiliate ceases to be an Affiliate of the assigning Party. Any purported assignment in violation of this Section 15.1 will be void.

15.2 **Force Majeure**. Each Party will be excused from the performance of its obligations under this Agreement to the extent that such performance is prevented by Force Majeure and the nonperforming Party promptly provides written notice of the Force Majeure to the other Party. Such excuse will continue for so long as the condition constituting a Force Majeure continues, on the condition that the nonperforming Party continues to use Commercially Reasonable Efforts to remove or mitigate the Force Majeure and resume performance of its obligations under this Agreement.

15.3 **Representation by Legal Counsel**. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, no presumption will exist or be implied against the Party that drafted such terms and provisions.

15.4 **Notices**. All written notices which are required or permitted hereunder will be in writing and sufficient if delivered personally or sent by nationally-recognized overnight courier, addressed as follows:

If to Ultragenyx:

Ultragenyx Pharmaceutical Inc.
60 Leveroni Court
Novato, CA 94949
Attention: Chief Business Officer
Telephone: [**]
Email: [**]

with a copy (which will not constitute notice) to:

Goodwin Procter LLP
100 Northern Avenue
Boston, Massachusetts 02210

Attention: [**]
Telephone: [**]
Email: [**]

If to Solid:

Solid Biosciences Inc.
141 Portland Street, Fifth Floor
Cambridge, MA 02139
Attention: [**]
Telephone: [**]
Email: [**]

with a copy (which will not constitute notice) to:

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Steven D. Barrett
Telephone: (617) 526-6000
E-mail: [**]

or to such other address as the Party to whom written notice is to be given may have furnished to the other Party in writing in accordance herewith. Any such written notice will be deemed to have been given and received by the other Party: (a) when delivered if personally delivered; or (b) on receipt if sent by overnight courier.

15.5 **Amendment**. No amendment, modification or supplement of any provision of this Agreement will be valid or effective unless made in writing and signed by a duly authorized officer of each of Ultragenyx and Solid.

15.6 **Waiver**. No provision of this Agreement will be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The waiver by either Party of any breach of any provision hereof by the other Party will not be construed to be a waiver of any succeeding breach of such provision or a waiver of the provision itself.

15.7 **Severability**. If any clause or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same will not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement will be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent possible. In any such event, this Agreement will be construed as if such clause of portion thereof had never been contained in this Agreement, and there will be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by Applicable Law.

15.8 **Descriptive Headings**. The descriptive headings of this Agreement are for convenience only and will be of no force or effect in construing or interpreting any of the provisions of this Agreement.

15.9 **Governing Law**. The Agreement will be construed and the respective rights of the Parties determined in accordance with the substantive laws of the State of New York, notwithstanding any provisions of New York Law or any other law governing conflicts of laws to the contrary. The provisions of the United Nations Convention on Contracts for the International Sale of Goods will not apply to this Agreement or any subject matter hereof.

15.10 **Dispute Resolution**. Except as otherwise expressly set forth in this Agreement, including Section 7.3, disputes of any nature arising under, relating to, or in connection with this Agreement ("**Disputes**") will be resolved pursuant to this Section 15.10.

15.10.1 **Tolling**. The Parties agree that all applicable statutes of limitation and time-based defenses (such as estoppel and laches), as well as all time periods in which a Party must exercise rights or perform obligation hereunder, will be tolled once the dispute resolution procedures set forth in this Section 15.10 have been initiated and for so long as they are pending, and the Parties will cooperate in taking all actions reasonably necessary to achieve such a result. Further, with respect to any time periods that have run during the pendency of the Dispute, the applicable Party will have a reasonable period of time or any specific timeframe established by the tribunal's decision to exercise any rights or perform any obligations affected by the running of such time periods.

15.10.2 **Informal Dispute Resolution; Escalation to Chief Executive Officers**. In the event of any Dispute, the Parties will first attempt in good faith to resolve such Dispute by negotiation and consultation between themselves. If, after [**] from receipt of the written notice of a Dispute, such Dispute has not been resolved on an informal basis, either Party may refer any Dispute to the Chief Executive Officers of the Parties by delivering written notice to the other Party, who will confer in good faith on the resolution of the issue for a [**] period following receipt of such written notice. If any Dispute is not resolved within such [**] period by the Chief Executive Officers, each Party may, at its sole discretion, pursue any and all rights and remedies available to such Party in law or equity in a court of competent jurisdiction.

15.10.3 **Injunctive Relief**. Notwithstanding the dispute resolution procedures set forth in this Section 15.10, in the event of an actual or threatened breach of this Agreement, the aggrieved Party may seek provisional equitable relief (including restraining orders, specific performance or other injunctive relief), without first submitting to any dispute resolution procedures hereunder or posting any bond.

15.10.4 **WAIVER OF JURY TRIAL**. EXCEPT AS LIMITED BY APPLICABLE LAW, EACH PARTY HERETO HEREBY IRREVOCABLY WAIVES ALL RIGHT TO TRIAL BY JURY IN ANY ACTION, PROCEEDING OR COUNTERCLAIM (WHETHER BASED IN CONTRACT, TORT OR OTHERWISE) ARISING OUT OF OR RELATING TO THIS AGREEMENT OR THE ACTIONS OF EITHER PARTY HERETO IN THE NEGOTIATION, ADMINISTRATION, PERFORMANCE AND ENFORCEMENT HEREOF.

15.11 **Entire Agreement**. This Agreement (together with all schedules and exhibits attached hereto) constitutes and contains the complete, final and exclusive understanding and agreement of the Parties and cancels and supersedes all prior negotiations, correspondence, understandings and agreements, whether oral or written, between the Parties respecting the subject matter hereof, including the CDA, which is hereby superseded and replaced in its entirety as of the Effective Date.

15.12 **Independent Contractors**. Both Parties are independent contractors under this Agreement. Nothing contained herein will be deemed to create an employment, agency, joint venture or partnership relationship between the Parties or any of their agents or employees, including for U.S. federal income and other applicable tax purposes, or any other legal arrangement that would impose liability upon one Party for the act or failure to act of the other Party. Neither Party will have any express or implied power to enter into any contracts or commitments or to incur any liabilities in the name of, or on behalf of, the other Party, or to bind the other Party in any respect whatsoever.

15.13 **Interpretation**. Except where the context expressly requires otherwise, (a) the use of any gender herein will be deemed to encompass references to either or both genders, and the use of the singular will be deemed to include the plural (and vice versa); (b) the words "include", "includes" and "including" will be deemed to be followed by the phrase "without limitation" and will not be interpreted to limit the provision to which it relates; (c) the word "will" will be construed to have the same meaning and effect as the word "shall"; (d) any definition of or reference to any agreement, instrument or other document herein will be construed as referring to such agreement, instrument or other document as from time to time amended, supplemented or otherwise modified (subject to any restrictions on such amendments, supplements or modifications set forth herein); (e) any reference herein to any Person will be construed to include the Person's successors and assigns; (f) the words "herein", "hereof" and "hereunder", and words of similar import, will be construed to refer to this Agreement in each of their entirety, as the context requires, and not to any particular provision hereof; (g) all references herein to Sections or Schedules or Exhibits will be construed to refer to Sections or Schedules or Exhibits of this Agreement, and references to this Agreement include all Schedules and Exhibits hereto; (h) the word "notice" means notice in writing (whether or not specifically stated) and will include notices, consents, approvals and other written communications contemplated under this Agreement; (i) provisions that require that a Party, the Parties or any committee hereunder "agree," "consent" or "approve" or the like will require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise (but excluding e-mail and instant messaging); (j) references to any specific law, rule or regulation, or article, section or other division thereof, will be deemed to include the then-current amendments thereto or any replacement or successor law, rule or regulation thereof; and (k) the term "or" will be interpreted in the inclusive sense commonly associated with the term "and/or".

15.14 **No Third Party Rights or Obligations**. No provision of this Agreement will be deemed or construed in any way to result in the creation of any rights or obligations in any Person not a Party to this Agreement.

15.15 **Use of Name**. No provision of this Agreement grants Ultragenyx, its Affiliates or Sublicensees any right or license to use the name or trademarks of (i) Solid or the names or identities of any employee or agent of Solid or (ii) the Institutions or the names or identities of any member of the faculty, staff, or student body of the Institutions. Except as required by Applicable Law, Ultragenyx will

not use, and will not permit any Affiliate or Sublicensee to use, any such trademarks, names, or identities without the applicable Institution's and, as the case may be, such member's prior written approval. No provision of this Agreement grants Solid or its Affiliates or (sub)licensees any right or license to use the name or trademarks of Ultragenyx or the names or identities of any employee or agent of Ultragenyx. Except as required by Applicable Law, Solid will not use, and will not permit any Affiliate to use, any such trademarks, names, or identities without Ultragenyx's prior written approval.

15.16 **Further Actions**. Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

15.17 **Counterparts**. This Agreement may be executed in two (2) counterparts, each of which will be an original and both of which will constitute together the same document. Counterparts may be signed and delivered by digital transmission (*e.g.*, .pdf), each of which will be binding when received by the applicable Party.

[Signature Page Follows]

IN WITNESS WHEREOF, the Parties have caused this Agreement to be executed by their duly authorized representatives as of the Effective Date.

ULTRAGENYX PHARMACEUTICAL INC.	**SOLID BIOSCIENCES INC.**
By: /s/ Emil D. Kakkis, M.D., Ph.D.	By: /s/ Ilan Ganot
Name: Emil D. Kakkis, M.D., Ph.D.	Name: Ilan Ganot
Title: Chief Executive Officer	Title: Chief Executive Officer

Exhibit A
Development Cost Sharing Terms

1. Development Plan and Budget. On an Option Product-by-Option Product basis, as set forth more fully in Section 3.3.5(a) of this Agreement, during the Development Option Period, Ultragenyx will prepare an Option Territory Development Plan for JSC review and approval. For clarity, each Option Territory Development Plan will contain a plan for Option Territory Development Activities as set forth more fully in Section 3.3.5(a).

2. Cost Sharing Responsibility. Solid will be responsible for thirty percent (30%) of the Option Territory Development Costs and Ultragenyx will be responsible for seventy percent (70%) of the Option Territory Development Costs, in each case that are incurred pursuant to the applicable Option Territory Development Budget for such activities as approved by the JSC.

3. Payment and Reimbursement. Within [**] after the end of each Calendar Quarter during the Development Option Period, each Party will provide to the JFC a good faith estimate of the Option Territory Development Costs that were incurred by or on behalf of such Party during such Calendar Quarter. Within [**] after the end of each Calendar Quarter during the Development Option Period, each Party will provide to the JFC a detailed expense report in the form approved by the JFC with respect to the Option Territory Development Costs incurred by or on behalf of such Party during such Calendar Quarter. Solid will then invoice Ultragenyx for seventy percent (70%) of such costs incurred by Solid and Ultragenyx will then invoice Solid for thirty percent (30%) of such costs incurred by Ultragenyx. Each Party will pay the other Party the undisputed amount set forth in the invoice received by such Party in accordance with this Section 3 of this Exhibit A within [**] following the end of the relevant Calendar Quarter, and will pay any disputed amounts within [**] of final resolution of the applicable dispute.

4. Deferrals. In the event Solid defers any Option Territory Development Costs in accordance with Section 3.3.5(a), Solid will pay such costs to Ultragenyx as set forth in such section.

Exhibit B
Cost/Income Sharing Terms

1. Sharing of Income and Losses. Net Income and Net Losses (each as defined below) in the Option Territory will be calculated in accordance with this Exhibit B. This Exhibit B applies solely to Income Share Products after the end of the corresponding Development Option Period and for the remainder of the relevant Income Option Period. On an Income Share Product-by-Income Share Product basis, Ultragenyx will be entitled to and will bear seventy percent (70%) of all Net Income and Net Losses incurred in each Calendar Quarter that the Cost/Income Share is in effect with respect to such Income Share Product being sold in the Option Territory by a Selling Party and Solid will be entitled to and will bear thirty percent (30%) of all Net Income and Net Losses incurred in each Calendar Quarter that the Cost/Income Share is in effect with respect to an Income Share Product being sold in the Option Territory by Ultragenyx or its Affiliate, in each case, pursuant to these Cost/Income Sharing Terms. Ultragenyx will have the sole right to record gross revenue on all sales as principal for Income Share Products.

2. Reports and Payments in General. Within [**] after the end of each Calendar Quarter, Solid will report to Ultragenyx the preliminary Allowable Expenses, and within [**] after the end of each Calendar Quarter, will submit to Ultragenyx the final report of Allowable Expenses, made or to be made by Solid with respect to each Income Share Product in the Option Territory for the just-ended Calendar Quarter. Ultragenyx will report to Solid, within [**] after the end of each Calendar Quarter a preliminary report, and within [**] after the end of each Calendar Quarter, the complete report, in each case, of the outstanding amount a Party will be responsible for paying to the other Party such that the Parties share Net Income or Net Losses, as applicable, with respect to the Income Share Product in accordance with the percentages set forth in Section 1 of this Exhibit B (such report, the "**Initial Balancing Report**"), along with [**]. Following Ultragenyx's issuance of the Initial Balancing Report, the Parties will have [**] to discuss such Initial Balancing Report, after which Ultragenyx will issue to Solid a final balancing report which will reflect all of Solid's reasonable and substantiated comments with respect to the corresponding Initial Balancing Report (such final report, the "**Final Balancing Report**"). Based on the Final Balancing Report, one Party will make a balancing payment (the "**Balancing Payment**") to the other Party as follows:

(a) If, based on such Final Balancing Report, Ultragenyx is responsible for making a Balancing Payment to Solid for such Calendar Quarter, then Solid will invoice Ultragenyx for the amount due and Ultragenyx will pay, within [**] after the date of the Initial Balancing Report, the amount due to Solid minus credits for a portion of, or all of, any amounts due from Solid to Ultragenyx (if any), in an amount not to exceed the aggregate amount of such invoice (regardless of whether related to the specific Income Share Product); or

(b) If, based on such Final Balancing Report, Solid is responsible for making a Balancing Payment to Ultragenyx for such Calendar Quarter, then Ultragenyx will invoice Solid for the amount due and Solid will pay, within [**] after the date of the Initial Balancing Report, the amount due to Ultragenyx minus credits for a portion of, or all of, any amounts due from Ultragenyx to Solid (if any), in an amount not to exceed the aggregate amount of such invoice (regardless of whether related to the specific Income Share Product).

3. Definitions.

(a) "**Allowable Expenses**" means, subject to the other provisions of this Agreement and this Exhibit B, the sum of the FTE Costs (if applicable) and Out-of-Pocket Costs, in each case, that are (i) incurred by a Party or any of its Affiliates or Sublicensees, (ii) specifically identifiable or reasonably allocable (including in accordance with the Allocation Methodology) to the LCM Development or Commercialization of the Income Share Product in the Option Territory, or

specifically identifiable or reasonably allocable (including in accordance with the Allocation Methodology) to the Manufacture of the Income Share Product for use in such LCM Development or Commercialization activities, and (iii) recorded in accordance with Accounting Standards. The Allowable Expenses will include the following costs, losses and expenses except to the extent such amounts are included in FTE Costs as Indirect Allocations:

[**].

Notwithstanding the foregoing, Allowable Expenses will exclude: [**] the "**Exclusions**"). Costs may not be included more than once in Allowable Expenses, even if a particular cost satisfies the definition of more than one cost category above.

Except for cost of goods, where the Allocation Methodology will be that same allocation of costs and expenses applied by Ultragenyx for the calculation of cost of standard goods in accordance with its Accounting Standards, to the extent that any of the costs described above apply to both the Option Territory and one or more other countries or territories in the Territory outside of the Option Territory, such costs will be allocated between the Option Territory and such other countries or territories in accordance with Ultragenyx's Accounting Standards (such methodology, the "**Allocation Methodology**") and included in Allowable Expenses only to the extent allocable to the Option Product in the Field in the Option Territory.

(b) "**Distribution Costs**" means, to the extent not included in Manufacturing Costs, the FTE Costs and Out-of-Pocket Costs recorded as an expense by a Selling Party that are specifically identifiable or reasonably allocable to the commercial distribution of the Income Share Product to a Third Party in the Option Territory in accordance with the Allocation Methodology, including:

[**].

(c) "**Manufacturing Costs**" means, with respect to an Income Share Product (including, to the extent applicable, in each case, works-in-progress thereof) and to the extent not included in Distribution Costs, the fully-burdened cost incurred by Ultragenyx or any of its Affiliates for Manufacturing or purchasing from a Third Party, as applicable, the Option Product only to the extent allocable to such Manufacture, including (i) the costs associated with inventory build-up in advance of the launch of the Income Share Product in the Option Territory (including validation batches if deemed saleable), but only until technical feasibility is determined and such Income Share Product inventory can be capitalized, and thereafter such Income Share Product inventory will be charged as a cost only at the time such inventory is sold or destroyed, and (ii) the cost to Manufacture samples of the Income Share Product distributed for use in the Option Territory, calculated in accordance with the Allocation Methodology as follows:

[**].

(d) "**HEOR Costs**" means the FTE Costs and Out-of-Pocket Costs associated with health and economic outcomes research and other reviews/analyses/studies relating to value and access issues.

(e) "**LCM Development Costs**" means any FTE Costs and Out-of-Pocket Costs associated with (i) Post-Marketing Clinical Trials of the Income Share Product and (ii) Development of the Income Share Product for the Option Territory after the first Marketing Approval for the Income Share Product in the Option Territory (such Development, "**LCM Development**").

(f) "**Medical Affairs Costs**" means the FTE Costs and Out-of-Pocket Costs, including costs for independent contractors engaged, incurred by Ultragenyx or any of its Affiliates during the

Term that are specifically identifiable or reasonably allocable to medical affairs with respect to the Income Share Product sold in the Option Territory.

(g) "**Net Income**" and, with correlative meaning, "**Net Losses**", means, with respect to the Income Share Product, [**]. Any positive amount resulting from such calculation will be a Net Income and any negative amount resulting from such calculation will be a Net Loss. For the avoidance of doubt, Net Income and Net Losses will exclude all of the payments set forth in Section 9.2 or 9.3 of this Agreement.

(h) "**Patent and Trademark Costs**" means those FTE Costs of in house legal counsel and related personnel and Out-of-Pocket Costs (including the reasonable fees and expenses paid to outside counsel and other Third Parties, and filing and maintenance fees paid to governmental authorities) recorded as an expense by Ultragenyx or any of its Affiliates during the Term, (i) in connection with the prosecution and maintenance of rights, including costs of patent interference, opposition, reissue, inter partes reviews, post grant reviews or reexamination proceedings and filing and registration fees with respect to any Patents Controlled by Ultragenyx, the Joint Patents or the Licensed Patents, that are specifically identifiable or reasonably allocable to the Income Share Product in the Option Territory, in each case, to the extent that they claim the Exploitation of the Income Share Product in the Option Territory, (ii) in connection with the prosecution and maintenance of rights with respect to trademarks that are specifically identifiable or reasonably allocable to the Income Share Product in the Option Territory, and (iii) the costs of litigation (enforcement or defense) or other proceedings described in the foregoing clauses (i) and (ii), in each case only to the extent related to the Income Share Product in the Option Territory and not reimbursed by a Third Party.

(i) "**Patent and Trademark Recoveries**" means all amounts recovered in connection with litigation or proceedings for the Income Share Product as contemplated under this Agreement that are allocated to the Option Territory in accordance with the Allocation Methodology, but excluding each Party's costs and expenses incurred in connection with such litigation or proceedings (which, for the avoidance of doubt, will be reimbursed or shared by the Parties pursuant to this Agreement or pursuant to these Cost/Income Sharing Terms) and the amount of any such recovery paid or payable to the Institutions in respect of the Institutions' rights under the Licensed Technology pursuant to the Existing In-License Agreements.

(j) "**Patient Assistance Program Costs**" means the FTE Costs and Out-of-Pocket Costs associated with programs designed to facilitate patient education and access, including engagements with patient advocacy groups, patient support programs, co-pay assistance, compassionate use programs and patient assistance programs.

(k) [**].

(l) "**Regulatory Expenses**" means those FTE Costs and Out-of-Pocket Costs (including filing, user, maintenance and other fees paid to Regulatory Authorities) recorded as an expense by or on behalf of a Selling Party in accordance with these Cost/Income Sharing Terms, that are specifically identifiable or reasonably and directly allocable to the preparation of regulatory submissions, and the obtaining and maintenance of Marketing Approvals, for the Income Share Product in the Option Territory, including compliance with such Marketing Approvals and requirements of such Regulatory Authorities, adverse event recordation and reporting and regulatory affairs activities.

(m) "**Sales and Marketing Costs**" means those FTE Costs and Out-of-Pocket Costs, including costs for independent contractors engaged as permitted under these Cost/Income Sharing Terms,

recorded as an expense by a Selling Party that are specifically identifiable or reasonably allocable to the sales and marketing of the Income Share Product in the Option Territory. Sales and Marketing Costs include any amounts paid by a Selling Party to Third Parties that are specifically identifiable to the Commercialization of the Income Share Product by such Third Party in the Option Territory, but exclude any Third Party Payments. Subject to the foregoing, Sales and Marketing Costs include costs incurred in connection with the following activities (but in each case only to the extent specifically identifiable or reasonably allocable to the sales and marketing of the Income Share Product in the Option Territory):

[**]

(n) "**Sublicense Revenues**" means all consideration received by Ultragenyx or its Affiliates from a Sublicensee as consideration for the grant of a (sub)license with respect to the Income Share Product in the Option Territory, but excluding any consideration received that is specifically identifiable and reasonably allocable to an independent negotiated-for arrangement that does not relate to the Income Share Product in the Option Territory, including [**]. Sublicensing Revenue in the form of non-cash consideration will be valued at fair market value at the time of receipt by Ultragenyx or its Affiliates.

(o) "**Third Party Payments**" means, with respect to Income Share Products, all [**].

Exhibit 10.2

STOCK PURCHASE AGREEMENT

By and Between

SOLID BIOSCIENCES INC.

AND

ULTRAGENYX PHARMACEUTICAL INC.

Dated as of October 22, 2020

TABLE OF CONTENTS

TABLE OF CONTENTS
(continued)

Page

Page

STOCK PURCHASE AGREEMENT

THIS STOCK PURCHASE AGREEMENT (this "**Agreement**"), dated as of October 22, 2020 (the "**Effective Date**"), is entered into by and between Solid Biosciences Inc., a Delaware corporation (the "**Company**"), and Ultragenyx Pharmaceutical Inc., a Delaware corporation (the "**Investor**"). The Investor and the Company are referred to herein collectively as the "**Parties**".

WHEREAS, pursuant to the terms and subject to the conditions set forth in this Agreement, the Company desires to issue and sell to the Investor, and the Investor desires to subscribe for and purchase from the Company, certain shares of common stock, par value $0.001 per share, of the Company ("**Common Stock**"); and

WHEREAS, simultaneously with the execution of this Agreement, the Company and the Investor are entering into the Collaboration and License Agreement (as defined below).

NOW, THEREFORE, in consideration of the following mutual promises and obligations, and for good and valuable consideration, the adequacy and sufficiency of which are hereby acknowledged, the Investor and the Company agree as follows:

DEFINITIONS

.

Defined Terms

. When used in this Agreement, the following terms shall have the respective meanings specified therefor below:

"**Affiliate**" shall mean, with respect to any Person, another Person that controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to "**control**" another Person if any of the following conditions is met: (i) in the case of corporate entities, direct or indirect ownership of more than fifty percent (50%) of the stock or shares having the right to vote for the election of directors; and (ii) in the case of non-corporate entities, direct or indirect ownership of more than fifty percent (50%) of the equity interest with the power to direct the management and policies of such non-corporate entities. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

"**Agreement**" shall have the meaning set forth in the Preamble, including all Exhibits attached hereto.

"**Business Day**" shall mean a day on which commercial banking institutions in New York, New York are open for business.

"**Collaboration and License Agreement**" shall mean the Collaboration and License Agreement between the Company and the Investor, dated as of the Effective Date.

"**Cross Receipt**" shall mean an executed document signed by each of the Company and the Investor, in substantially the form of Exhibit A attached hereto.

"**Effect**" shall have the meaning set forth in the definition of "Material Adverse Effect."

"**Governmental Authority**" shall mean any court, agency, authority, department, regulatory body or other instrumentality of any government or country or of any national, federal, state, provincial, regional, county, city or other political subdivision of any such government or country or any supranational organization of which any such country is a member.

"**Intellectual Property**" shall mean trademarks, trade names, trade dress, service marks, copyrights, and similar rights (including registrations and applications to register or renew the registration of any of the foregoing), patents and patent applications, trade secrets, and any other similar intellectual property rights.

"**Intellectual Property License**" shall mean any license, permit, authorization, approval, contract or consent granted, issued by or with any Person relating to the use of Intellectual Property.

"**Investor Agreement**" shall mean that certain Investor Agreement between the Investor and the Company, dated as of the Effective Date, in the form of Exhibit B attached hereto.

"**Law**" or "**Laws**" shall mean all laws, statutes, rules, regulations, orders, judgments, injunctions and/or ordinances of any Governmental Authority.

"**Liens**" shall mean all liens, charges, pledges, security interests, encumbrances, rights of first refusal, preemptive rights, restrictions on transfer, claims of Third Parties or other restrictions.

"**Material Adverse Effect**" shall mean any change, event or occurrence (each, an "Effect") that, individually or when taken together with all other Effects, has had (i) a material adverse effect on the business, financial condition, assets or results of operations of the Company and its subsidiaries, taken as a whole, or (ii) a material adverse effect on the Company's ability to perform its obligations, or consummate the Transaction, in accordance with the terms of this Agreement, except in the case of (i) or (ii) to the extent that any such Effect results from or arises out of: (A) changes in conditions in the United States or global economy or capital or financial markets generally, including changes in interest or exchange rates, (B) changes in general legal, regulatory or political conditions or changes in generally accepted accounting principles in the United States or interpretations thereof, (C) changes in general business or economic conditions affecting the Company's industry, (D) the announcement of the Transaction Agreements, the Collaboration and License Agreement or the Transaction, (E) any change in the trading prices or trading volume of the Common Stock or any failure to meet internal projections or forecasts or published revenue or earnings projections of industry analysts (it being understood that the facts giving rise to or contributing to any such change or failure may be deemed to constitute, or be taken into account when determining whether there has been or will be, a Material Adverse Effect), (F) acts of war, sabotage or terrorism, or any escalation or worsening of any such acts of

war, sabotage or terrorism, (G) earthquakes, hurricanes, floods or other natural disasters, (H) pandemics or other health crises, including but not limited to the COVID-19 pandemic, (I) any action taken by the Company with the Investor's express written consent; (J) any breach by the Investor or any of its Affiliates under the Collaboration and License Agreement; or (K) shareholder litigation arising out of or in connection with the execution, delivery or performance of the Transaction Agreements; provided, that, with respect to clauses (A), (B), (C), (F), (G) and (H) such Effect does not have a materially disproportionate and adverse effect on the Company relative to other companies in the biotechnology or biopharmaceutical industries.

"**Organizational Documents**" shall mean (i) the Certificate of Incorporation of the Company, dated as of January 25, 2018, as may be amended and/or restated from time to time, and (ii) the By-laws of the Company, dated as of January 25, 2018, as may be amended and/or restated from time to time.

"**Per Share Purchase Price**" shall mean $5.1113, which is an amount equal to a 33% premium to the volume weighted average price of the Common Stock for the ten (10) trading days prior to the Effective Date, to be calculated and agreed to by each party.

"**Person**" shall mean any individual, partnership, limited liability company, firm, corporation, trust, unincorporated organization, government or any department or agency thereof or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

"**Third Party**" shall mean any Person (other than a Governmental Authority) other than the Investor, the Company or any Affiliate of the Investor or the Company.

"**Transaction**" shall mean the issuance and sale of the Shares by the Company, and the purchase of the Shares by the Investor, in accordance with the terms hereof.

"**Transaction Agreements**" shall mean this Agreement and the Investor Agreement.

Additional Defined Terms

. In addition to the terms defined in Section 1.1, the following terms shall have the respective meanings assigned thereto in the sections indicated below:

Defined Term	Section
Aggregate Purchase Price	Section 2
Closing	Section 3.1
Closing Date	Section 3.1
Common Stock	Preamble
Company	Preamble
Company Rights	Section 4.14(b)
Company SEC Documents	Section 4.11(a)
Company Studies and Trials	Section 4.14(c)
Exchange Act	Section 4.11(a)
Investor	Preamble
Modified Clause	Section 7.7

Defined Term	Section
Money Laundering Laws	Section 4.26
Parties	Preamble
Permits	Section 4.10
Proprietary Rights	Section 4.14(b)
Rule 144	Section 5.9
SEC	Section 4.2(f)
Securities Act	Section 4.11(a)
Shares	Section 2

PURCHASE AND SALE OF COMMON STOCK

. Subject to the terms and conditions of this Agreement, at the Closing, the Company shall issue and sell to the Investor, free and clear of all Liens (other than any Liens arising as a result of any action by the Investor), and the Investor shall purchase from the Company, the number of shares of Common Stock equal to the lesser of (a) the quotient of $40,000,000 divided by the Per Share Purchase Price (rounded down to the nearest whole share) and (b) the number of shares of Common Stock that, if issued to the Investor at the Closing, would result in the Investor holding 19.9% of the Company's outstanding Common Stock (the lesser of (a) and (b), the "**Shares**"), in exchange for the sum of the product of the number of Shares and the Per Share Purchase Price (the "**Aggregate Purchase Price**").

CLOSING DATE; DELIVERIES

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Closing Date

. The closing of the purchase and sale of the Shares hereunder (the "**Closing**") shall take place remotely via the exchange of documents and signatures on the Effective Date or at such other time, date and place as the Parties mutually agree. The date the Closing occurs is hereinafter referred to as the "**Closing Date**."

Deliveries

.

(a) Deliveries by the Company. On the Effective Date, the Company shall deliver to the Investor a counterpart signature page to the Collaboration and License Agreement, duly executed by the Company. At the Closing, the Company shall instruct its transfer agent to register the Shares in book-entry in the name of the Investor. The Company shall also deliver to the Investor at the Closing: (i) a duly executed Cross Receipt; (ii) a duly executed Investor Agreement; and (iii) a certificate of the secretary of the Company dated as of the Closing Date certifying (A) that attached thereto are true and complete copies of the Organizational Documents in effect on the Closing Date; (B) that attached thereto is a true and complete copy of all resolutions adopted by the Board of Directors of the Company authorizing the execution, delivery and performance of the Transaction Agreements and the Transaction and that all such resolutions are in full force and effect and are all the resolutions adopted in connection with the transactions contemplated hereby as of the Closing Date; and (C) as to the incumbency and specimen signature of any officer of the Company executing a Transaction Agreement on behalf of the Company.

(b) Deliveries by the Investor. On the Effective Date, the Investor shall deliver, or cause to be delivered, to the Company a counterpart signature page to the

Collaboration and License Agreement, duly executed by the Investor. At the Closing, the Investor shall deliver, or cause to be delivered, the Aggregate Purchase Price to the Company by wire transfer in immediately available funds to an account designated by the Company. The Investor shall also deliver, or cause to be delivered, to the Company at the Closing: (i) a duly executed Cross Receipt; (ii) a duly executed Investor Agreement; and (iii) a certificate of the secretary of the Investor dated as of the Closing Date certifying as to the incumbency and specimen signature of any officer executing a Transaction Agreement on behalf of the Investor.

REPRESENTATIONS AND WARRANTIES OF THE COMPANY

. The Company hereby represents and warrants the following to the Investor as of the date hereof (except for the representations and warranties that speak as of a specific date, which shall be made as of such date):

Organization, Good Standing and Qualification

.

(a) The Company is a corporation duly organized, validly existing and in good standing under the laws of the State of Delaware. The Company has all requisite corporate power and corporate authority to own, lease and operate its properties and assets, to carry on its business as now conducted, and as proposed to be conducted as described in the Company SEC Documents, to enter into the Transaction Agreements, to issue and sell the Shares and to carry out the other transactions contemplated by the Transaction Agreements.

(b) The Company is qualified to transact business and is in good standing in each jurisdiction in which the character of the properties owned, leased or operated by the Company or the nature of the business conducted by the Company makes such qualification necessary, except where the failure to be so qualified would not have or be reasonably likely to have a Material Adverse Effect.

Capitalization and Voting Rights

.

(a) The authorized capital of the Company as of the Effective Date consists of: (i) 300,000,000 shares of Common Stock of which, as of October 20, 2020, 46,315,889 shares were issued and outstanding, and (ii) 10,000,000 shares of undesignated preferred stock, par value $0.001 per share, none of which are issued and outstanding as of the date of this Agreement. All of the issued and outstanding shares of Common Stock (i) have been duly authorized and validly issued, (ii) are fully paid and non-assessable and (iii) were issued in compliance with all applicable federal and state securities Laws.

(b) All of the authorized shares of Common Stock are entitled to one (1) vote per share.

(c) Except as described or referred to in Section 4.2(a) above, as provided in the Investor Agreement, as set forth in the Company SEC Documents or as disclosed to the Investor on Schedule 4.2(c) hereto, as of the Effective Date, there are not: (i) any outstanding equity securities, options, warrants, rights (including conversion or preemptive rights) or other agreements pursuant to which the Company is or may become obligated to issue, sell or repurchase any shares of its capital stock or any other securities of the Company or (ii)

except as set forth in the Investor Agreement, any restrictions on the transfer of capital stock of the Company other than pursuant to state and federal securities Laws.

(d) Except as provided in the Investor Agreement or as set forth in the Company SEC Documents, the Company is not a party to or subject to any agreement or understanding relating to the voting of shares of capital stock of the Company or the giving of written consents by a stockholder or director of the Company.

(e) Except as provided in the Investor Agreement or as set forth in the Company SEC Documents, no Person has any right to cause the Company to effect the registration under the Securities Act of any securities of the Company.

(f) The Common Stock is registered pursuant to Section 12(b) or 12(g) of the Exchange Act, and the Company has taken no action designed to, or which to its knowledge is likely to have the effect of, terminating the registration of the Common Stock under the Exchange Act nor has the Company received any notification that the Securities and Exchange Commission (the "**SEC**") is contemplating terminating such registration.

Subsidiaries

. The Company has disclosed all of its subsidiaries required to be disclosed pursuant to Item 601(b)(21) of Regulation S-K in an exhibit to its Annual Report on Form 10-K. The Company owns, directly or indirectly, all of the capital stock or other equity interests of each subsidiary free and clear of any Liens, and all of the issued and outstanding shares of capital stock of each subsidiary are validly issued and are fully paid, non-assessable and free of preemptive and similar rights to subscribe for or purchase securities.

Authorization

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(a) All requisite corporate action on the part of the Company and its directors and stockholders required by applicable Law for the authorization, execution and delivery by the Company of the Transaction Agreements and the performance of all obligations of the Company hereunder and thereunder, including the authorization, issuance and delivery of the Shares, has been taken.

(b) Each of the Transaction Agreements have been duly executed and delivered by the Company, and upon the due execution and delivery thereof by the Investor will constitute valid and legally binding obligations of the Company, enforceable against the Company in accordance with their respective terms (except as such enforceability may be limited by (i) applicable bankruptcy, insolvency, reorganization, moratorium or other Laws of general application relating to or affecting enforcement of creditors' rights and (ii) rules of Law governing specific performance, injunctive relief or other equitable remedies and limitations of public policy).

(c) No stop order or suspension of trading of the Common Stock has been imposed by Nasdaq, the SEC or any other Governmental Authority and remains in effect.

No Defaults

. The Company is not in default under or in violation of (a) its Organizational Documents, (b) any provision of applicable Law or any ruling, writ, injunction,

order, Permit, judgment or decree of any Governmental Authority or (c) any agreement, arrangement or instrument, whether written or oral, by which the Company or any of its assets are bound, except, in the case of subsections (b) and (c), as would not have or be reasonably likely to have a Material Adverse Effect. There exists no condition, event or act which after notice, lapse of time, or both, would constitute a default or violation by the Company under any of the foregoing, except, in the case of subsections (b) and (c), as would not have or be reasonably likely to have a Material Adverse Effect.

No Conflicts

. The execution, delivery and performance of the Transaction Agreements and compliance with the provisions hereof and thereof by the Company do not and shall not: (a) violate any provision of applicable Law or any ruling, writ, injunction, order, permit, judgment or decree of any Governmental Authority, (b) constitute a breach of, or default under (or an event which, with notice or lapse of time or both, would become a default under) or conflict with, or give rise to any right of termination, cancellation or acceleration of, any agreement, arrangement or instrument, whether written or oral, by which the Company or any of its assets are bound, (c) result in any encumbrance upon any of the Shares, other than restrictions pursuant to the Investor Agreement or securities Laws, or on any of the properties or assets of the Company or any subsidiary or (d) violate or conflict with any of the provisions of the Organizational Documents, except, in the case of subsections (a), (b) and (c), as would not have or be reasonably likely to have a Material Adverse Effect.

No Governmental Authority or Third Party Consents

. No consent, approval, authorization or other order of, or filing with, or notice to, any Governmental Authority or other Third Party is required to be obtained or made by the Company in connection with the authorization, execution and delivery by the Company of any of the Transaction Agreements, or with the authorization, issue and sale by the Company of the Shares, except (a) such filings as may be required to be made with the SEC and with any state blue sky or securities regulatory authority, which filings shall be made in a timely manner in accordance with all applicable Laws and (b) if required, with respect to the Shares, the filing with The Nasdaq Stock Market LLC of, and the absence of unresolved issues with respect to, a Notification Form: Listing of Additional Shares.

Valid Issuance of Shares

. When issued, sold and delivered at the Closing in accordance with the terms hereof for the Aggregate Purchase Price, the Shares shall be duly authorized, validly issued, fully paid and nonassessable, free from any Liens or restrictions on transfer, other than as arising pursuant to the Investor Agreement, as a result of any action by the Investor or under federal or state securities Laws.

Litigation

. Except as set forth in the Company SEC Documents filed prior to the date of this Agreement, there is no action, suit, proceeding or investigation pending (of which the Company has received notice or otherwise has knowledge) or, to the Company's knowledge, threatened, against the Company or which the Company intends to initiate which has had or is reasonably likely to have a Material Adverse Effect. None of the Company or any property, right or asset of the Company is a party to, or subject to the provisions of, any order writ, injunction, judgment, award, ruling or decree of, or settlement agreement with, any Governmental Authority which has had or is reasonably likely to have a Material Adverse Effect.

Licenses and Other Rights; Compliance with Laws

. The Company has all franchises, permits, licenses and other rights and privileges ("**Permits**") necessary to permit it to own its properties and to conduct its business as presently conducted and is in compliance thereunder, except where the failure to be in compliance does not and would not have or be reasonably likely to have a Material Adverse Effect. The Company has not taken any action that would interfere with the Company's ability to renew all such Permit(s), except where the failure to renew such Permit(s) would not have or be reasonably likely to have a Material Adverse Effect. The Company is and has been in compliance with all Laws applicable to its business, properties and assets, and to the products and services sold by it, except where the failure to be in compliance does not and would not have or be reasonably likely to have a Material Adverse Effect.

Company SEC Documents; Financial Statements; Nasdaq Stock Market

.

(a) Since January 25, 2018, the Company has timely filed with the SEC all reports, schedules, forms, statements and other documents (including exhibits and all other information incorporated therein), and any amendments to any of the foregoing, in each case required to be filed with the SEC under the Securities Act of 1933, as amended (the "**Securities Act**"), or the Securities Exchange Act of 1934, as amended (the "**Exchange Act**"), (the "**Company SEC Documents**"). As of their respective filing dates, each of the Company SEC Documents complied in all material respects with the requirements of the Securities Act and the Exchange Act and the rules and regulations of the SEC promulgated thereunder applicable to such Company SEC Documents, and no Company SEC Documents when filed, declared effective or mailed, as applicable, contained any untrue statement of a material fact or omitted to state a material fact required to be stated therein or necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading.

(b) As of the date of this Agreement, other than as has been disclosed to the Investor, there are no outstanding or unresolved comments in comment letters received from the SEC or its staff. As of the date of this Agreement, none of the Company's subsidiaries is subject to the reporting requirements of Section 13(a) or 15(d) under the Exchange Act.

(c) The financial statements of the Company included in its Annual Report on Form 10-K for the fiscal year ended December 31, 2019 and in its quarterly reports on Form 10-Q for the quarterly periods ended March 31, 2020 and June 30, 2020 comply as to form in all material respects with applicable accounting requirements and the published rules and regulations of the SEC with respect thereto, have been prepared in accordance with United States generally accepted accounting principles applied on a consistent basis during the periods involved (except as may be indicated in the notes thereto) and fairly present in all material respects the financial position of the Company as of the dates thereof and the results of its operations and cash flows for the periods then ended. Except (i) as set forth in the Company SEC Documents or (ii) for liabilities incurred in the ordinary course of business subsequent to the date of the most recent balance sheet contained in the Company SEC Documents, the Company has no liabilities, whether absolute or accrued, contingent or otherwise, other than those that would not, individually or in the aggregate, have or be reasonably likely to have a Material Adverse Effect.

(d) As of the date of this Agreement, the Common Stock is listed on The Nasdaq Global Select Market, and the Company has taken no action designed to, or which to its knowledge is likely to have the effect of, terminating the registration of the Common Stock under the Exchange Act or delisting the Common Stock from The Nasdaq Global Select Market. As of the date of this Agreement, the Company has not received any notification that, and has no knowledge that, the SEC or The Nasdaq Stock Market LLC is contemplating terminating such listing or registration nor, to the Company's knowledge, is there any reasonable basis for, the delisting of the Common Stock from Nasdaq.

Absence of Certain Changes

. Except as disclosed in the Company SEC Documents filed prior to the date hereof, since December 31, 2019:

(a) there has not occurred any event that has caused or would reasonably be expected to cause a Material Adverse Effect;

(b) the Company has not (i) declared or paid any dividends, or authorized or made any distribution upon or with respect to any class or series of its capital stock, or (ii) sold, exchanged or otherwise disposed of any of its material assets or rights;

(c) the Company has not incurred any material liabilities or obligations (contingent or otherwise) other than such liabilities not required to be reflected in the Company's financial statements pursuant to United States generally accepted accounting principles or disclosed in the Company SEC Documents or such liabilities and obligations incurred in the ordinary course of business consistent with past practice since the date of the most recent consolidated balance sheet of the Company and its subsidiaries included in the Company SEC Documents publicly available prior to the date hereof;

(d) the Company has not altered in any material respect its method of accounting or the manner in which it keeps its accounting books and records; and

(e) the Company has not admitted in writing its inability to pay its debts generally as they become due, filed or consented to the filing against it of a petition in bankruptcy or a petition to take advantage of any insolvency act, made an assignment for the benefit of creditors, consented to the appointment of a receiver for itself or for the whole or any substantial part of its property, or had a petition in bankruptcy filed against it, been adjudicated a bankrupt, or filed a petition or answer seeking reorganization or arrangement under the federal bankruptcy laws or any other laws of the United States or any other jurisdiction.

Internal Controls; Disclosure Controls and Procedures

. The Company maintains internal control over financial reporting as defined in Rule 13a-15(f) under the Exchange Act. The Company has implemented the "disclosure controls and procedures" (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) required in order for the Principal Executive Officer and Principal Financial Officer of the Company to engage in the review and evaluation process mandated by the Exchange Act, and is in compliance with such disclosure controls and procedures in all material respects. Each of the Principal Executive Officer and the Principal Financial Officer of the Company (or each former Principal Executive Officer of the Company and each former Principal Financial Officer of the Company, as

applicable) has made all certifications required by Sections 302 and 906 of the Sarbanes-Oxley Act of 2002 with respect to all reports, schedules, forms, statements and other documents required to be filed by the Company with the SEC.

Intellectual Property

.

(a) The Intellectual Property that is owned by the Company is owned free from any Liens or restrictions, and all of the Company's material Intellectual Property Licenses are in full force and effect in accordance with their terms, are free of any Liens or restrictions, and neither the Company nor to the Company's knowledge any other party thereto, is in material breach of any such material Intellectual Property License, and no event has occurred that with notice or lapse of time or both would constitute such a material breach or default thereunder or would result in the termination thereof or would cause or permit the acceleration or other change of any right or obligation of the loss of any benefit thereunder by the Company, except (i) for such failures to be in full force and effect, such Liens or restrictions, and such material breaches, that would not reasonably be expected to have a Material Adverse Effect, or (ii) as set forth in any such Intellectual Property License. Except as set forth in the Company SEC Documents, there is no legal claim or demand of any Person pertaining to, or any proceeding which is pending (of which the Company has received notice or otherwise has knowledge) or, to the knowledge of the Company, threatened, (i) challenging the right of the Company in respect of any Company Intellectual Property, or (ii) that claims that any default exists under any Intellectual Property License, except, in the case of (i) and (ii) above, where any such claim, demand or proceeding would not have or reasonably be expected to have a Material Adverse Effect.

(b) Except as set forth in the Company SEC Documents: (i) the Company or one of its subsidiaries owns, free and clear of any Lien, or has a valid license to, or has an enforceable right to use, as it is used or held for use, all U.S. and non-U.S. patents, trade secrets, know-how, trademarks, service marks, copyrights, and other proprietary and intellectual property rights, and all grants and applications with respect to the foregoing (collectively, the "**Proprietary Rights**") necessary for the conduct of the Company's business as currently conducted, the absence of which would not have or reasonably be expected to have a Material Adverse Effect (such Proprietary Rights owned by or licensed to the Company collectively, the "**Company Rights**"); and (ii) the Company and its subsidiaries have taken reasonable measures to protect the Company Rights, consistent with prudent commercial practices in the biotechnology industry, except where failure to take such measures would not have or reasonably be expected to have a Material Adverse Effect.

(c) The studies, tests and preclinical or clinical trials conducted by or on behalf of the Company (the "**Company Studies and Trials**") were and, if still pending, are being conducted in all material respects in accordance with experimental protocols, procedures and controls pursuant to, where applicable, accepted professional scientific standards. The descriptions of the results of the Company Studies and Trials contained in the Company SEC Documents are accurate in all material respects. Except as set forth in the Company SEC Documents, the Company has not received any notices or correspondence from the United States Food and Drug Administration or any Governmental Authority exercising comparable authority requiring the termination, suspension or material modification of any Company Studies and

Trials, except for any such termination, suspension or material modification that has not had and would not reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect and, to the Company's knowledge, there are no reasonable grounds for the same.

Tax Returns, Payments and Elections

. The Company has filed all tax returns and reports as required, and within the time prescribed, by law and has paid or made provision for the payment of all accrued and unpaid taxes to which the Company is subject and which are not currently due and payable, except where any failure would not have a Material Adverse Effect. No U.S. federal, state, local or non-U.S. tax audits or administrative or judicial tax proceedings are pending or being conducted with respect to the Company. The Company has not received from any U.S. federal state, local or non-U.S. taxing authorities any (i) notice indicating an intent to open and audit or other review, (ii) request for information related to tax matters, or (iii) notice of deficiency or proposed adjustment for any amount of tax proposed, asserted, or assessed by any taxing authority against the Company or any of its subsidiaries.

Offering

. Subject to the accuracy of the Investor's representations set forth in Sections 5.5, 5.6, 5.7, 5.9 and 5.10, the offer, sale and issuance of the Shares to be issued in conformity with the terms of this Agreement constitute transactions which are exempt from the registration requirements of the Securities Act and from all applicable state registration or qualification requirements. Neither the Company nor any Person acting on its behalf will take any action that would cause the loss of such exemption.

No Integration

. The Company has not, directly or through any agent, sold, offered for sale, solicited offers to buy or otherwise negotiated in respect of, any security (as defined in the Securities Act) which is or will be integrated with the Shares sold pursuant to this Agreement in a manner that would require the registration of the Shares under the Securities Act.

Brokers' or Finders' Fees

. No broker, finder, investment banker or other Person is entitled to any brokerage, finder's or other fee or commission from the Company in connection with the transactions contemplated by the Transaction Agreements.

Not Investment Company

. The Company is not, and immediately after receipt of the Aggregate Purchase Price will not be, an "investment company" as defined in the Investment Company Act of 1940, as amended.

Insurance

. The Company is insured by insurers of recognized financial responsibility against such losses and risks and in such amounts as are prudent and customary in the businesses in which the Company is engaged and for an enterprise at a substantially similar stage of lifecycle as the Company, including, but not limited to, directors and officers insurance coverage. To the Company's knowledge, it will be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage from similar insurers as may be necessary to continue its business.

No General Solicitation

. Neither the Company nor any Person acting on behalf of the Company has offered or sold any of the Shares by any form of general solicitation or general advertising. The Company has not, directly or through any agent, sold, offered for sale, solicited offers to buy or otherwise negotiated in respect of, any security (as defined in the

Securities Act) in a manner or under any circumstances that would require the registration of the Shares under the Securities Act (including, without limitation, by virtue of the integration of the offering of the Shares with any prior offering of Company shares).

Foreign Corrupt Practices

. Neither the Company nor, to the knowledge of the Company, any agent or other person acting on behalf of the Company, has: (i) directly or indirectly, used any funds for unlawful contributions, gifts, entertainment or other unlawful expenses related to foreign or domestic political activity, (ii) made any unlawful payment to foreign or domestic government officials or employees or to any foreign or domestic political parties or campaigns from corporate funds, (iii) failed to disclose fully any contribution made by the Company (or made by any Person acting on its behalf of which the Company is aware) which is in violation of Law or (iv) violated in any material respect any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable non-U.S. anti-bribery Law.

U.S. Real Property Holding Corporation

. The Company is not and has never been a U.S. real property holding corporation within the meaning of Section 897 of the Internal Revenue Code of 1986, as amended.

4.24 Title to Assets. Except as set forth in the Company SEC Documents, the Company has good and marketable title to all real property and personal property owned by the Company that is material to the business of the Company taken as a whole, in each case free and clear of all Liens, except for Liens that do not materially affect the value of such property and do not materially interfere with the use made and proposed to be made of such property by the Company and Liens for the payment of federal, state or other taxes, the payment of which is neither delinquent nor subject to penalties. Any real property and facilities held under lease by the Company that are material to the business of the Company are held by the Company under valid, subsisting and enforceable leases with which the Company is in compliance.

4.25 Office of Foreign Assets Control. Neither the Company nor, to the Company's knowledge, any director, officer, agent, employee or Affiliate of the Company is currently subject to any U.S. sanctions administered by the Office of Foreign Assets Control of the U.S. Treasury Department.

4.26 Money Laundering. The operations of the Company are and have been conducted at all times in compliance with applicable financial record-keeping and reporting requirements of the Currency and Foreign Transactions Reporting Act of 1970, as amended, and applicable money laundering statutes and applicable rules and regulations thereunder and any related or similar rules, regulations or guidelines, issued, administered or enforced by any Governmental Authority (collectively, the "**Money Laundering Laws**"), and no action, suit or proceeding involving the Company with respect to the Money Laundering Laws is pending or, to the knowledge of the Company, threatened.

REPRESENTATIONS AND WARRANTIES OF THE INVESTOR

. The Investor hereby represents and warrants to the Company that:

Organization; Good Standing

. The Investor is a corporation duly organized, validly existing and in good standing under the laws of the State of Delaware. The

Investor has all requisite power and authority to enter into the Transaction Agreements, to purchase the Shares and to perform its obligations under and to carry out the other transactions contemplated by the Transaction Agreements.

Authorization

. All requisite action on the part of the Investor and its directors and stockholders required by applicable Law for the authorization, execution and delivery by the Investor of the Transaction Agreements and the performance of all of its obligations thereunder, including the subscription for and purchase of the Shares, has been taken. Each of the Transaction Agreements have been duly executed and delivered by the Investor and upon the due execution and delivery thereof by the Company, will constitute valid and legally binding obligations of the Investor, enforceable against the Investor in accordance with their respective terms (except as such enforceability may be limited by (a) applicable bankruptcy, insolvency, reorganization, moratorium or other Laws of general application relating to or affecting enforcement of creditors' rights and (b) rules of Law governing specific performance, injunctive relief or other equitable remedies and limitations of public policy).

No Conflicts

. The execution, delivery and performance of the Transaction Agreements and compliance with the provisions hereof and thereof by the Investor do not and shall not: (a) violate any provision of applicable Law or any ruling, writ, injunction, order, permit, judgment or decree of any Governmental Authority, (b) constitute a breach of, or default under (or an event which, with notice or lapse of time or both, would become a default under) or conflict with, or give rise to any right of termination, cancellation or acceleration of, any agreement, arrangement or instrument, whether written or oral, by which the Investor or any of its assets, are bound, or (c) violate or conflict with any of the provisions of the Investor's organizational documents (including any articles or memoranda of organization or association, charter, bylaws or similar documents), except, in the case of subsections (a) or (b), as would not materially impair the ability of the Investor to consummate the Transaction and perform its obligations under the Transaction Agreements.

No Governmental Authority or Third Party Consents

. No consent, approval, authorization or other order of any Governmental Authority or other Third Party is required to be obtained by the Investor in connection with the authorization, execution and delivery of any of the Transaction Agreements or with the subscription for and purchase of the Shares.

Purchase Entirely for Own Account

. The Shares shall be acquired for investment for the Investor's own account, not as a nominee or agent, and not with a view to the resale or distribution of any part thereof, and the Investor has no present intention of selling, granting any participation or otherwise distributing the Shares. The Investor does not have any contract, undertaking, agreement or arrangement with any Person to sell, transfer or grant participation to a Person any of the Shares.

Disclosure of Information

. The Investor has had the opportunity to review the Company SEC Documents and has received or has had full access to all the information from the Company and its management that the Investor considers necessary or appropriate for deciding whether to purchase the Shares hereunder. The Investor further represents that it has had an opportunity to ask questions and receive answers from the Company regarding the

Company, its financial condition, results of operations and prospects and the terms and conditions of the offering of the Shares sufficient to enable it to evaluate its investment.

Investment Experience and Accredited Investor Status

. The Investor is an "accredited investor" (as defined in Regulation D under the Securities Act). The Investor has such knowledge and experience in financial or business matters that it is capable of evaluating the merits and risks of the investment in the Shares to be purchased hereunder.

Acquiring Person

. As of the date of this Agreement and immediately prior to the Closing, neither the Investor nor any of its Affiliates beneficially owns, or will beneficially own (as determined pursuant to Rule 13d-3 under the Exchange Act without regard for the number of days in which a Person has the right to acquire such beneficial ownership, and without regard to the Investor's rights under this Agreement), any securities of the Company, except for securities that may be owned by an employee benefit plan of Investor or any mutual fund or similar investment entity in which Investor and its Affiliates own less than 5% in the aggregate, and over which neither the Investor nor its Affiliates exercise direct management or investment control.

Restricted Securities

. The Investor understands that the Shares, when issued, shall be "restricted securities" under the federal securities Laws inasmuch as they are being acquired from the Company in a transaction not involving a public offering and that under such Laws the Shares may be resold without registration under the Securities Act only in certain limited circumstances. The Investor represents that it is familiar with Rule 144 of the Securities Act, as presently in effect ("**Rule 144**").

Legends

. The Investor understands that the Shares, whether certificated or in book entry form, shall be subject to the following legends:

(a) "These securities have not been registered under the Securities Act of 1933. They may not be sold, offered for sale, pledged or hypothecated in the absence of a registration statement in effect with respect to the securities under the Securities Act or an opinion of counsel (which counsel shall be reasonably satisfactory to Solid Biosciences Inc.) that such registration is not required or unless sold pursuant to Rule 144 of the Securities Act."

(b) "These securities are subject to transfer restrictions set forth in an Investor Agreement by and between Solid Biosciences Inc. and Ultragenyx Pharmaceutical Inc., a copy of which is on file with the Secretary of Solid Biosciences Inc."

Financial Assurances

. The Investor has access to cash in an amount sufficient to pay to the Company the Aggregate Purchase Price.

Brokers

. There are no brokers, finders or financial advisory fees or commissions that will be payable by the Investor in respect of the transactions contemplated by this Agreement.

Additional Covenants and Agreements

.

Integration

. The Company shall not sell, offer for sale or solicit offers to buy or otherwise negotiate in respect of any security (as defined in the Securities Act) that would be integrated with the offer or sale of the Shares to be issued to the Investor hereunder for purposes of the rules and regulations of any of the markets or exchanges on which the Common Stock of the Company is listed or quoted for trading on the date in question, including but not limited to the Nasdaq Capital Market, the Nasdaq Global Market, or the Nasdaq Global Select Market.

Blue Sky Filings

. The Company shall take such action as the Company shall reasonably determine is necessary in order to obtain an exemption for, or to qualify the Shares for, sale to the Investor at the Closing under applicable securities or "Blue Sky" laws of the states of the United States, and shall provide evidence of such actions promptly upon request of the Investor.

Legend Removal

. After the expiration of the Lock-up Term (as defined in the Investor Agreement), the Company shall (i) cause the legends set forth in Section 5.10(a) and (b) to be removed from the Shares, no later than three (3) Business Days from receipt of a request from the Investor pursuant to this Section 6.3 and receipt from the Investor by the Company and its transfer agent of customary representations and other documentation reasonably requested by the Company and its transfer agent in connection therewith, if (a) the Shares have been or will be resold under an effective registration statement under the Securities Act, (b) the Shares have been or will be transferred in compliance with Rule 144 under the Securities Act, (c) the Shares are eligible for resale pursuant to Rule 144(b)(1)(i) under the Securities Act without the requirement for the Company to be in compliance with the current public information required under Rule 144 under the Securities Act as to such Shares and without volume or manner-of-sale restrictions or (d) the Investor shall have provided the Company with an opinion of counsel, reasonably satisfactory to the Company, stating that such securities may lawfully be transferred without registration under the Securities Act (assuming for this purpose that the Investor is not an affiliate of the Company) and (ii) cause its transfer agent to make a new, unlegended entry for such book entry Shares sold or disposed of without restrictive legends. The Company shall be responsible for the fees of its transfer agent and all Depository Trust Company fees associated with any such issuance.

6.4 Assistance and Cooperation. Following the Closing, each of the Parties agrees to use all reasonable efforts to take, or cause to be taken, all actions and to do, or cause to be done, and to assist and cooperate with the other Party in doing, all things necessary, proper or advisable to consummate and make effective, in the most expeditious manner practicable, the Transaction.

MISCELLANEOUS

.

Governing Law; Submission to Jurisdiction

. This Agreement shall be governed by and construed in accordance with the Laws of the State of Delaware, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. Any action brought, arising out of, or relating to this Agreement shall be

brought in the Court of Chancery of the State of Delaware. Each Party hereby irrevocably submits to the exclusive jurisdiction of said Court in respect of any claim relating to the validity, interpretation and enforcement of this Agreement, and hereby waives, and agrees not to assert, as a defense in any action, suit or proceeding in which any such claim is made that it is not subject thereto or that such action, suit or proceeding may not be brought or is not maintainable in such courts, or that the venue thereof may not be appropriate or that this agreement may not be enforced in or by such courts. The Parties hereby consent to and grant the Court of Chancery of the State of Delaware jurisdiction over such Parties and over the subject matter of any such claim. The Parties hereby consent to and agree that mailing of process or other papers in connection with any such action, suit or proceeding in the manner provided in Section 7.3 or in such other manner as may be permitted by law shall be valid and sufficient thereof.

Waiver

. Waiver by a Party of a breach hereunder by the other Party shall not be construed as a waiver of any subsequent breach of the same or any other provision. No delay or omission by a Party in exercising or availing itself of any right, power or privilege hereunder shall preclude the later exercise of any such right, power or privilege by such Party. No waiver shall be effective unless made in writing with specific reference to the relevant provision(s) of this Agreement and signed by a duly authorized representative of the Party granting the waiver.

Notices

. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant Party set forth on Exhibit C attached hereto and shall be (a) delivered personally, (b) sent by registered or certified mail, return receipt requested, postage prepaid, (c) sent via a reputable nationwide overnight courier service or (d) sent by electronic mail, with a confirmation copy to be sent by registered or certified mail, return receipt requested, postage prepaid. Any such notice, instruction or communication shall be deemed to have been delivered upon receipt if delivered by hand, three (3) Business Days after it is sent by registered or certified mail, return receipt requested, postage prepaid, one (1) Business Day after it is sent via a reputable nationwide overnight courier service or when transmitted with electronic confirmation of receipt, if transmitted by electronic mail (if such transmission is made during regular business hours of the recipient on a Business Day; or otherwise, on the next Business Day following such transmission). Either Party may change its address by giving notice to the other Party in the manner provided above.

Entire Agreement

. This Agreement, together with the Investor Agreement and the Collaboration and License Agreement, contain the entire agreement among the Parties with respect to the subject matter hereof and thereof and supersede all prior and contemporaneous arrangements or understandings, whether written or oral, with respect hereto and thereto.

Amendments

. No provision in this Agreement shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the Investor and the Company.

Headings; Nouns and Pronouns; Section References

. Headings in this Agreement are for convenience of reference only and shall not be considered in construing this

Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

Severability

. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the Parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of either Party and to match the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

Assignment

. Except for an assignment by the Investor of this Agreement or any rights hereunder to a subsidiary that is wholly-owned, directly or indirectly, by the Investor (which assignment will not relieve the Investor of any obligations hereunder), neither this Agreement nor any of the rights or obligations hereunder may be assigned by either the Investor or the Company without (a) the prior written consent of the Company in the case of any assignment by the Investor or (b) the prior written consent of the Investor in the case of an assignment by the Company.

Successors and Assigns

. This Agreement shall be binding upon and inure to the benefit of the Parties hereto and their respective successors and permitted assigns.

Counterparts

. This Agreement may be executed in counterparts, each of which shall be deemed an original but which together shall constitute one and the same instrument. Facsimile signatures and signatures transmitted via PDF shall be treated as original signatures.

Third Party Beneficiaries

. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party, including any creditor of any Party. No Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any Party.

No Strict Construction

. This Agreement has been prepared jointly and will not be construed against either Party.

Survival of Warranties

. The representations and warranties of the Company and the Investor contained in this Agreement shall survive the Closing, except for the representation and warranty of the Investor in Section 5.11, which shall not survive the Closing. The Parties hereby acknowledge and agree that the rights of the Parties hereunder are special, unique and of extraordinary character, and that if any Party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, such refusal or

failure would result in irreparable injury to the Company or the Investor as the case may be, the exact amount of which would be difficult to ascertain or estimate and the remedies at law for which would not be reasonable or adequate compensation. Accordingly, if any Party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, then, in addition to any other remedy which may be available to any damaged Party at law or in equity, such damaged Party will be entitled to seek specific performance and injunctive relief, without posting bond or other security, and without the necessity of proving actual or threatened damages, which remedy such damaged Party will be entitled to seek in any court of competent jurisdiction.

Remedies

. The rights, powers and remedies of the Parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such Parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a Party hereunder shall preclude any other or further assertion or exercise thereof.

Expenses

. Each Party shall pay its own fees and expenses in connection with the preparation, negotiation, execution and delivery of the Transaction Agreements.

(*Signature Page Follows*)

IN WITNESS WHEREOF, the Parties have executed and delivered this Agreement as of the Effective Date.

SOLID BIOSCIENCES INC.

By: /s/ Ilan Ganot
Name: Ilan Ganot
Title: Chief Executive Officer

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil Kakkis
Name: Emil Kakkis
Title: Chief Executive Officer

EXHIBIT A

FORM OF CROSS RECEIPT

CROSS RECEIPT

Solid Biosciences Inc. hereby acknowledges receipt from Ultragenyx Pharmaceutical Inc. on October 22, 2020 of $39,999,996.21, representing the purchase price for 7,825,797 shares of Common Stock, par value $0.001 per share, of Solid Biosciences Inc., pursuant to that certain Stock Purchase Agreement, dated as of October 22, 2020, by and between Solid Biosciences Inc. and Ultragenyx Pharmaceutical Inc.

SOLID BIOSCIENCES INC.

By:
Name:
Title:

Ultragenyx Pharmaceutical Inc. hereby acknowledges receipt from Solid Biosciences Inc. on October 22, 2020 of 7,825,797 shares of Common Stock, par value $0.001 per share, of Solid Biosciences Inc., delivered pursuant to that certain Stock Purchase Agreement, dated as of October 22, 2020, by and between Solid Biosciences Inc. and Ultragenyx Pharmaceutical Inc.

ULTRAGENYX PHARMACEUTICAL INC.

By:
Name: Emil Kakkis
Title: Chief Executive Officer

EXHIBIT B

FORM OF INVESTOR AGREEMENT

EXHIBIT C

NOTICES

(a) If to the Company:

Solid Biosciences Inc.
141 Portland Street, Fifth Floor
Cambridge, MA 02139
Attention: Lynette Herscha
Email:

with a copy to:

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Lia Der Marderosian
Email: lia.dermarderosian@wilmerhale.com

(b) If to the Investor:

Ultragenyx Pharmaceutical Inc.
60 Leveroni Court
Novato, CA 94949
Attention: Chief Business Officer; General Counsel
Email:

with a copy to:

Goodwin Procter LLP
100 Northern Avenue
Boston, MA 02210
Attention: Ed Amer
Email: EAmer@goodwinlaw.com

Exhibit 10.3

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed. Double asterisks denote omissions.

INVESTOR AGREEMENT

By and Between

SOLID BIOSCIENCES INC.

AND

ULTRAGENYX PHARMACEUTICAL INC.

Dated as of October 22, 2020

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TABLE OF CONTENTS

INVESTOR AGREEMENT

THIS INVESTOR AGREEMENT (this "**Agreement**") is made as of October 22, 2020, by and between Solid Biosciences Inc., a Delaware corporation (the "**Company**"), and Ultragenyx Pharmaceutical Inc., a Delaware corporation (the "**Investor**").

WHEREAS, the Stock Purchase Agreement, dated as of the date hereof, by and between the Investor and the Company (the "**Purchase Agreement**") provides for the issuance and sale by the Company to the Investor, and the purchase by the Investor, of shares (the "**Purchased Shares**") of the Company's common stock, par value $0.001 per share (the "**Common Stock**"); and

WHEREAS, as a condition to consummating the transactions contemplated by the Purchase Agreement, the Investor and the Company have agreed upon certain rights and restrictions as set forth herein with respect to the Purchased Shares and other securities of the Company beneficially owned by the Investor and its Affiliates, and it is a condition to the execution of the Purchase Agreement that this Agreement be executed and delivered by the Investor and the Company.

NOW, THEREFORE, in consideration of the premises and mutual agreements hereinafter set forth, and for other valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the parties hereto agree as follows:

Definitions

. As used in this Agreement, the following terms shall have the following meanings:

(a) "**Affiliate**" shall mean, with respect to any Person, another Person that controls, is controlled by or is under common control with such Person. A Person shall be deemed to control another Person if such Person possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of such Person, whether through the ownership of voting securities, by contract or otherwise. Without limiting the generality of the foregoing, a Person shall be deemed to "**control**" another Person if any of the following conditions is met: (i) in the case of corporate entities, direct or indirect ownership of more than fifty percent (50%) of the stock or shares having the right to vote for the election of directors; and (ii) in the case of non-corporate entities, direct or indirect ownership of more than fifty percent (50%) of the equity interest with the power to direct the management and policies of such non-corporate entities. For the purposes of this Agreement, in no event shall the Investor or any of its Affiliates be deemed Affiliates of the Company or any of its Affiliates, nor shall the Company or any of its Affiliates be deemed Affiliates of the Investor or any of its Affiliates.

(b) "**Agreement**" shall have the meaning set forth in the Preamble to this Agreement, including all Exhibits attached hereto.

(c) "**beneficial owner**," "**beneficially owns**," "**beneficial ownership**" and terms of similar import used in this Agreement shall, with respect to a Person, have the

meaning set forth in Rule 13d-3 under the Exchange Act (i) assuming the full conversion into, and exercise and exchange for, shares of Common Stock of all Common Stock Equivalents beneficially owned by such Person and (ii) determined without regard for the number of days in which such Person has the right to acquire such beneficial ownership.

(d) "**Business Day**" shall mean a day on which commercial banking institutions in New York, New York are open for business.

(e) "**Change of Control**" shall mean, with respect to a Party, (a) a merger or consolidation of such Party with a Third Party that results in the voting securities of such Party outstanding immediately prior thereto, or any securities into which such voting securities have been converted or exchanged, ceasing to represent more than fifty percent (50%) of the combined voting power of the surviving entity or the parent of the surviving entity immediately after such merger or consolidation, (b) a transaction or series of related transactions in which a Third Party, together with its Affiliates, becomes the beneficial owner of more than fifty percent (50%) of the combined voting power of the outstanding securities of such Party, or (c) the sale or other transfer to a Third Party of all or substantially all of such Party's business to which the subject matter of the Collaboration and License Agreement relates.

(f) "**Closing**" shall have the meaning set forth in the Purchase Agreement.

(g) "**Collaboration and License Agreement**" shall mean the Collaboration and License Agreement between the Company and the Investor, dated as of the date hereof.

(h) "**Common Stock**" shall have the meaning set forth in the Preamble to this Agreement.

(i) "**Common Stock Equivalents**" shall mean any options, warrants or other securities or rights convertible into or exercisable or exchangeable for, whether directly or following conversion into or exercise or exchange for other options, warrants or other securities or rights, shares of Common Stock.

(j) "**Company**" shall have the meaning set forth in the Preamble to this Agreement.

(k) "**Competitor**" shall mean any biopharmaceutical enterprise significantly involved in developing and commercializing therapies for the treatment of Duchenne muscular dystrophy, or any other Person that directly or indirectly beneficially owns a majority of the voting securities or voting interests in such an enterprise, or any direct or indirect majority-owned subsidiary of such an enterprise or of such a Person.

(l) "**Demand Request**" shall have the meaning set forth in Section 2.1.

(m) "**Disposition**" or "**Dispose of**" shall mean any (i) offer, pledge, sale, contract to sell, sale of any option or contract to purchase, purchase of any option or contract to sell, grant of any option, right or warrant for the sale of, or other disposition of or transfer of any shares of Common Stock, or any Common Stock Equivalents, including, without limitation, any "short sale" or similar arrangement, or (ii) swap or any other agreement or any transaction that transfers, in whole or in part, directly or indirectly, the economic consequence of ownership of shares of Common Stock or Common Stock Equivalents, whether any such swap or transaction is to be settled by delivery of securities, in cash or otherwise.

(n) "**Exchange Act**" shall mean the Securities Exchange Act of 1934, as amended, and the rules and regulations of the SEC promulgated thereunder.

(o) "**Filing Date**" shall mean (i) with respect to any Registration Statement to be filed on Form S-1 (or any applicable successor form), ninety (90) days after receipt by the Company of a Demand Request for such Registration Statement and (ii) with respect to any Registration Statement to be filed on Form S-3 (or any applicable successor form), thirty (30) days after receipt by the Company of a Demand Request for such Registration Statement.

(p) "**Governmental Authority**" shall mean any court, agency, authority, department, regulatory body or other instrumentality of any government or country or of any national, federal, state, provincial, regional, county, city or other political subdivision of any such government or country or any supranational organization of which any such country is a member.

(q) "**Group**" shall have the meaning set forth in Section 3.1.

(r) "**Holders**" shall mean (but, in each case, only for so long as such Person remains an Affiliate of the Investor) the Investor and any Permitted Transferee thereof, if any, in accordance with Section 2.13.

(s) "**Initiating Holder**" shall have the meaning set forth in Section 2.3.

(t) "**Interference**" shall have the meaning set forth in Section 2.6.

(u) "**Investor**" shall have the meaning set forth in the Preamble to this Agreement.

(v) "**Irrevocable Proxy**" shall have the meaning set forth in Section 5.1.

(w) "**Law**" or "**Laws**" shall mean all laws, statutes, rules, regulations, orders, judgments, injunctions and/or ordinances of any Governmental Authority.

(x) "**Lock-Up Securities**" shall have the meaning set forth in Section 4.1.

(y) "**Lock-Up Term**" shall mean from and after the Closing until the earlier of (i) 18 months after the date of the Closing and (ii) the termination of the Collaboration and License Agreement (other than pursuant to Section 13.2.1 of the Collaboration and License Agreement).

(z) "**Modified Clause**" shall have the meaning set forth in Section 8.7.

(aa) "**Other Holders**" shall mean any Person having rights to participate in a registration of the Company's securities.

(bb) "**Permitted Transferee**" shall mean (i) a controlled Affiliate of the Investor or (ii) a controlling Affiliate of the Investor (or any controlled Affiliate of such controlling Affiliate) or the acquiring Person in the case of a Change of Control of the Investor; provided, however, that no such Affiliate shall be deemed a Permitted Transferee for any purpose under this Agreement unless (A) the Permitted Transferee, prior to or simultaneously with such transfer or assignment, shall have agreed in writing to be subject to and bound by all restrictions and obligations set forth in this Agreement, and (B) the Investor shall, prior to such transfer, furnish to the Company written notice of the name and address of such Permitted Transferee, details of its status as a Permitted Transferee and details of the Registrable Securities with respect to which such registration rights are being assigned.

(cc) "**Permitted Transferee Irrevocable Proxy**" shall have the meaning set forth in Section 5.1.

(dd) "**Person**" shall mean any individual, limited liability company, partnership, firm, corporation, association, trust, unincorporated organization, government or any department or agency thereof or other entity, as well as any syndicate or group that would be deemed to be a Person under Section 13(d)(3) of the Exchange Act.

(ee) "**Prospectus**" shall mean the prospectus forming a part of any Registration Statement, as supplemented by any and all prospectus supplements and as amended by any and all amendments (including post-effective amendments) and including all material incorporated by reference or explicitly deemed to be incorporated by reference in such prospectus.

(ff) "**Purchase Agreement**" shall have the meaning set forth in the Preamble to this Agreement, and shall include all Exhibits attached thereto.

(gg) "**Purchased Shares**" shall have the meaning set forth in the Preamble to this Agreement, and shall be adjusted for (i) any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Common Stock issued as

(or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Purchased Shares.

(hh) "**registers**," "**registered**," and "**registration**" refer to a registration effected by preparing and filing a Registration Statement or similar document in compliance with the Securities Act, and the declaration or ordering of effectiveness of such Registration Statement or document by the SEC.

(ii) "**Registrable Securities**" shall mean (i) the Purchased Shares, together with any shares of Common Stock issued in respect thereof as a result of any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization and (ii) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the shares of Common Stock described in clause (i) of this definition, excluding in all cases, however, (A) any Registrable Securities if and after they have been transferred to a Permitted Transferee in a transaction in connection with which registration rights granted hereunder are not assigned or (B) any Registrable Securities sold to or through a broker or dealer or underwriter in a public distribution or a public securities transaction.

(jj) "**Registration Expenses**" shall mean all expenses incurred by the Company in connection with any Required Registration pursuant to Section 2.1 or the Company's compliance with Section 2.8, including, without limitation, all registration and filing fees, fees and expenses of compliance with securities or blue sky Laws (including reasonable fees and disbursements of counsel in connection with blue sky qualifications of any Registrable Securities), expenses of printing (i) certificates for any Registrable Securities in a form eligible for deposit with the Depository Trust Company or (ii) Prospectuses if the printing of Prospectuses is requested by Holders, messenger and delivery expenses, fees and disbursements of counsel for the Company and its independent certified public accountants (including the expenses of any management review, cold comfort letters or any special audits required by or incident to such performance and compliance), Securities Act liability insurance (if the Company elects to obtain such insurance), the reasonable fees and expenses of any special experts retained by the Company in connection with such registration, fees and expenses of other Persons retained by the Company and the reasonable fees and expenses (such fees and expenses not to exceed $50,000) of one (1) counsel for the Holders of Registrable Securities in each Required Registration, selected by the Holders of a majority of the Registrable Securities to be included in such Required Registration. In addition, the Company will pay its internal expenses (including, without limitation, all salaries and expenses of its officers and employees performing legal or accounting duties), the expense of any annual audit and the fees and expenses incurred in connection with the listing of the Registrable Securities to be registered on each securities exchange, if any, on which equity securities issued by the Company are then listed or the quotation of such securities on any national securities exchange on which equity securities issued by the Company are then quoted.

(kk) "**Registration Rights Term**" shall have the meaning set forth in Section 2.1.

(ll) "**Registration Statement**" shall mean any registration statement of the Company under the Securities Act that covers any of the Registrable Securities pursuant to the provisions of this Agreement, including the related Prospectus, all amendments and supplements to such registration statement (including post-effective amendments), and all exhibits and all materials incorporated by reference or explicitly deemed to be incorporated by reference in such Registration Statement.

(mm) "**Representative**" shall mean with, respect to any Person, such Person's directors, officers, employees, agents, attorneys, accountants, consultants and financial advisors.

(nn) "**Required Period**" with respect to a Required Registration shall mean the earlier of (i) the date on which all Registrable Securities covered by such Required Registration are sold pursuant thereto and (ii) one hundred twenty (120) days following the first day of effectiveness of the Registration Statement for such Required Registration, in each case subject to extension as set forth herein; provided, however, that in no event will the Required Period expire prior to the expiration of the applicable period referred to in Section 4(a)(3) of the Securities Act and Rule 174 promulgated thereunder.

(oo) "**Required Registration**" shall have the meaning set forth in Section 2.1.

(pp) "**SEC**" shall mean the United States Securities and Exchange Commission.

(qq) "**Securities Act**" shall mean the Securities Act of 1933, as amended, and the rules and regulations of the SEC promulgated thereunder.

(rr) "**Selling Expenses**" shall mean all underwriting discounts and selling commissions applicable to the sale of Registrable Securities pursuant to this Agreement.

(ss) "**Shares of Then Outstanding Common Stock**" shall mean, at any time, the issued and outstanding shares of Common Stock at such time, as well as all capital stock issued and outstanding at such time as a result of any stock split, stock dividend, or reclassification of Common Stock distributable, on a pro rata basis, to all holders of Common Stock.

(tt) "**Standstill Parties**" shall have the meaning set forth in Section 3.1.

(uu) "**Standstill Term**" shall have the meaning set forth in Section 3.1.

(vv) "**Third Party**" shall mean any Person other than the Investor, the Company or any of their respective Affiliates.

(ww) "**Underwritten Registration**" or "**Underwritten Offering**" shall mean a registration in which Registrable Securities are sold to an underwriter for reoffering to the public.

(xx) "**Violation**" shall have the meaning set forth in Section 2.11(a).

(yy) "**Voting Agreement Term**" shall have the meaning set forth in Section 5.1.

Registration Rights

. Effective as of the Closing:

2.1 Required Registration

. If, at any time after the expiration of the Lock-Up Term but no later than the tenth (10th) anniversary of the date hereof (the "**Registration Rights Term**"), the Company receives from any Holder or Holders a written request or requests (each, a "**Demand Request**") that the Company file a Registration Statement under the Securities Act to effect the registration (a "**Required Registration**") of Registrable Securities, the Company shall use all reasonable efforts to file a Registration Statement covering such Holders' Registrable Securities as soon as practicable (and by the applicable Filing Date) and shall use all reasonable efforts to, as soon as practicable thereafter, effect the registration of the Registrable Securities to permit or facilitate the sale and distribution (including, upon request of such Holder or Holders, in an Underwritten Offering) of all or such portion of such Holder's or Holders' Registrable Securities as are specified in such Demand Request, subject however, to the conditions and limitations set forth herein; provided, however, that the Company shall not be obligated to effect any registration of Registrable Securities upon receipt of a Demand Request pursuant to this Section 2.1 if:

(i) the Company has already completed two (2) Required Registrations;

(ii) in the event that the market value of the Registrable Securities proposed to be included in the registration, based on the average closing price during the ten (10) consecutive trading days period prior to the making of the Demand Request, is less than the lesser of (x) ten million dollars ($10,000,000) or (y) the total market value of Registrable Securities outstanding;

(iii) the Company furnishes to the Holders a certificate signed by an authorized officer of the Company stating that (A) within sixty (60) days after receipt of the Demand Request under this Section 2.1, the Company expects to file a registration statement for the public offering of securities for the account of the Company (other than a registration of securities (x) issuable pursuant to an employee stock option, stock purchase or similar plan, (y) issuable pursuant to a merger, exchange offer or a transaction of the type specified in Rule 145(a) under the Securities Act or (z) in which

the only securities being registered are securities issuable upon conversion of debt securities which are also being registered), provided, that the Company is actively employing good faith efforts to cause such registration statement to become effective or (B) the Company is engaged in a material transaction or has an undisclosed material corporate development, in either case, which would be required to be disclosed in the Registration Statement, and in the good faith judgment of the Company's Board of Directors, such disclosure would be materially detrimental to the Company and its stockholders at such time (in which case, the Company shall disclose the matter as promptly as reasonably practicable and thereafter file the Registration Statement, and each Holder agrees not to disclose any information about such material transaction or corporate development to Third Parties until such disclosure has occurred or such information has entered the public domain other than through breach of this provision by such Holder), provided, however, that the Company shall have the right to only defer the filing of the Registration Statement pursuant to this subsection once in any twelve (12) month period and, such deferral may not exceed a period of more than ninety (90) days after receipt of a Demand Request;

(iv) the Company has, within the twelve (12) month period preceding the date of the Demand Request, already effected one (1) Required Registration for any Holder pursuant to this Section 2.1; or

(v) at any time during the period between the Company's receipt of the Demand Request and the completion of the Required Registration, any Holder is in breach of or has failed to cause its Affiliates to comply with the obligations and restrictions of Section 3 of this Agreement, the Company has provided notice of such breach to a Holder and such breach or failure is ongoing and has not been remedied; it being understood that a one-time, inadvertent and de minimis breach of Section 4 shall not be deemed to be a breach of the obligations and restrictions under Section 4 for purposes of this Section 2.1(v).

2.2 Company Registration

. Effective from the expiration of the Lock-Up Term until the earlier of (a) the tenth (10th) anniversary of the date hereof and (b) the date on which the Holders no longer beneficially own at least one percent (1%) of the Shares of Then Outstanding Common Stock, (provided, however, if the Holders reacquire beneficial ownership representing at least one percent (1%) of the Shares of Then Outstanding Common Stock at any time within ten (10) year period set forth in clause (a) of this Section 2.2, the provisions of this Section 2.2 shall automatically again become applicable to the Holders) the Company shall notify the Holders in writing at least ten (10) days prior to the filing of any Registration Statement including shares of Common Stock by one or more selling stockholders (other than the Holders) ("**Registration Notice**") and will afford each Holder an opportunity, subject to the terms and conditions of this Agreement, to include in such Registration Statement the number of Registrable Securities then held by such Holder that such Holder wishes to include in such Registration Statement. Each Holder desiring to include in any such Registration Statement all or any part of the Registrable Securities held by such Holder shall, within five (5) days after

receipt of the Registration Notice, so notify the Company in writing, and in such notification, inform the Company of the number of Registrable Securities such Holder wishes to include in such Registration Statement. If a Holder decides not to include Registrable Securities in any Registration Statement thereafter filed by the Company, such Holder shall nevertheless continue to have the right to include Registrable Securities in any subsequent Registration Statement or Registration Statements as may be filed by the Company with respect to offerings of its securities, all upon the terms and conditions set forth herein. Each Holder shall keep confidential and not disclose to any third party (i) its receipt of any Registration Notice and (ii) any information regarding the proposed offering as to which such notice is delivered, except as required by law, regulation or as compelled by subpoena. If a registration pursuant to this Section 2.2 is an Underwritten Offering, the right of any such Holder to include Registrable Securities in a registration statement pursuant to this Section 2.2 shall be conditioned upon such Holder's participation in such underwriting and the inclusion of such Holder's Registrable Securities in the underwriting to the extent provided herein. The Company and all Holders proposing to distribute their Registrable Securities through such underwriting shall enter into an underwriting agreement in customary form with the managing underwriter or underwriters selected for such underwriting. Notwithstanding any other provision of this Section 2, if the managing underwriter for the Underwritten Offering determines in good faith that marketing factors require a limitation of the number of shares of Registrable Securities to be included in such Underwritten Offering and advises the Holders of such determination in writing, then the managing underwriter may exclude shares (including up to 100% of the Registrable Securities) from the registration and the underwriting, with the number of Registrable Securities, if any, included in the registration and the underwriting being allocated to each of the Holders requesting inclusion of their Registrable Securities in such Registration Statement and all other Persons selling shares of Common Stock pursuant to such Registration Statement on a pro rata basis based on the total number of shares of Common Stock then held by each such Holder or other stockholder. Notwithstanding the foregoing, the Company shall have the right to terminate or withdraw any registration initiated by it under this Section 2.2 prior to the effectiveness of such registration whether or not any Holder has elected to include securities in such registration.

2.3 Underwritten Registration Required; Priority in Underwritten Offering

. The underwriter for any Underwritten Offering requested pursuant to Section 2.1 shall be selected by the Company, subject to approval by a majority in interest of the Holders initiating the Required Registration hereunder (such Holder(s) initiating the registration request, the "**Initiating Holders**"), which approval shall not be unreasonably withheld, conditioned or delayed. The right of any Holder to include its Registrable Securities in the Underwritten Offering shall be conditioned upon such Holder's participation in such Underwritten Offering and the inclusion of such Holder's Registrable Securities to the extent provided herein. All Holders requesting the inclusion of their Registrable Securities in such Underwritten Offering shall (together with the Company as provided in Section 2.8(h)) enter into an underwriting agreement in customary form with the underwriter or underwriters selected for such Underwritten Offering. Notwithstanding any other provision of this Section 2, if the managing underwriter for the Underwritten Offering determines in good faith that marketing factors require a limitation of the number of shares of Registrable Securities to be included in such Underwritten

Offering, and advises the Holders of such determination in writing, then the Company shall so advise all Holders which requested inclusion of their Registrable Securities in such Underwritten Offering, and the number of shares of Registrable Securities that may be included in such Underwritten Offering shall be allocated among the Holders in proportion (as nearly as practicable) to the amount of Registrable Securities of the Company owned by each Holder; provided, however, that the number of shares of Registrable Securities to be included in such Underwritten Offering shall not be reduced unless all other securities are first entirely excluded from such Underwritten Offering. In the event the Company advises the Holders of its intent to decrease the total number of Registrable Securities that may be included by the Holders in such Required Registration such that the number of Registrable Securities included in such Required Registration would be less than seventy-five percent (75%) of all Registrable Securities which the Holders requested be included in such Required Registration, then Holders representing a majority of the Registrable Securities requested to be included in such Required Registration will have the right to withdraw, on behalf of all Holders of all Registrable Securities requested to be so included, such Required Registration, in which case, such Required Registration will not count as a Required Registration for the purposes of Section 2.1(i), and the Company shall bear all Registration Expenses in connection therewith; provided, that, the right to withdraw a registration and have it not count as a Required Registration may only be exercised once by the Holders (taken collectively).

2.4 Priority in Required Registration

. With respect to any Required Registration of Registrable Securities requested pursuant to Section 2.1, the Company may also (i) propose to sell shares of Common Stock on its own behalf and (ii) provide written notice of such Required Registration to Other Holders and permit all such Other Holders who request to be included in the Required Registration to include any or all Company securities held by such Other Holders in such Required Registration on the same terms and conditions as the Registrable Securities. Notwithstanding the foregoing, if the managing underwriter or underwriters of the Underwritten Offering to which any Required Registration relates advise the Company in writing and advises the Holders of Registrable Securities of such determination in writing, that, in its good faith determination, the total amount of securities that such Holders, Other Holders, and the Company intend to include in such Required Registration is in an amount in the aggregate which would adversely affect the success of such Underwritten Offering, then such Required Registration shall include (i) first, all Registrable Securities of the Holders allocated, if the amount is less than all the Registrable Securities requested to be sold, *pro rata* on the basis of the total number of Registrable Securities held by such Holders; and (ii) second, as many other securities proposed to be included in the Required Registration by the Company and any Other Holders, allocated *pro rata* among the Company and such Other Holders, on the basis of the amount of securities requested to be included therein by the Company and each such Other Holder so that the total amount of securities to be included in such Underwritten Offering is the full amount that, in the written opinion of such managing underwriter, can be sold without materially and adversely affecting the success of such Underwritten Offering.

2.5 Revocation of Required Registration

. With respect to one (1) Required Registration only, the Holders of at least a majority of the Registrable Securities to be included

in a Registration Statement with respect to such Required Registration may, at any time prior to the effective date of such Registration Statement, on behalf of all Holders of all Registrable Securities requested to be included therein, revoke the request to have Registrable Securities included therein and revoke the request for such Required Registration by providing a written notice to the Company, in which case such Required Registration that has been revoked will be deemed not to have been effected and will not count as a Required Registration for purposes of Section 2.1(i) if, and only if, the Holders of Registrable Securities which had requested inclusion of Registrable Securities in such Required Registration promptly reimburse the Company for all Registration Expenses incurred by the Company in connection with such Required Registration. Notwithstanding the foregoing sentence, the parties agree and acknowledge that the Holders of a majority of the Registrable Securities requested to be included in such Required Registration may revoke any Required Registration (without any obligation to reimburse the Company for Registration Expenses incurred in connection therewith) if such revocation is based on (i) a material adverse change in circumstances with respect to the Company and its subsidiaries, taken as a whole, caused by an act or failure to act by the Company or any of its subsidiaries and not known to any Holder at the time the Required Registration was first made or (ii) the Company's failure to comply in any material respect with its obligations hereunder, and any such revocation based on an event described in (i) or (ii) above shall be exercisable at any time and shall not be counted as the one (1) revocation of a Required Registration permitted by the first sentence of this Section 2.5.

2.6 Effective Required Registrations

. A Required Registration will not be deemed to be effected for purposes of Section 2.1(i) if the Registration Statement for such Required Registration has (a) not been declared effective by the SEC or (b) become effective in accordance with the Securities Act and the rules and regulations thereunder and not been kept effective for the Required Period. In addition, if after such Registration Statement has been declared or becomes effective, (i) the offering of Registrable Securities pursuant to such Registration Statement is interfered with by any stop order, injunction, or other order or requirement of the SEC or other governmental agency or court such that the continued offer and sale of Registrable Securities being offered pursuant to such Registration Statement would violate applicable Law and such stop order, injunction or other order or requirement of the SEC or other governmental agency or court does not result from any act or omission of any Holder whose Registrable Securities are registered pursuant to such Registration Statement (an "**Interference**") and (ii) any such Interference is not cured within ninety (90) days thereof, such Required Registration will be deemed not to have been effected and will not count as a Required Registration. In the event such Interference occurs and is cured, the Required Period relating to such Registration Statement will be extended by the number of days of such Interference, including the date such Interference is cured.

2.7 Continuous Effectiveness of Registration Statement

. The Company will use all reasonable efforts to cause each Registration Statement filed pursuant to this Section 2 to be declared effective by the SEC or to become effective under the Securities Act as promptly as practicable and to keep each such Registration Statement that has been declared or becomes effective continuously effective for the Required Period.

2.8 Obligations of the Company

. Whenever required under Section 2.1 to effect the registration of any Registrable Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a Registration Statement with respect to such Registrable Securities sought to be included therein; provided that at least five (5) Business Days prior to filing any Registration Statement or Prospectus or any amendments or supplements thereto, the Company shall furnish to the Holders of the Registrable Securities covered by such Registration Statement, their counsel and the managing underwriter copies of all such documents proposed to be filed, and any such Holder shall have the opportunity to comment on any information pertaining solely to such Holder and its plan of distribution that is contained therein and the Company shall make the corrections reasonably requested by such Holder or the managing underwriter with respect to such information prior to filing any such Registration Statement or amendment;

(b) prepare and file with the SEC such amendments and post-effective amendments to any Registration Statement and any Prospectus used in connection therewith as may be necessary to keep such Registration Statement effective for the Required Period, and cause the Prospectus to be supplemented by any required prospectus supplement, and as so supplemented to be filed pursuant to Rule 424 under the Securities Act, to comply with the provisions of the Securities Act with respect to the disposition of all Registrable Securities covered by such registration statement for the Required Period; provided that at least five (5) Business Days prior to filing any such amendments and post effective amendments or supplements thereto, the Company shall furnish to the Holders of the Registrable Securities covered by such Registration Statement, their counsel and the managing underwriter copies of all such documents proposed to be filed, and any such Holder or managing underwriter shall have the opportunity to comment on any information pertaining solely to such Holder and its plan of distribution that is contained therein and the Company shall make the corrections reasonably requested by such Holder and the managing underwriter with respect to such information prior to filing any such Registration Statement or amendment;

(c) furnish to the Holders of Registrable Securities covered by such Registration Statement and the managing underwriter such numbers of copies of such Registration Statement, each amendment and supplement thereto, the Prospectus included in such Registration Statement (including each preliminary prospectus or free writing prospectus) in conformity with the requirements of the Securities Act, and such other documents as they may reasonably request in order to facilitate the disposition of Registrable Securities owned by them;

(d) notify the Holders of Registrable Securities covered by such Registration Statement, promptly after the Company shall receive notice thereof, of the time when such Registration Statement becomes or is declared effective or when any amendment or supplement or any Prospectus forming a part of such Registration Statement has been filed;

(e) notify the Holders of Registrable Securities covered by such Registration Statement promptly of any request by the SEC for the amending or supplementing of such Registration Statement or Prospectus or for additional information and promptly deliver to such Holders copies of any comments received from the SEC;

(f) notify the Holders promptly of any stop order suspending the effectiveness of such Registration Statement or Prospectus or the initiation of any proceedings for that purpose, and use all reasonable efforts to obtain the withdrawal of any such order or the termination of such proceedings;

(g) use all reasonable efforts to register and qualify the Registrable Securities covered by such Registration Statement under such other securities or blue sky Laws of such jurisdictions as shall be reasonably requested by the Holders, use all reasonable efforts to keep each such registration or qualification effective, including through new filings, or amendments or renewals, during the Required Period, and notify the Holders of Registrable Securities covered by such Registration Statement of the receipt of any written notification with respect to any suspension of any such qualification; provided, however, that the Company shall not be required in connection therewith or as a condition thereto to qualify to do business or to file a general consent to service of process in any such states or jurisdictions, except as may be required by the Securities Act;

(h) in the event of any Underwritten Offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the managing underwriter of the Underwritten Offering pursuant to which such Registrable Securities are being offered;

(i) use all reasonable efforts to obtain: (A) at the time of effectiveness of the Registration Statement covering such Registrable Securities, a "cold comfort letter" from the Company's independent certified public accountants covering such matters of the type customarily covered by "cold comfort letters" as the underwriters may reasonably request; and (B) at the time of any underwritten sale pursuant to such Registration Statement, a "bring-down comfort letter," dated as of the date of such sale, from the Company's independent certified public accountants covering such matters of the type customarily covered by "bring-down comfort letters" as the underwriters may reasonably request;

(j) promptly notify each Holder of Registrable Securities covered by such Registration Statement at any time when a Prospectus relating thereto is required to be delivered under the Securities Act of the happening of any event as a result of which the Prospectus included in such Registration Statement or any offering memorandum or other offering document includes an untrue statement of a material fact or omits to state any material fact required to be stated therein or necessary to make the statements therein not misleading in the light of the circumstances then existing, and promptly prepare a supplement or amendment to such Prospectus or file any other required document so that, as thereafter delivered to the

purchasers of such Registrable Securities, such Prospectus will not contain an untrue statement of material fact or omit to state any fact necessary to make the statements therein not misleading;

(k) permit any Holder of Registrable Securities covered by such Registration Statement, which Holder in its reasonable judgment could reasonably be deemed to be an underwriter with respect to the Underwritten Offering pursuant to which such Registrable Securities are being offered, or to be a controlling Person of the Company, to reasonably participate in the preparation of such Registration Statement and to require the insertion therein of information to the extent concerning such Holder, furnished to the Company in writing, which in the reasonable judgment of such Holder and its counsel should be included;

(l) in connection with any Underwritten Offering, use all reasonable efforts to obtain an opinion or opinions addressed to the underwriter or underwriters in customary form and scope from counsel for the Company;

(m) upon reasonable notice and during normal business hours, subject to the Company receiving customary confidentiality undertakings or agreements from any Holder of Registrable Securities covered by such Registration Statement or other person obtaining access to Company records, documents, properties or other information pursuant to this subsection (m), make available for inspection by a representative of such Holder and any underwriter participating in any disposition of such Registrable Securities and any attorneys or accountants retained by any such Holder or underwriter, relevant financial and other records, pertinent corporate documents and properties of the Company, and use all reasonable efforts to cause the officers, directors and employees of the Company to supply all information reasonably requested by any such representative, underwriter, attorneys or accountants in connection with the Registration Statement;

(n) with respect to one (1) Required Registration which includes Registrable Securities the market value of which is at least one hundred million dollars ($100,000,000), participate, to the extent requested by the managing underwriter, in efforts extending for no more than three (3) days scheduled by such managing underwriter and reasonably acceptable to the Company's senior management, to sell the Registrable Securities being offered pursuant to such Required Registration (including participating during such period in customary "roadshow" meetings with prospective investors);

(o) use all reasonable efforts to comply with all applicable rules and regulations of the SEC relating to such registration and make generally available to its security holders earning statements satisfying the provisions of Section 11(a) of the Securities Act, provided that the Company will be deemed to have complied with this Section 2.8(o) with respect to such earning statements if it has satisfied the provisions of Rule 158;

(p) if requested by the managing underwriter or any selling Holder, promptly incorporate in a prospectus supplement or post-effective amendment such information as the managing underwriter or any selling Holder reasonably requests to be included therein,

with respect to the Registrable Securities being sold by such selling Holder, including, without limitation, the purchase price being paid therefor by the underwriters and with respect to any other terms of the Underwritten Offering of Registrable Securities to be sold in such offering, and promptly make all required filings of such prospectus supplement or post-effective amendment;

(q) cause the Registrable Securities covered by such Registration Statement to be listed on each securities exchange, if any, on which equity securities issued by the Company are then listed; and

(r) reasonably cooperate with each selling Holder and each underwriter participating in the disposition of such Registrable Securities and their respective counsel in connection with filings required to be made with the Financial Industry Regulatory Authority, Inc., if any.

2.9 Furnish Information

. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 2 with respect to the Registrable Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself and the Registrable Securities held by it as shall be reasonably necessary to effect the registration of such Holder's Registrable Securities.

2.10 Expenses

. Except as specifically provided herein, all Registration Expenses shall be borne by the Company. All Selling Expenses incurred in connection with any registration hereunder shall be borne by the Holders of Registrable Securities covered by a Registration Statement, pro rata on the basis of the number of Registrable Securities registered on their behalf in such Registration Statement.

2.11 Indemnification

. In the event any Registrable Securities are included in a Registration Statement under this Agreement:

(a) The Company shall indemnify and hold harmless each Holder including Registrable Securities in any such Registration Statement, any underwriter (as defined in the Securities Act) for such Holder and each Person, if any, who controls such Holder or underwriter within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act and the officers, directors, owners, agents and employees of such controlling Persons, against any and all losses, claims, damages or liabilities (joint or several) to which they may become subject under any securities Laws including, without limitation, the Securities Act, the Exchange Act, or any other statute or common law of the United States or any other country or political subdivision thereof, or otherwise, including the amount paid in settlement of any litigation commenced or threatened (including any amounts paid pursuant to or in settlement of claims made under the indemnification or contribution provisions of any underwriting or similar agreement entered into by such Holder in connection with any offering or sale of securities covered by this Agreement), and shall promptly reimburse them, as and when incurred, for any legal or other expenses incurred by them in connection with investigating any claims and

defending any actions, insofar as any such losses, claims, damages or liabilities (or actions in respect thereof) arise out of or are based upon any of the following statements, omissions or violations (each, a "**Violation**"): (i) any untrue statement or alleged untrue statement of a material fact contained in or incorporated by reference into such Registration Statement, including any preliminary prospectus or final prospectus contained therein or any free writing prospectus or any amendments or supplements thereto, or in any offering memorandum or other offering document relating to the offering and sale of such securities, (ii) the omission or alleged omission to state therein a material fact required to be stated therein, or necessary to make the statements therein not misleading or (iii) any violation or alleged violation by the Company (or any of its agents or Affiliates) of the Securities Act, the Exchange Act, any state securities Law, or any rule or regulation promulgated under any state securities Law, in each case arising from such Registration Statement; provided, however, the Company shall not be liable in any such case for any such loss, claim, damage, liability or action to the extent that it (A) arises out of or is based upon a Violation which occurs solely in reliance upon and in conformity with written information furnished expressly for use in connection with such registration by such Holder; or (B) is caused by such Holder's disposition of Registrable Securities during any period during which such Holder is obligated to discontinue any disposition of Registrable Securities as a result of any stop order suspending the effectiveness of any registration statement or prospectus with respect to Registrable Securities. The Company shall pay, as incurred, any legal or other expenses reasonably incurred by any Person intended to be indemnified pursuant to this Section 2.11(a), in connection with investigating or defending any such loss, claim, damage, liability or action; provided, however, that the indemnity agreement contained in this Section 2.11(a) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without consent of the Company, which consent shall not be unreasonably withheld, conditioned or delayed.

(b) Each Holder including Registrable Securities in a registration statement shall indemnify and hold harmless the Company, each of its directors, each of its officers who has signed the registration statement, each Person, if any, who controls the Company within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act and the officers, directors, owners, agents and employees of such controlling Persons, any underwriter, any other Holder selling securities in such registration statement and any controlling Person of any such underwriter or other Holder, against any losses, claims, damages or liabilities (joint or several) to which any of the foregoing Persons may become subject, under liabilities (or actions in respect thereto) which arise out of or are based upon any Violation, in each case to the extent (and only to the extent) that such Violation: (i) arises out of or is based upon a Violation which occurs solely in reliance upon and in conformity with written information furnished expressly for use in connection with such registration by such Holder; or (ii) is caused by such Holder's disposition of Registrable Securities during any period during which such Holder is obligated to discontinue any disposition of Registrable Securities as a result of any stop order suspending the effectiveness of any registration statement or prospectus with respect to Registrable Securities of which such Holder has received written notice. Each such Holder shall pay, as incurred, any legal or other expenses reasonably incurred by any Person intended to be indemnified pursuant to this Section 2.11(b), in connection with investigating or defending any

such loss, claim, damage, liability or action; provided, however, that the indemnity agreement contained in this Section 2.11(b) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability or action if such settlement is effected without consent of the Holder, which consent shall not be unreasonably withheld.

(c) Promptly after receipt by an indemnified party under this Section 2.11 of notice of the commencement of any action (including any action by a Governmental Authority), such indemnified party shall, if a claim in respect thereof is to be made against any indemnifying party under this Section 2.11, deliver to the indemnifying party a written notice of the commencement thereof and the indemnifying party shall have the right to participate in, and, to the extent the indemnifying party so desires, jointly with any other indemnifying party similarly noticed, to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain its own counsel, with the reasonable fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such proceeding. The failure to deliver written notice to the indemnifying party within a reasonable time of the commencement of any such action, if prejudicial to its ability to defend such action, shall relieve such indemnifying party of any liability to the indemnified party under this Section 2.11, but the omission so to deliver written notice to the indemnifying party shall not relieve it of any liability that it may have to any indemnified party otherwise than under this Section 2.11.

(d) In order to provide for just and equitable contribution to joint liability in any case in which a claim for indemnification is made pursuant to this Section 2.11 but it is judicially determined (by the entry of a final judgment or decree by a court of competent jurisdiction and the expiration of time to appeal or the denial of the last right of appeal) that such indemnification may not be enforced in such case notwithstanding the fact that this Section 2.11 provided for indemnification in such case, the Company and each Holder of Registrable Securities shall contribute to the aggregate losses, claims, damages or liabilities to which they may be subject (after contribution from others) in proportion to the relative fault of the Company, on the one hand, and such Holder, severally, on the other hand; provided, however, that in any such case, no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any Person who was not guilty of such fraudulent misrepresentation; provided further, however, that in no event shall any contribution under this Section 2.11(d) on the part of any Holder exceed the net proceeds received by such Holder from the sale of Registrable Securities giving rise to such contribution obligation, except in the case of willful misconduct or fraud by such Holder.

(e) The obligations of the Company and the Holders under this Section 2.11 shall survive the completion of any offering of Registrable Securities in a registration statement under this Agreement and otherwise.

2.12 SEC Reports

. With a view to making available to the Holders the benefits of Rule 144 under the Securities Act and any other rule or regulation of the SEC that may at any time permit a Holder to sell Registrable Securities of the Company to the public without registration, the Company agrees to at any time that it is a reporting company under Section 13 or 15(d) of the Exchange Act:

(a) make and keep available adequate current public information, as those terms are understood and defined in Rule 144; and

(b) furnish to any Holder, so long as such Holder owns any Registrable Securities, forthwith upon request (i) a written statement by the Company that it has complied with the reporting requirements of Rule 144, (ii) a copy of the most recent annual or quarterly report of the Company and such other reports and documents so filed by the Company, and (iii) such other information as may be reasonably requested in availing any Holder of any rule or regulation of the SEC (exclusive of Rule 144A) which permits the selling of any Registrable Securities without registration.

2.13 Assignment of Registration Rights

. The rights to cause the Company to register any Registrable Securities pursuant to this Agreement may be assigned in whole or in part (but only with all restrictions and obligations set forth in this Agreement) by a Holder to a Permitted Transferee which acquires at least 1,000,000 Registrable Securities (subject to adjustment in the event of any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization) from such Holder; provided, however, (a) such Holder shall, within five (5) days prior to such transfer, furnish to the Company written notice of the name and address of such Permitted Transferee, details of its status as a Permitted Transferee and details of the Registrable Securities with respect to which such registration rights are being assigned, (b) the Permitted Transferee, prior to or simultaneously with such transfer or assignment, shall agree in writing to be subject to and bound by all restrictions and obligations set forth in this Agreement, (c) the Investor shall continue to be bound by all restrictions and obligations set forth in this Agreement and (d) such transfer or assignment shall be effective only if immediately following such transfer or assignment the further disposition of such Registrable Securities by the Permitted Transferee is restricted under the Securities Act and other applicable securities Law.

3. Standstill.

3.1 During the period from and after the Closing until 24 months after the date of the Closing (such period, the "**Standstill Term**"), neither the Investor nor any of its controlled Affiliates, nor any other Affiliates of the Investor acting at the direction of the Investor, nor any Representatives of the Investor acting at its direction (collectively, the "**Standstill Parties**") shall (and the Investor shall cause its controlled Affiliates and Representatives not to) in any manner, directly or indirectly, except as expressly approved or invited in advance in writing by the Company:

(a) effect or seek, offer or propose (whether publicly or otherwise) to effect, or cause or participate in or in any way advise, assist or knowingly encourage any other person to effect or seek, offer or propose (whether publicly or otherwise) to effect or participate in, (i) any acquisition of any voting securities (or beneficial ownership thereof) or assets of the Company, or any rights to acquire any such voting securities (including derivate securities representing the right to vote or economic benefit of any such securities) or assets; (ii) any tender or exchange offer, merger or other business combination involving the Company; (iii) any recapitalization, restructuring, liquidation, dissolution or other extraordinary transaction with respect to the Company; or (iv) any "solicitation" of "proxies" (as such terms are used in the proxy rules of the SEC) or consents to vote any voting securities of the Company;

(b) form, join or in any way participate in a "group" (as defined under the Exchange Act, a "**Group**") with respect to any securities of the Company;

(c) otherwise act, alone or in concert with others, to seek to control or influence the management, Board of Directors or policies of the Company;

(d) take any action which would reasonably be expected to legally require the Company to make a public announcement regarding any of the types of matters set forth in clause (a) above; or

(e) enter into any discussions or arrangements with any third party with respect to any of the foregoing.

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Notwithstanding anything in this Agreement to the contrary, nothing herein shall prevent the Investor from communicating to the Chief Executive Officer of the Company or the Chair of its Board of Directors in a non-public manner at any time a proposal or offer for, or a request for discussions (including further discussions) regarding, any transaction or activity otherwise prohibited under clause (i) or (ii) of Section 3.1(a), provided that such communication would not reasonably be expected to legally require the Company to make any public announcement or disclosure regarding the existence or receipt of such proposal or the terms or conditions thereof.

Restrictions on Dispositions

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4.1 Lock-Up

. During the Lock-Up Term , without the prior approval of the Company, the Investor shall not, and shall cause its Affiliates not to, Dispose of (x) any of the Purchased Shares or any shares of Common Stock beneficially owned by the Investor and its Affiliates as of the date of this Agreement, together with any shares of Common Stock issued in respect thereof as a result of any stock split, stock dividend, share exchange, merger, consolidation or similar recapitalization, and (y) any Common Stock issued as (or issuable upon the exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange or in replacement of, the Purchased Shares or shares of Common Stock described in clause (x) of this sentence (collectively, the "**Lock-Up**

Securities"); provided, however, that the foregoing shall not prohibit the Investor from (A) transferring any of the Lock-Up Securities to a Permitted Transferee in accordance with and subject to the terms of Section 2.13 and (B) Disposing of any of the Lock-Up Securities in order to reduce the beneficial ownership of the Investor and its Affiliates to 19.9% or such lesser percentage, as advised in good faith and in writing by the Investor's certified public accountants, that would be necessary pursuant to applicable accounting rules and guidelines so as to not require the Investor to include in its financial statements its portion of the Company's financial results, of the Shares of Then Outstanding Common Stock.

4.2 Certain Tender Offers

. Notwithstanding any other provision of this Section 4, this Section 4 shall not prohibit or restrict any Disposition of shares of Common Stock and/or Common Stock Equivalents by the Investor or its Affiliates into (a) a tender offer by a Third Party which is not opposed by the Company's Board of Directors (but only after the Company's filing of a Schedule 14D-9, or any amendment thereto, with the SEC disclosing the recommendation of the Company's Board of Directors with respect to such tender offer) or (b) an issuer tender offer by the Company.

4.3 Sale Limitations. Subject to the restrictions set forth in Section 4.1, the Investor agrees that, except for any transfer of Shares of Then Outstanding Common Stock and/or Common Stock Equivalents by the Investor to a Permitted Transferee or the Company, it shall not, and shall cause its Affiliates not to, Dispose of any Shares of Then Outstanding Common Stock and/or Common Stock Equivalents (for the avoidance of doubt, other than as a result of a Change of Control of the Investor) at any time to any Person that such Investor or Affiliate knows (after a reasonable inquiry in a private placement) is a Competitor.

4.4 Offering Lock-Up

. If the Holders together beneficially own at least five percent (5%) of the Shares of Then Outstanding Common Stock, the Holders shall, if requested by the Company and an underwriter of Common Stock of the Company, agree not to Dispose of any shares of Common Stock and/or Common Stock Equivalents for a specified period of time, such period of time not to exceed ninety (90) days. Such agreement shall be in writing in a form satisfactory to the Company, the underwriter(s) in such offering and shall contain customary exceptions to the restrictions set forth therein. The Company may impose stop transfer instructions with respect to the shares of Common Stock and/or Common Stock Equivalents to the extent consistent with any such agreement until the end of the specified period of time. The foregoing provisions of this Section 4.4 shall apply to the Holders only if the Company's directors, officers and any holders of an equal or greater number of shares of Common Stock that are party to a collaboration, license or similar agreement with the Company are subject to similar lock-up restrictions. Any discretionary waiver or termination of the restrictions of any or all of such agreements by the Company or the underwriters shall apply pro rata to all Holders subject to such agreements, based on the number of shares subject to such agreements.

5. Voting Agreement.

5.1 Voting of Securities. From and after the Closing and until the date on

which the Investor and any Permitted Transferees together beneficially own less than ten percent (10%) of the Shares of Then Outstanding Common Stock (the "**Voting Agreement Term**"), the Investor shall, and shall cause any Permitted Transferees to, vote or execute a written consent with respect to the Purchased Shares, in the sole discretion of the Investor, in accordance with the recommendation of the Company's Board of Directors solely with respect to (i) the election of directors, provided that such directors are unanimously recommended by the Company's Board of Directors; (ii) the approval of the Company's auditor; (iii) the approval of, on a non-binding, advisory basis, the compensation of the Company's named executive officers; and (iv) the approval of an increase to the number of shares reserved for issuance or the issuance of shares under the Company's equity compensation or share plans for employees, consultants and directors. In furtherance of this Section 5.1, the Investor hereby irrevocably appoints the Company and any individuals designated by the Company (such designated individuals to be limited to the President and Chief Executive Officer, Chief Financial Officer or Secretary of the Company), and each of them individually, as the attorneys, agents and proxies, with full power of substitution and re-substitution in each of them, for the Investor, and in the name, place and stead of the Investor, to vote (or cause to be voted) in such manner as set forth in this Section 5.1 with respect to the Purchased Shares to which the Investor is or may be entitled to vote at any meeting of the Company held after the date hereof, whether annual or special and whether or not an adjourned meeting (the "**Irrevocable Proxy**"); provided that, this Irrevocable Proxy shall only be effective and exercisable if, at any annual or special meeting of the stockholders of the Company and at any adjournments or postponements of any such meetings, the Investor (i) fails to appear or otherwise fails to cause its voting securities of the Company to be counted as present for purposes of calculating a quorum, or (ii) fails to vote such voting securities in accordance with this Section 5.1, in each case at least five (5) Business Days prior to the date of such stockholders' meeting. This Irrevocable Proxy is coupled with an interest, shall be irrevocable and binding on any successor-in-interest of the Investor and shall not be terminated by operation of law upon the occurrence of any event. This Irrevocable Proxy shall operate to revoke and render void any prior proxy as to voting securities heretofore granted by the Investor which is inconsistent herewith. The Irrevocable Proxy shall terminate upon the expiration or termination of the Voting Agreement Term. The Investor shall cause any Permitted Transferee to promptly execute and deliver to the Company an irrevocable proxy, substantially in the form of Exhibit B attached hereto, and irrevocably appoint the Company and any individuals designated by the Company, and each of them individually, with full power of substitution and resubstitution, as its attorney, agent and proxy to vote (or cause to be voted) such Purchased Shares of the Company as to which such Permitted Transferee is entitled to vote, in such manner as each such attorney, agent and proxy or his substitute shall in its, his or her sole discretion deem appropriate or desirable with respect to the matters set forth in this Section 5.1 (the "**Permitted Transferee Irrevocable Proxy**"). The Investor acknowledges, and shall cause any Permitted Transferees to acknowledge, that any such proxy executed and delivered shall be coupled with an interest, shall constitute, among other things, an inducement for the Company to enter into this Agreement, shall be irrevocable and binding on any successor-in-interest of such Permitted Transferee and shall not be terminated by operation of Law upon the occurrence of any event. Such proxy shall operate to revoke and render void any prior proxy as to any voting securities of the Company heretofore granted by such Permitted Transferee, to the extent it is

inconsistent herewith. The Investor acknowledges and agrees that it shall be a condition to any proposed transfer of voting securities of the Company by the Investor to such Permitted Transferee that such Permitted Transferee execute and deliver to the Company a Permitted Transferee Irrevocable Proxy, and that any purported transfer shall be void and of no force or effect if such Permitted Transferee Irrevocable Proxy is not so executed and delivered at the closing of such transfer. Such proxy shall terminate upon the earlier of the expiration or termination of the Voting Agreement Term. The Investor acknowledges and agrees that it shall be a condition to any proposed transfer of voting securities of the Company by the Investor to any Permitted Transferee during the Voting Agreement Term that such Permitted Transferee shall agree in writing to be subject to and bound by all restrictions and obligations set forth in this Section 5.1.

In the event the Company's stockholders are permitted to act by written consent, the Company and the Investor shall each negotiate in good faith with the other provisions as consistent as possible with the foregoing to govern the voting of the Investor's and its Permitted Transferees' Purchased Shares as closely as practicable to the foregoing.

5.2 Quorum. In furtherance of Section 5.1, the Investor shall be, and shall cause each of its Permitted Transferees to be, present in person or represented by proxy at all meetings of stockholders to the extent necessary so that all voting securities of the Company as to which they are entitled to vote shall be counted as present for the purpose of determining the presence of a quorum at such meeting.

6. Information Rights.

6.1 Financial Information and Reporting. At any time when the Investor and its Affiliates beneficially own more than 19.9% (or such lesser percentage, as advised in good faith and in writing by the Investor's certified public accountants, that would be necessary pursuant to applicable accounting rules and guidelines so as to require the Investor to include in its financial statements its portion of the Company's financial results) of the Shares of Then Outstanding Common Stock, the Company will provide to the Investor or its designated Affiliate any financial information reasonably requested by the Investor solely so that the Investor can assess whether the Investor needs to account for the investment using the equity method of accounting or otherwise (the "**Purpose**"), including making its management reasonably available to the Investor for reasonable inquiries regarding its financial statements for the Purpose. If the Investor or its Affiliate (a) determines in good faith, in accordance with applicable accounting rules and guidance, that such financial information is reasonably likely to be necessary with respect to the financial reporting of the Investor or its Affiliates and (b) provides the Company with written notice to such effect, then beginning as promptly as reasonably practicable but in no event later than the 90th day following such notice, the Company will deliver via overnight delivery and electronic mail to the Investor or its Affiliate such reasonably requested financial information. The Investor and its Affiliates shall not use any financial information received pursuant to this Section 6 for any reason other than the Purpose.

6.2 Confidentiality. All confidential or proprietary information and data provided by the Company to either the Investor or its Affiliate pursuant to this Section 6 shall be deemed "Confidential Information" under the Collaboration and License Agreement and accordingly shall be subject to the terms and conditions of the Collaboration and License Agreement governing "Confidential Information." The confidentiality obligations of the Investor and its Affiliates under this Section 6 shall survive any termination or expiration of this Agreement.

6.3 Acknowledgment of Securities Law. The Investor is aware of the restrictions imposed by the U.S. securities law on the purchase or sale of securities by any person who has received material, non-public information from the issuer of such securities and on the communication of such information to any other person when it is reasonably foreseeable that such other person is likely to purchase or sell such securities in reliance upon such information.

Termination of Certain Rights and Obligations

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7.1 Termination of Registration Rights

. Except for Section 2.11, which shall survive until the expiration of any applicable statutes of limitation, Section 2 shall terminate automatically and have no further force or effect upon the earliest to occur of:

(a) the expiration of the Registration Rights Term;

(b) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act; and

(c) a liquidation or dissolution of the Company.

7.2 Termination of Standstill Agreement

. Section 3 shall terminate and have no further force or effect, upon the earliest to occur of:

(a) such time that the Company publicly announces that it has entered into with a Third Party a written definitive agreement for the acquisition (by way of merger, tender offer or otherwise), in each case, the consummation of which would result in a Change of Control of the Company;

(b) the expiration of the Standstill Term;

(c) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act; and

(d) a liquidation or dissolution of the Company.

7.3 Termination of Restrictions on Dispositions

. Section 4 shall terminate and have no further force or effect upon the earliest to occur of:

(a) the consummation of a Change of Control of the Company;

(b) a liquidation or dissolution of the Company; and

(c) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act.

7.4 Termination of Voting Agreement Term

. Section 5 shall terminate and have no further force or effect upon the earliest to occur of:

(a) the consummation of a Change of Control of the Company;

(b) the expiration of the Standstill Term;

(c) the date on which the Common Stock ceases to be registered pursuant to Section 12 of the Exchange Act; and

(d) a liquidation or dissolution of the Company.

7.5 Termination of Information Rights

. Section 6.1 shall terminate and have no further force or effect upon the earliest to occur of:

(a) the consummation of a Change of Control of the Company; and

(b) a liquidation or dissolution of the Company.

7.6 Effect of Termination

. No termination pursuant to any of Sections 7.1, 7.2, 7.3, 7.4 or 7.5 shall relieve any of the parties (or the Permitted Transferee, if any) for liability for breach of or default under any of their respective obligations or restrictions under any terminated provision of this Agreement, which breach or default arose out of events or circumstances occurring or existing prior to the date of such termination.

Miscellaneous

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8.1 Governing Law; Submission to Jurisdiction

. This Agreement shall be governed by and construed in accordance with the Laws of the State of Delaware, without regard to the conflict of laws principles thereof that would require the application of the Law of any other jurisdiction. Any action brought, arising out of, or relating to this Agreement shall be brought in the Court of Chancery of the State of Delaware. Each party hereby irrevocably submits to the exclusive jurisdiction of said Court in respect of any claim relating to the validity, interpretation and enforcement of this Agreement, and hereby waives, and agrees not to assert, as a defense in any action, suit or proceeding in which any such claim is made that it is not subject thereto or that such action, suit or proceeding may not be brought or is not maintainable in such courts, or that the venue thereof may not be appropriate or that this agreement may not be enforced in or by such courts. The parties hereby consent to and grant the Court of Chancery of

the State of Delaware jurisdiction over such parties and over the subject matter of any such claim and agree that mailing of process or other papers in connection with any such action, suit or proceeding in the manner provided in Section 8.3 or in such other manner as may be permitted by law, shall be valid and sufficient thereof.

8.2 Waiver

. Waiver by a party of a breach hereunder by another party shall not be construed as a waiver of any subsequent breach of the same or any other provision. No delay or omission by a party in exercising or availing itself of any right, power or privilege hereunder shall preclude the later exercise of any such right, power or privilege by such party. No waiver shall be effective unless made in writing with specific reference to the relevant provision(s) of this Agreement and signed by a duly authorized representative of the party granting the waiver.

8.3 Notices

. All notices, instructions and other communications hereunder or in connection herewith shall be in writing, shall be sent to the address of the relevant party set forth on Exhibit A attached hereto and shall be (a) delivered personally, (b) sent by registered or certified mail, return receipt requested, postage prepaid, (c) sent via a reputable nationwide overnight courier service or (d) sent by electronic mail, with a confirmation copy to be sent by registered or certified mail, return receipt requested, postage prepaid. Any such notice, instruction or communication shall be deemed to have been delivered upon receipt if delivered by hand, three (3) Business Days after it is sent by registered or certified mail, return receipt requested, postage prepaid, one (1) Business Day after it is sent via a reputable nationwide overnight courier service or when transmitted with electronic confirmation of receipt, if transmitted by electronic mail (if such transmission is made during regular business hours of the recipient on a Business Day; or otherwise, on the next Business Day following such transmission). Any party may change its address by giving notice to the other parties in the manner provided above.

8.4 Entire Agreement

. This Agreement, the Purchase Agreement and the Collaboration and License Agreement contain the entire agreement among the parties with respect to the subject matter hereof and thereof and supersede all prior and contemporaneous arrangements or understandings, whether written or oral, with respect hereto and thereto.

8.5 Amendments

. No provision in this Agreement shall be supplemented, deleted or amended except in a writing executed by an authorized representative of each of the parties hereto.

8.6 Headings; Nouns and Pronouns; Section References

. Headings in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement. Whenever the context may require, any pronouns used herein shall include the corresponding masculine, feminine or neuter forms, and the singular form of names and pronouns shall include the plural and vice-versa. References in this Agreement to a section or subsection shall be deemed to refer to a section or subsection of this Agreement unless otherwise expressly stated.

8.7 Severability

. If, under applicable Laws, any provision hereof is invalid or unenforceable, or otherwise directly or indirectly affects the validity of any other material provision(s) of this Agreement in any jurisdiction ("**Modified Clause**"), then, it is mutually agreed that this Agreement shall endure and that the Modified Clause shall be enforced in such jurisdiction to the maximum extent permitted under applicable Laws in such jurisdiction; provided that the parties shall consult and use all reasonable efforts to agree upon, and hereby consent to, any valid and enforceable modification of this Agreement as may be necessary to avoid any unjust enrichment of either party and to match the intent of this Agreement as closely as possible, including the economic benefits and rights contemplated herein.

8.8 Assignment

. Neither this Agreement nor any rights or duties of a party hereto may be assigned by such party, in whole or in part, without (a) the prior written consent of the Company in the case of any assignment by the Investor, except as provided by Section 2.13 with respect to the Investor's assignment to a Permitted Transferee; or (b) the prior written consent of the Investor in the case of an assignment by the Company.

8.9 Successors and Assigns

. This Agreement shall be binding upon and inure to the benefit of the parties hereto and their respective successors and permitted assigns.

8.10 Counterparts

. This Agreement may be executed in counterparts, each of which shall be deemed an original but which together shall constitute one and the same instrument.

8.11 Third Party Beneficiaries

. None of the provisions of this Agreement shall be for the benefit of or enforceable by any Third Party. No Third Party shall obtain any right under any provision of this Agreement or shall by reason of any such provision make any claim in respect of any debt, liability or obligation (or otherwise) against any party hereto.

8.12 No Strict Construction

. This Agreement has been prepared jointly and will not be construed against any party.

8.13 Remedies

. The rights, powers and remedies of the parties under this Agreement are cumulative and not exclusive of any other right, power or remedy which such parties may have under any other agreement or Law. No single or partial assertion or exercise of any right, power or remedy of a party hereunder shall preclude any other or further assertion or exercise thereof.

8.14 Specific Performance

. The Company and the Investor hereby acknowledge and agree that the rights of the parties hereunder are special, unique and of extraordinary character, and that if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this Agreement, such refusal or failure would result in irreparable injury to the Company or the Investor, as the case may be, the exact amount of which would be difficult to ascertain or estimate and the remedies at law for which would not be reasonable or adequate compensation. Accordingly, if any party refuses or otherwise fails to act, or to cause its Affiliates to act, in accordance with the provisions of this

Agreement, then, in addition to any other remedy which may be available to any damaged party at law or in equity, such damaged party will be entitled to seek specific performance and injunctive relief, without posting bond or other security, and without the necessity of proving actual or threatened damages, which remedy such damaged party will be entitled to seek in any court of competent jurisdiction.

8.15 No Conflicting Agreements

. The Investor hereby represents and warrants to the Company that neither it nor any of its Affiliates is, as of the date of this Agreement, a party to, and agrees that neither it nor any of its Affiliates shall, on or after the date of this Agreement, enter into any agreement that conflicts with the rights granted to the Company in this Agreement. The Company hereby represents and warrants to each Holder that it is not, as of the date of this Agreement, a party to, and agrees that it shall not, on or after the date of this Agreement, enter into, any agreement or approve any amendment to its Organizational Documents (as defined in the Purchase Agreement) with respect to its securities that conflicts with the rights granted to the Holders in this Agreement. The Company further represents and warrants that the rights granted to the Holders hereunder do not in any way conflict with the rights granted to any other holder of the Company's securities under any other agreements.

8.16 Use of Proceeds.

The Company shall use the proceeds from the sale of the Purchased Shares primarily for the purpose of funding research, development and related activities, and other working capital purposes.

(*Signature Page Follows*)

IN WITNESS WHEREOF, the parties have executed and delivered this Agreement as of the date first above written.

SOLID BIOSCIENCES INC.

By: /s/ Ilan Ganot
Name: Ilan Ganot
Title: Chief Executive Officer

ACTIVE/104419179.14

ULTRAGENYX PHARMACEUTICAL INC.

By: /s/ Emil D. Kakkis
Name: Emil D. Kakkis, M.D. Ph.D.
Title: Chief Executive Officer

EXHIBIT A

NOTICES

(a) If to the Company:

Solid Biosciences Inc.
141 Portland Street, Fifth Floor
Cambridge, MA 02139
Attention: Lynette Herscha
Email:

with a copy to:

Wilmer Cutler Pickering Hale and Dorr LLP
60 State Street
Boston, MA 02109
Attention: Lia Der Marderosian
Email: lia.dermarderosian@wilmerhale.com

(b) If to the Investor:

Ultragenyx Pharmaceutical Inc.
60 Leveroni Court
Novato, CA 94949
Attention: Chief Business Officer; General Counsel
Email:

with a copy to:

Goodwin Procter LLP
100 Northern Avenue
Boston, MA 02210
Attention: Ed Amer
Email: EAmer@goodwinlaw.com

EXHIBIT B

FORM OF IRREVOCABLE PROXY

In order to secure the performance of the duties of the undersigned pursuant to Section 5.1 of the Investor Agreement, dated as of as of October 22, 2020 (the "**Agreement**"), by and between Ultragenyx Pharmaceutical Inc. and Solid Biosciences Inc. (the "**Company**"), the undersigned hereby irrevocably appoints the Company and any individual designated by the Company, and each of them individually, as the attorneys, agents and proxies, with full power of substitution and resubstitution in each of them, for the undersigned, and in the name, place and stead of the undersigned, to vote (or cause to be voted) in such manner as set forth in Section 5.1 of the Agreement with respect to all Purchased Shares, which the undersigned is or may be entitled to vote at any meeting of the Company held after the date hereof, whether annual or special and whether or not an adjourned meeting. This proxy is coupled with an interest, shall be irrevocable and binding on any successor-in-interest of the undersigned and shall not be terminated by operation of law upon the occurrence of any event. This proxy shall operate to revoke and render void any prior proxy as to voting securities heretofore granted by the undersigned which is inconsistent herewith. Notwithstanding the foregoing, this irrevocable proxy shall be effective if, at any annual or special meeting of the stockholders of the Company (or any consent in lieu thereof) and at any adjournments or postponements of any such meetings, the undersigned (i) fails to appear or otherwise fails to cause its voting securities of the Company to be counted as present for purposes of calculating a quorum, or (ii) fails to vote such voting securities in accordance with Section 5.1 of the Agreement, in each case at least five (5) Business Days prior to the date of such stockholders' meeting. This proxy shall terminate upon the earlier of the expiration or termination of the Voting Agreement Term.

ULTRAGENYX PHARMACEUTICAL INC.

By:

Name:
Title:

Exhibit 10.4

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) would likely cause competitive harm to the Company, if publicly disclosed.
Double asterisks denote omissions.

FIRST AMENDMENT
TO THE EXCLUSIVE PATENT LICENSE

This First Amendment dated as of October 9, 2020 (the "Amendment") is made to that Exclusive Patent License between Solid Biosciences Inc. ("Solid") and the University of Washington acting through UW CoMotion ("University") dated as of October 16, 2015 with a University reference number 37475A (the "Agreement"). Capitalized terms used herein and not defined have the meaning as set forth in the Agreement.

Whereas, the Parties acknowledge that Solid is in discussions with Ultragenyx Pharmaceutical Inc. ("Collaborator") to potentially Sublicense certain rights to the Licensed Patents to the Collaborator (the "Transaction"); and

Whereas, the Parties wish to amend the Sublicensing Consideration solely as it relates to the Transaction.

Now, therefore, in consideration of the agreements set forth herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Parties hereby agree as follows:

1. Sublicensing Consideration. The Parties hereby acknowledge that Solid has achieved Milestone [**] as set forth in Section A.2. of Exhibit A to the Agreement, which will result in Solid paying to University [**] of the Sublicensing Consideration received by Solid from the Transaction. Notwithstanding the foregoing, and notwithstanding Section 6.1 and Exhibit A of the Agreement, the Parties hereby agree that, solely in the event that Solid and Collaborator consummate the Transaction within [**] after the date of this Amendment, Solid shall pay University $[**] solely in lieu of the [**] of Sublicensing Consideration due to University from the upfront consideration received by Solid from Collaborator at the time the Transaction is executed or substantially contemporaneously therewith, including any premium over Fair Market Value received by Solid from Collaborator on any sale and issuance of Solid's common stock in connection with the execution of the Transaction. All other payments due to University in respect of Sublicensing Consideration from the Transaction shall be payable as set forth in the Agreement and due according to Section 6.1 of the Agreement and Section A3.7 of Exhibit A of the Agreement.

2. Financial Milestones. The Parties hereby agree to amend the Financial Milestones set forth in Section A3.5 of Exhibit A to the Agreement, whether achieved by Company or a Sublicensee, as follows:

Section	Financial Milestone	Amount Payable
A3.5.2	[**]	[**]
A3.5.3	[**]	[**]
A3.5.4	[**]	[**]
A3.5.5	[**]	[**]

Except as expressly set forth herein, all of the terms and conditions of the Agreement remain in full force and effect. To the extent that there are any inconsistencies between the terms of the Agreement and Amendment, the terms of this Amendment shall prevail in effect.

The parties acknowledge that the Agreement and this Amendment set forth the entire understanding and intentions of the parties hereto as to the subject matter hereof, and supersedes all previous understandings between the parties, written or oral, regarding such subject matter.

IN WITNESS WHEREOF, Solid and University have caused this Amendment to be executed by their duly authorized officers as of the date first set forth above.

SOLID BIOSCIENCES INC. UNIVERSITY OF WASHINGTON

By: /s/ Ilan Ganot By: /s/ Fiona Wills

Print Name: Ilan Ganot Print name: Fiona Wills

Title: Chief Executive Officer Title: Associate Vice Provost, Innovation Development

Exhibit 31.1

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

I, Ilan Ganot, certify that:

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

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

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

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

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

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

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

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

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

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

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

/s/ Ilan Ganot
Ilan Ganot
President and Chief Executive Officer
(Principal Executive Officer)
Dated: November 5, 2020

Exhibit 31.2

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

I, Jennifer Ziolkowski, certify that:

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

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

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

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

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

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

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

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

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

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

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

/s/ Jennifer Ziolkowski
Jennifer Ziolkowski
Chief Financial Officer
(Principal Financial and Accounting Officer)
Dated: November 5, 2020

Exhibit 32.1

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

In connection with the Quarterly Report on Form 10-Q of Solid Biosciences Inc. (the "Company") for the quarter ended September 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Ilan Ganot, Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to his knowledge:

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

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

Dated: November 5, 2020

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

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

Exhibit 32.2

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

In connection with the Quarterly Report on Form 10-Q of Solid Biosciences Inc. (the "Company") for the quarter ended September 30, 2020 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Jennifer Ziolkowski, Chief Financial Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to her knowledge:

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

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

Dated: November 5, 2020

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

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