UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 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 March 31, 2020

OR

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

For the transition period from to

Commission file number 001-14895

SAREPTA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**93-0797222**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
215 First Street, Suite 415 Cambridge, MA	**02142**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 274-4000

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

Title of each class	Trading Symbol	Name of exchange on which registered
Common Stock, $0.0001 par value per share	SRPT	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, a smaller reporting company or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer", "smaller reporting company" and "emerging growth company" in Rule 12b-2 of the Exchange Act:

Large accelerated filer	☒	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 ☒

Indicate the number of shares outstanding of each of the issuer's classes of common stock, as of the latest practicable date.

Common Stock with $0.0001 par value	77,988,744
(Class)	**(Outstanding as of May 1, 2020)**

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Item 1. Financial Statements

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited, in thousands, except share and per share amounts)

	As of March 31, 2020	As of December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 1,764,212	$ 835,080
Short-term investments	406,940	289,668
Accounts receivable	106,875	90,879
Inventory	173,168	171,379
Other current assets	96,153	81,907
Total current assets	2,547,348	1,468,913
Property and equipment, net	137,325	129,620
Intangible assets, net	12,813	12,497
Right of use assets	63,097	37,933
Other non-current assets	186,805	173,859
Total assets	$ 2,947,388	$ 1,822,822
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 26,595	$ 68,094
Accrued expenses	176,298	185,527
Deferred revenue, current portion	91,073	3,303
Other current liabilities	12,463	7,843
Total current liabilities	306,429	264,767
Long-term debt	687,953	681,900
Lease liabilities	65,263	47,720
Deferred revenue, net of current portion	732,667	—
Other non-current liabilities	10,248	10,248
Total liabilities	1,802,560	1,004,635
Commitments and contingencies (Note 15)		
Stockholders' equity:		
Preferred stock, $0.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $0.0001 par value, 99,000,000 shares authorized; 77,957,790 and 75,184,863 issued and outstanding at March 31, 2020, and December 31, 2019, respectively	8	8
Additional paid-in capital	3,455,689	3,112,130
Accumulated other comprehensive income, net of tax	624	50
Accumulated deficit	(2,311,493)	(2,294,001)
Total stockholders' equity	1,144,828	818,187
Total liabilities and stockholders' equity	$ 2,947,388	$ 1,822,822

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(unaudited, in thousands, except per share amounts)

	For the Three Months Ended March 31,	
	2020	**2019**
Revenues:		
Products, net	$ 100,448	$ 87,011
Collaboration	13,226	—
Total revenues	113,674	87,011
Cost and expenses:		
Cost of sales (excluding amortization of in-licensed rights)	12,622	12,063
Research and development	136,144	90,553
Selling, general and administrative	82,768	60,566
Amortization of in-licensed rights	166	216
Total cost and expenses	231,700	163,398
Operating loss	(118,026)	(76,387)
Other income (loss):		
Gain from sale of Priority Review Voucher	108,069	—
Other expense, net	(7,420)	(172)
Total other income (loss)	100,649	(172)
Loss before income tax expense	(17,377)	(76,559)
Income tax expense	115	84
Net loss	(17,492)	(76,643)
Other comprehensive income:		
Unrealized gains on investments, net of tax	574	118
Total other comprehensive income	574	118
Comprehensive loss	$ (16,918)	$ (76,525)
Net loss per share - basic and diluted	$ (0.23)	$ (1.07)
Weighted average number of shares of common stock used in computing basic and diluted net loss per share	76,432	71,731

See accompanying notes to unaudited condensed consolidated financial statements.

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY
(unaudited, in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2019	75,185	$ 8	$ 3,112,130	$ 50	$ (2,294,001)	$ 818,187
Exercise of options for common stock	97	—	3,687	—	—	3,687
Vest of restricted stock units	98	—	—	—	—	—
Issuance of common stock to Roche, net of issuance costs	2,522	—	312,053	—	—	312,053
Issuance of common stock under employee stock purchase plan	56	—	3,795	—	—	3,795
Stock-based compensation	—	—	24,024	—	—	24,024
Unrealized gains from available-for-sale securities, net of tax	—	—	—	574	—	574
Net loss	—	—	—	—	(17,492)	(17,492)
Balance at March 31, 2020	77,958	$ 8	$ 3,455,689	$ 624	$ (2,311,493)	$ 1,144,828

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive (Loss) Income	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2018	71,072	$ 7	$ 2,611,294	$ (99)	$ (1,578,926)	$ 1,032,276
Exercise of options for common stock	382	—	9,973	—	—	9,973
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	35	—	—	—	—	—
Shares withheld for taxes	(7)	—	(889)	—	—	(889)
Issuance of common stock for cash, net of offering costs	2,604	—	365,264	—	—	365,264
Issuance of common stock under employee stock purchase plan	48	—	2,326	—	—	2,326
Stock-based compensation	—	—	16,139	—	—	16,139
Unrealized gains from available-for-sale securities	—	—	—	118	—	118
Net loss	—	—	—	—	(76,643)	(76,643)
Balance at March 31, 2019	74,134	$ 7	$ 3,004,107	$ 19	$ (1,655,569)	$ 1,348,564

SAREPTA THERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(unaudited, in thousands)

	For the Three Months Ended March 31,	
	2020	**2019**
Cash flows from operating activities:		
Net loss	$ (17,492)	$ (76,643)
Adjustments to reconcile net loss to cash flows from operating activities:		
Gain from sale of Priority Review Voucher	(108,069)	—
Depreciation and amortization	6,529	4,879
Reduction in the carrying amounts of the right of use assets	2,153	1,491
Amortization of investment discount	(1,284)	(3,476)
Non-cash interest expense	6,342	5,208
Stock-based compensation	24,024	16,139
Other	(1,381)	88
Changes in operating assets and liabilities, net:		
Net increase in accounts receivable	(15,996)	(1,466)
Net increase in inventory	(1,789)	(15,022)
Net increase in other assets	(27,966)	(79,564)
Net increase in deferred revenue	820,437	682
Net (decrease) increase in accounts payable, accrued expenses and other liabilities	(57,718)	1,451
Net cash provided by (used in) operating activities	627,790	(146,233)
Cash flows from investing activities:		
Purchase of property and equipment	(9,120)	(16,263)
Proceeds from sale of Priority Review Voucher, net of commission	108,069	—
Purchase of available-for-sale securities	(365,437)	(494,481)
Maturity and sale of available-for-sale securities	250,000	646,830
Other	(1,192)	(855)
Net cash (used in) provided by investing activities	(17,680)	135,231
Cash flows from financing activities:		
Proceeds from issuance of common stock to Roche	316,338	—
Proceeds from sale of common stock, net of offering costs	—	365,264
Proceeds from exercise of stock options and purchase of stock under the Employee Stock Purchase Program	7,482	12,299
Taxes paid related to net share settlement of equity awards	(4,798)	—
Net cash provided by financing activities	319,022	377,563
Increase in cash, cash equivalents and restricted cash	929,132	366,561
Cash, cash equivalents and restricted cash:		
Beginning of period	843,645	370,829
End of period	$ 1,772,777	$ 737,390
Reconciliation of cash, cash equivalents and restricted cash:		
Cash and cash equivalents	$ 1,764,212	$ 732,190
Restricted cash in other assets	8,565	5,200
Total cash, cash equivalents and restricted cash	$ 1,772,777	$ 737,390
Supplemental disclosure of cash flow information:		
Cash paid during the period for interest	$ 5,385	$ —
Supplemental schedule of non-cash investing activities and financing activities:		
Issuance costs related to the Roche Collaboration in accrued expenses	$ 4,285	$ —
Manufacturing right of use asset additions	$ 27,554	$ —
Manufacturing lease liability additions	$ 24,783	$ —
Sale of available-for-sale securities included in investment receivable	$ —	$ 42,300
Intangible assets and property and equipment included in accrued expenses	$ 6,154	$ 3,108

See accompanying notes to unaudited condensed consolidated financial statements.

1. ORGANIZATION AND NATURE OF BUSINESS

Sarepta Therapeutics, Inc. (together with its wholly-owned subsidiaries, "Sarepta" or the "Company") is a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying its proprietary, highly-differentiated and innovative technologies, and through collaborations with its strategic partners, the Company is developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne muscular dystrophy ("DMD"), Limb-girdle muscular dystrophies ("LGMDs"), Mucopolysaccharidosis type IIIA ("MPS IIIA") and other neuromuscular and central nervous system ("CNS") disorders.

Its first and second commercial products in the U.S., EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51") and VYONDYS 53 (golodirsen) Injection ("VYONDYS 53") were granted accelerated approval by the U.S. Food and Drug Administration (the "FDA") on September 19, 2016 and December 12, 2019, respectively. EXONDYS 51 and VYONDYS 53 are indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 and exon 53 skipping, respectively. EXONDYS 51 and VYONDYS 53 use the Company's phosphorodiamidate morpholino oligomer ("PMO") chemistry and exon-skipping technology to skip exon 51 and exon 53, respectively, of the dystrophin gene. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

As of March 31, 2020, the Company had approximately $2,180.7 million of cash, cash equivalents and investments, consisting of $1,764.2 million of cash and cash equivalents, $406.9 million of short-term investments, and $9.6 million of restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of the date of the issuance of this report is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue raising additional cash resources through public or private debt and equity financings, seek additional government contracts and establish collaborations with or license its technology to other companies.

2. SUMMARY OF SIGNIFICANT ACCOUNTING POLICIES AND RECENT ACCOUNTING PRONOUNCEMENTS

Basis of Presentation

The accompanying unaudited condensed consolidated financial statements, which have been prepared in accordance with accounting principles generally accepted in the United States ("U.S. GAAP"), reflect the accounts of Sarepta Therapeutics, Inc. and its wholly-owned subsidiaries. All intercompany transactions between and among its consolidated subsidiaries have been eliminated. Management has determined that the Company operates in one segment: discovering, developing, manufacturing and delivering therapies to patients with rare diseases.

These unaudited condensed consolidated financial statements should be read in conjunction with the audited consolidated financial statements and related notes for the year ended December 31, 2019 which are contained in the Company's Annual Report on Form 10-K filed with the U.S. Securities and Exchange Commission on February 26, 2020. The results for the three months ended March 31, 2020 are not necessarily indicative of the results to be expected for the full year.

Estimates and Uncertainties

The preparation of the unaudited condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, revenue, expenses and the disclosure of contingent assets and liabilities. Actual results could differ from those estimates.

Concentration of Credit Risk

Financial instruments which potentially subject the Company to concentrations of credit risk consist of accounts receivable from customers, cash held at financial institutions and cash equivalents and investments.

As of March 31, 2020, the majority of the Company's accounts receivable arose from product sales in the U.S. and all customers have standard payment terms which generally require payment within 60 to 91 days. Outside of the U.S., the majority of the Company's customers have payment terms ranging between 45 and 150 days. Three individual customers accounted for 44%, 40% and 12% of net product revenues for the three months ended March 31, 2020 and 41%, 40% and 15% of net product revenues for the three months ended March 31, 2019. Three individual customers accounted for 49%, 37% and 11% of accounts receivable from product sales as of March 31, 2020 and 57%, 26% and 8% of accounts receivable from product sales as of March 31, 2019. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in its customers' credit profile. As of March 31, 2020, the Company believes that such customers are of high credit quality.

As of March 31, 2020, the Company's cash was concentrated at three financial institutions in the U.S., which potentially exposes the Company to credit risks. However, the Company does not believe that there is significant risk of non-performance by the financial institutions.

Significant Accounting Policies

For details about the Company's accounting policies, please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* of the Annual Report on Form 10-K for the year ended December 31, 2019.

Collaboration revenue

The Company's collaboration revenue is generated from its collaboration arrangement with F. Hoffman-La Roche Ltd. ("Roche"). For more information, please read *Note 3, Collaboration and License Agreements*. At the inception of a collaboration arrangement, the Company first assesses whether the contractual arrangement is within the scope of ASC 808, *Collaborative Arrangements* ("ASC 808") to determine whether the arrangement involves a joint operating activity and involves two (or more) parties that are both active participants in the activity and exposed to significant risks and rewards dependent on the commercial success of such activity. Then the Company determines whether the collaboration arrangement in its entirety represents a contract with a customer as defined by ASC 606, *Revenue from Contracts with Customers* ("ASC 606"). If only a portion of the collaboration arrangement is potentially with a customer, the Company applies the distinct good or service unit-of-account guidance in ASC 606 to determine whether there is a unit of account that should be accounted for under ASC 606. For the units of account in the collaboration arrangement that do not represent a vendor-customer relationship, the Company will (i) consider applying other GAAP, including by analogy, or (ii) if there is no appropriate analogy, consistently apply a reasonable and rational accounting policy election.

In general, by analogy to ASC 606, the Company identifies the performance obligations within the collaboration arrangement and identifies and allocates the transaction price the Company expects to receive on a relative standalone selling price basis to each performance obligation. The residual approach is used to determine estimated standalone selling prices when the selling price is uncertain. Variable consideration, consisting of development and regulatory milestones, will be included in the transaction price only if the Company expects to receive such consideration and if it is probable that the inclusion of the variable consideration will not result in a significant reversal in the cumulative amount of revenue recognized under the arrangement. Sales-based royalty and milestone payments are excluded from the transaction price the Company expects to receive until the underlying sales occur because the license to the Company's intellectual property is deemed to be the predominant item to which the royalties or milestones relate as it is the primary driver of value in its collaboration arrangement.

For the recognition of revenue associated with each performance obligation, if the Company determines ASC 606 is not appropriate to apply by analogy, the Company will apply a reasonable, rational, and consistently applied accounting policy election to faithfully depict the transfer of services to the collaboration partner over the estimated performance period. Up-front payments from a collaboration partner are recognized as deferred revenue when received and recognized as revenue over the estimated performance period. Reimbursement payments from a collaboration partner associated with research and development cost sharing provisions in a collaboration arrangement are recognized as the related expense is incurred and classified as an offset to research and development expense.

The Company's collaboration arrangements may contain options which provide the collaboration partner with the right to obtain additional licenses. If an arrangement contains customer options, by analogy to ASC 606, the Company evaluates the customer options to determine whether they represent material rights, which may include options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent material rights, they are recognized as a separate performance obligation at inception of the arrangement. The Company allocates a portion of the transaction price of the collaboration arrangement to material rights based on the relative standalone selling price. Amounts allocated to material rights are not recognized as revenue until related options are exercised or expire.

Key assumptions to determine the standalone selling price of units of account in a collaboration arrangement include, but are not limited to, forecasted revenues, development timelines, incremental costs related to the arrangement, discount rates and probabilities of technical and regulatory success.

Leases

In accordance with ASC 842, *Leases* ("ASC 842"), components of a lease should be bifurcated between lease components and non-lease components. The fixed and in-substance fixed contract consideration identified must then be allocated based on the relative standalone price to the lease and non-lease components. However, ASC 842 provides entities with a practical expedient that allows them to make an accounting policy election to not separate lease and non-lease components by class of underlying asset. In using this expedient, entities would account for each lease component and the related non-lease component together as a single

component. For new and amended real estate leases beginning after January 1, 2019, the Company has elected to account for the lease and non-lease components together for existing classes of underlying assets and allocates the contract consideration to the lease component only. In contrast, the Company does not apply the practical expedient for embedded leases in manufacturing and supply agreements with certain of its contract manufacturing organizations and has instead allocated contract consideration between the lease and non-lease components based on their relative standalone price.

There have not been any other material changes to the Company's accounting policies through March 31, 2020.

Recent Accounting Pronouncements

Not yet adopted

In December 2019, the Financial Accounting Standards Board ("FASB") issued ASU 2019-12, "*Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes"*, which is intended to simplify the accounting for income taxes. This ASU removes certain exceptions to the general principles in Topic 740 and clarifies and amends existing guidance to improve consistent application. The new standard will be effective beginning January 1, 2021. As of March 31, 2020, the Company is continuing to evaluate the potential impact this ASU may have on its financial position and results of operations upon adoption.

Recently adopted

In August 2018, the FASB issued ASU No. 2018-13, *"Fair Value Measurement (Topic 820), Disclosure Framework – Changes to the Disclosure Requirements for Fair Value Measurement"*. This ASU removed the following disclosure requirements: (1) the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy; (2) the policy for timing of transfers between levels; and (3) the valuation processes for Level 3 fair value measurements. Additionally, this update added the following disclosure requirements: (1) the changes in unrealized gains and losses for the period included in other comprehensive income and loss for recurring Level 3 fair value measurements held at the end of the reporting period; (2) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. For certain unobservable inputs, an entity may disclose other quantitative information (such as the median or arithmetic average) in lieu of the weighted average if the entity determines that other quantitative information would be a more reasonable and rational method to reflect the distribution of unobservable inputs used to develop Level 3 fair value measurements. ASU No. 2018-13 was effective beginning January 1, 2020. The adoption of this guidance did not have a material effect on the Company's consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-15, "*Intangibles – Goodwill and Other – Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That is a Service Contract"*. This ASU requires a customer in a cloud computing arrangement (i.e., hosting arrangement) that is a service contract to follow the internal-use software guidance contained in ASC Subtopic 350-40 to determine which implementation costs to capitalize as assets or expense as incurred. Capitalized implementation costs related to a hosting arrangement that is a service contract will be amortized over the term of the hosting arrangement, beginning when the module or component of the hosting arrange is ready for its intended use. ASU No. 2018-15 was effective beginning January 1, 2020. The adoption of this guidance did not have a material effect on the Company's consolidated financial statements.

In June 2016, the FASB issued ASU No. 2016-13, "*Financial Instruments - Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments"*. This ASU requires that credit losses for financial instruments measured at amortized cost be reported using an expected losses model rather than the incurred losses model that is currently used, and establishes additional disclosures related to credit risks. For available-for-sale debt securities with unrealized losses, this standard now requires allowances to be recorded instead of reducing the amortized cost of the investment. ASU 2016-13 limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and requires the reversal of previously recognized credit losses if fair value increases. ASU 2016-13 was effective beginning January 1, 2020. The adoption of this guidance did not have a material effect on the Company's consolidated financial statements. The Company has no historical write-offs of its accounts receivable and its terms range from 60 to 91 days for sales within the U.S. and 45 and 150 days for the majority of sales outside the U.S. The Company monitors the creditworthiness of its customers and payments such that it can properly assess and respond to changes in the customers' credit profile or any specific issues. Upon adoption and as of March 31, 2020, the Company believes that such customers are of high credit quality and the expected credit losses are insignificant.

In November 2018, the FASB issued ASU 2018-18, "*Collaborative Arrangements (Topic 808): Clarifying the Interaction between Topic 808 and Topic 606"*. This ASU: (i) clarifies that certain transactions between collaborative arrangement participants should be accounted for as revenue under ASC 606 when the collaborative arrangement participant is a customer, (ii) provides guidance specifying that a distinct good or service is the unit of account for evaluating whether a transaction is with a customer, and (iii) precludes a company from presenting transactions with a collaborative arrangement participant that are not in the scope of ASC 606 together with revenue from contracts with customers. The new standard was effective beginning January 1, 2020. The Company presents collaboration revenue separate from product revenues.

3. LICENSE AND COLLABORATION AGREEMENTS

Roche Holding A.G.

On December 21, 2019, the Company entered into a license, collaboration and option agreement with Roche and a stock purchase agreement with an affiliate of Roche (collectively, the "Roche Agreement"), providing Roche with exclusive commercial rights to SRP-9001, the Company's investigational gene therapy for DMD, outside the U.S. The Company retains all rights to SRP-9001 in the U.S. and will perform all development activities within the joint global development plan necessary to obtain and maintain regulatory approvals for SRP-9001 in the U.S. and the EU. Further: (i) research and development expenses incurred under the joint global development plan will be equally shared between the Company and Roche, (ii) Roche is solely responsible for all costs incurred in connection with any development activities (other than those within the joint global development plan) that are necessary to obtain or maintain regulatory approvals outside the U.S, and (iii) the Company will continue to be responsible for the manufacturing of clinical and commercial supplies of SRP-9001. The Company has also granted Roche options to acquire ex-U.S. rights to certain future DMD-specific programs (the "Options") in exchange for separate option exercise payments, milestone and royalty considerations, and cost sharing provisions. The agreement became effective on February 4, 2020 ("Effective Date").

Within 10 days of the Effective Date, the Company received an aggregate of approximately $1.2 billion in cash consideration from Roche, consisting of an up-front payment and an equity investment in the Company. Additionally, the Company may receive up to $1.7 billion in development, regulatory and sales milestones related to SRP-9001. Upon commercialization, the Company is also eligible to receive tiered royalty payments based on net sales.

The Roche Agreement is governed by a joint steering committee ("JSC") formed by representatives from Roche and the Company. The JSC, among other activities, manages the overall strategic alignment between the parties, approves any material update to the joint global development plan and budget and oversees the operations of the subcommittees.

The Company determined that the Roche Agreement represents a collaboration arrangement subject to the scope of ASC 808. To determine if the collaboration arrangement was also within the scope of ASC 606, using the unit of account guidance in ASC 606, the Company identified the distinct goods or services in the Roche Agreement and evaluated whether they were transferred to a customer. However, since the Company's ordinary activities do not include contracting with third parties to provide them with research and development services, it was determined that the Roche Agreement was not within the scope of ASC 606. Thus, for recognition and measurement purposes, the Company must apply other GAAP, including by analogy, or if there is no appropriate analogy, apply a reasonable, rational and consistently applied accounting policy election.

Accordingly, the Company has analogized to ASC 606 for the accounting for certain aspects of the Roche Agreement. Of the $1.2 billion cash received from Roche, $316.3 million was allocated to the 2,522,227 shares of the Company's common stock issued to Roche based on the closing price when the shares were issued. Further, $491.0 million was allocated to the Options, as the Company determined that the option exercise payments (ranging from $20.0 million to $125.0 million per Option) are priced at a discount, resulting in material rights. The residual amount of $342.7 million was allocated to a single, combined performance obligation ("Combined Performance Obligation") comprised of: (i) the license of IP relating to SRP-9001 transferred to Roche, (ii) the related research and development services provided under the joint global development plan, (iii) the services provided to manufacture clinical supplies of SRP-9001, and (iv) the Company's participation in the JSC, because the Company determined that the license of IP and related activities were not capable of being distinct from one another.

The Company recorded $312.1 million of common shares issued to Roche, based on the closing price of the Company's stock on the date such shares were issued, less direct transaction fees incurred of $4.3 million. This net amount is reflected as an increase to common stock and additional paid-in-capital in the accompanying unaudited condensed consolidated balance sheets.

The $491.0 million allocated to the material rights associated with the Options was based on their estimated standalone selling prices, determined using an income approach of projected incremental discounted cash flows from each Option. The discounted cash flows incorporate the likelihood of success of the individual product candidates and the related commercial opportunity. The value assigned to the individual material rights is reflected as deferred revenue and will not be recognized until an option is either: (i) exercised by Roche, or (ii) expires. If exercised, the value of the material right will be aggregated with the option exercise price and recognized over the applicable performance period. If expired, the related transaction price will be recognized immediately. Through March 31, 2020, no options have been exercised or expired.

The $342.7 million allocated to the Combined Performance Obligation was determined using the residual approach because the standalone selling price is uncertain. The Company recognizes revenue related to the Combined Performance Obligation on a straight-line basis over the expected performance period of the joint global development plan, which is expected to extend through the

fourth quarter of 2023. The Company believes this method represents the best depiction of the transfer of services to Roche, as the estimated full-time equivalent employees dedicated to the services is not expected to materially vary over the expected service period.

Revenue relating to future development, regulatory and sales milestones will be recognized when the milestone is probable of achievement (which is typically when the milestone has occurred). Any royalties payable by Roche in the future will be recognized in the period earned. In addition, the Company determined that the supply of commercial product to Roche under the agreement is not priced at a discount and represents optional goods or services (i.e., a contingent promise). Accordingly, any revenues associated with the supply of commercial product in the future will be recognized in the period earned.

The Company classifies all revenues recognized under the Roche Agreement as collaboration revenues within the unaudited condensed consolidated statements of operations. For the three months ended March 31, 2020, the Company recognized $13.2 million of collaboration revenue, all of which relates to the Combined Performance Obligation. As of March 31, 2020, the Company has total deferred revenue of $820.4 million associated with the Roche Agreement, of which $87.8 million is classified as current. The portion of deferred revenue related to the separate material rights for the Options was $491.0 million as of March 31, 2020.

The costs associated with co-development activities performed under the Roche Agreement are included in research and development expenses, with any reimbursement of costs by Roche reflected as a reduction of such expenses when the related expense is incurred. For the three months ended March 31, 2020, costs reimbursable by Roche and reflected as a reduction to research and development expenses were $16.4 million and is included in accounts receivable as of March 31, 2020.

Milestone Obligations

The Company has license and collaboration agreements in place for which it could be obligated to pay, in addition to the payment of up-front fees upon execution of the agreements, certain milestone payments as a product candidate proceeds from the submission of an investigational new drug application through approval for commercial sale and beyond. As of March 31, 2020, the Company may be obligated to make up to $3.2 billion in future development, regulatory, commercial, royalty and up-front milestone payments associated with its license and collaboration agreements. For the three months ended March 31, 2020 and 2019, the Company recognized up-front, milestone, and other expenses of $8.5 million and $1.1 million, respectively, as research and development expense in the accompanying unaudited condensed consolidated statement of operations and comprehensive loss.

4. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

In February 2020, the Company entered into an agreement with Vifor (International) Ltd. to sell the rare pediatric disease Priority Review Voucher (“PRV”) it received from the FDA in connection with the approval of VYONDYS 53. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2020, the Company completed its sale of the PRV and received proceeds of $108.1 million, net of commission, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale.

5. FAIR VALUE MEASUREMENTS

The Company has certain financial assets and liabilities that are recorded at fair value which have been classified as Level 1, 2 or 3 within the fair value hierarchy as described in the accounting standards for fair value measurements.

- Level 1 — quoted prices for identical instruments in active markets;
- Level 2 — quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active, and model-derived valuations in which all significant inputs and significant value drivers are observable in active markets; and
- Level 3 — valuations derived from valuation techniques in which one or more significant value drivers are unobservable.

The tables below present information about the Company's financial assets and liabilities that are measured and carried at fair value and indicate the level within the fair value hierarchy of valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of March 31, 2020			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 1,047,762	$ 1,047,762	$ —	$ —
Government and government agency bonds	719,046	719,046	—	—
Strategic equity investments	33,697	3,697	—	30,000
Certificates of deposit	1,001	1,001	—	—
Total assets	$ 1,801,506	$ 1,771,506	$ —	$ 30,000
Liabilities				
Contingent consideration	$ 5,200	$ —	$ —	$ 5,200
Total liabilities	$ 5,200	$ —	$ —	$ 5,200

	Fair Value Measurement as of December 31, 2019			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Money market funds	$ 203,410	$ 203,410	$ —	$ —
Government and government agency bonds	809,159	809,159	—	—
Strategic equity investments	31,937	1,937	—	30,000
Certificates of deposit	1,001	1,001	—	—
Total assets	$ 1,045,507	$ 1,015,507	$ —	$ 30,000
Liabilities				
Contingent consideration	$ 5,200	$ —	$ —	$ 5,200
Total liabilities	$ 5,200	$ —	$ —	$ 5,200

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds, government and government agency bonds, the Company's strategic investment in Lysogene S.A. and certificates of deposit. Certain of the government and government agency bonds are traded fixed income securities and are presented as cash equivalents on the unaudited condensed consolidated balance sheets as of March 31, 2020.

The Company's asset with fair value categorized as Level 3 within the fair value hierarchy consists of a strategic investment in Series A preferred stock of Lacerta Therapeutics, Inc. ("Lacerta"). For more information related to Lacerta, please read *Note 3, License and Collaboration Agreements* of the Company's Annual Report on Form 10-K for the year ended December 31, 2019. The fair value of the asset was initially based on a cost approach corroborated by the Black-Scholes option pricing model. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. At the end of each reporting period, the fair value will be adjusted if Lacerta issues similar or identical equity securities or when there is a triggering event for impairment. There were no changes in the fair value of the Lacerta strategic investment during the three months ended March 31, 2020.

The Company's contingent consideration liability with fair value categorized as Level 3 within the fair value hierarchy relate to the regulatory-related contingent payments to Myonexus selling shareholders as well as to an academic institution under a separate license agreement that meet the definition on a derivative. For more information related to Myonexus, please read *Note 3, License and Collaboration Agreements* of the Company's Annual Report on Form 10-K for the year ended December 31, 2019. This amount was estimated through an income approach based on the probability-weighted expected cash flows that incorporated industry-based probability adjusted assumptions relating to the achievement of the milestone and thus the likelihood of making the payments. This fair value measurement was based upon significant inputs not observable in the market and therefore represented a Level 3 measurement. At the end of each reporting period, the fair value is adjusted to reflect the most current assumptions through earnings. There were no changes in the fair value of the contingent consideration during the three months ended March 31, 2020. As of March 31, 2020, the contingent consideration was recorded as a non-current liability on the Company's unaudited condensed consolidated balance sheets.

The carrying amounts reported in the unaudited condensed consolidated balance sheets for cash and cash equivalents, accounts receivable and accounts payable approximated fair value because of the short-term maturity of these financial instruments.

6. CASH, CASH EQUIVALENTS AND MARKETABLE SECURITIES

The following table summarizes the Company's financial assets with maturities of less than 90 days from the date of purchase included in cash equivalents in the unaudited condensed consolidated balance sheets for each of the periods indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Money market funds	$ 1,047,762	$ 203,410
Government and government agency bonds	312,106	519,491
Total	$ 1,359,868	$ 722,901

It is the Company's policy to mitigate credit risk in its financial assets by maintaining a well-diversified portfolio that limits the amount of exposure as to maturity and investment type. The weighted average maturity of the Company's available-for-sale securities as of March 31, 2020 and December 31, 2019 was approximately one and two months, respectively.

The following tables summarize the Company's cash, cash equivalents and short-term investments for each of the periods indicated:

	As of March 31, 2020			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
Cash and money market funds	$ 1,452,106	$ —	$ —	$ 1,452,106
Government and government agency bonds	718,175	871	—	719,046
Total cash, cash equivalents and investments	$ 2,170,281	$ 871	$ —	$ 2,171,152
As reported:				
Cash and cash equivalents	$ 1,763,925	$ 287	$ —	$ 1,764,212
Short-term investments	406,356	584	—	406,940
Total cash, cash equivalents and investments	$ 2,170,281	$ 871	$ —	$ 2,171,152

	As of December 31, 2019			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
	(in thousands)			
Cash and money market funds	$ 315,589	$ —	$ —	$ 315,589
Government and government agency bonds	809,090	71	(2)	809,159
Total cash, cash equivalents and investments	$ 1,124,679	$ 71	$ (2)	$ 1,124,748
As reported:				
Cash and cash equivalents	$ 835,044	$ 36	$ —	$ 835,080
Short-term investments	289,635	35	(2)	289,668
Total cash, cash equivalents and investments	$ 1,124,679	$ 71	$ (2)	$ 1,124,748

7. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

The following table summarizes the components of the Company's accounts receivable for the periods indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Product sales receivable, net of discounts and allowances	$ 89,960	$ 90,409
Collaboration receivable	16,445	—
Government contract receivables	470	470
Total accounts receivable, net	$ 106,875	$ 90,879

The balance for government contract receivables for both periods presented is subject to government audit and will not be collected until the completion of the audit.

The following tables summarize an analysis of the change in reserves for discounts and allowances for each of the periods indicated:

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
	(in thousands)				
Balance, as of December 31, 2019	$ 588	$ 44,738	$ 1,506	$ 4,671	$ 51,503
Provision	2,435	12,083	1,373	2,103	17,994
Payments/credits	(2,336)	(10,979)	(1,291)	(4,023)	(18,629)
Balance, as of March 31, 2020	$ 687	$ 45,842	$ 1,588	$ 2,751	$ 50,868

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
	(in thousands)				
Balance, as of December 31, 2018	$ 1,378	$ 24,276	$ 538	$ 2,318	$ 28,510
Provision	2,566	9,429	936	1,064	13,995
Payments/credits	(3,049)	(5,191)	(791)	(2,015)	(11,046)
Balance, as of March 31, 2019	$ 895	$ 28,514	$ 683	$ 1,367	$ 31,459

The following table summarizes the total reserves included in the Company's unaudited condensed consolidated balance sheets for the periods indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Reduction to accounts receivable	$ 4,147	$ 6,254
Component of accrued expenses	46,721	45,249
Total reserves	$ 50,868	$ 51,503

8. INVENTORY

The following table summarizes the components of the Company's inventory for the period indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Raw materials	$ 81,736	$ 82,030
Work in progress	84,521	88,031
Finished goods	6,911	1,318
Total inventory	$ 173,168	$ 171,379

9. OTHER ASSETS

The following table summarizes the Company's other current assets for each of the periods indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 61,890	$ 54,276
Prepaid clinical and pre-clinical expenses	11,722	8,263
Prepaid maintenance services	5,849	4,366
Prepaid income tax	5,303	2,114
Leasehold improvement receivable	3,059	3,059
Prepaid insurance	1,420	2,573
Prepaid research expenses	1,043	2,007
Other	5,867	5,249
Total other current assets	$ 96,153	$ 81,907

The following table summarizes the Company's other non-current assets for each of the periods indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 135,963	$ 122,091
Strategic investments	33,697	31,937
Restricted cash and investments	9,566	9,566
Prepaid clinical expenses	5,164	4,665
Other	2,415	5,600
Total other non-current assets	$ 186,805	$ 173,859

10. ACCRUED EXPENSES

The following table summarizes the Company's accrued expenses for each of the periods indicated:

	As of March 31, 2020	As of December 31, 2019
	(in thousands)	
Product revenue related reserves	$ 46,721	$ 45,249
Accrued contract manufacturing costs	26,098	27,622
Accrued clinical and pre-clinical costs	20,292	18,010
Accrued employee compensation costs	18,993	43,240
Accrued Roche transaction costs	15,577	—
Accrued professional fees	11,219	10,707
Accrued milestone expense	8,893	18,390
Accrued royalties	6,400	6,301
Accrued property and equipment	5,887	1,181
Accrued collaboration cost sharing	4,821	9,000
Accrued interest expense	3,183	1,045
Other	8,214	4,782
Total accrued expenses	$ 176,298	$ 185,527

11. STOCK-BASED COMPENSATION

The following table summarizes the Company's stock awards granted for each of the periods indicated:

	For the Three Months Ended March 31,			
	2020		2019	
	Grants	Weighted Average Grant Date Fair Value	Grants	Weighted Average Grant Date Fair Value
Stock options	1,085,330	$ 56.80	990,234	$ 76.44
Restricted stock units	498,910	$ 114.28	324,630	$ 143.14

Stock-based Compensation Expense

For the three months ended March 31, 2020 and 2019, total stock-based compensation expense was $24.0 million and $16.1 million, respectively. The following table summarizes stock-based compensation expense by function included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended March 31,	
	2020	2019
	(in thousands)	
Research and development	$ 9,249	$ 5,087
Selling, general and administrative	14,775	11,052
Total stock-based compensation expense	$ 24,024	$ 16,139

The following table summarizes stock-based compensation expense by grant type included within the unaudited condensed consolidated statements of operations and comprehensive loss:

	For the Three Months Ended March 31,	
	2020	2019
	(in thousands)	
Stock options	$ 15,355	$ 11,457
Restricted stock awards/units	6,745	3,916
Employee stock purchase plan	1,924	766
Total stock-based compensation expense	$ 24,024	$ 16,139

12. OTHER INCOME (LOSS)

The following table summarizes other income (loss) for the periods indicated:

	For the Three Months Ended March 31,	
	2020	2019
	(in thousands)	
Interest expense	$ (13,615)	$ (7,335)
Interest income	2,139	2,361
Amortization of investment discount	2,964	4,332
Gain from sale of Priority Review Voucher	108,069	—
Other income	1,092	470
Total other income (loss), net	$ 100,649	$ (172)

13. LEASES

The Company has real estate operating leases in Cambridge, Andover and Burlington, Massachusetts and Dublin and Columbus, Ohio that provide for scheduled annual rent increases throughout each lease's term. There have been no significant changes to the real estate leases in the three months ended March 31, 2020. The Company has also identified embedded leases in its manufacturing and supply agreements with Catalent, Inc. ("Catalent") and Thermo Fisher Scientific, Inc. ("Thermo"). For additional details relating to these two agreements, please read *Note 21, Commitments and Contingencies* of the Annual Report on Form 10-K for the year ended December 31, 2019.

Catalent, Inc.

In October 2018 and February 2019, the Company entered into a manufacturing collaboration agreement and a manufacturing and supply agreement, respectively, (collectively, the "Catalent Agreements") with Catalent, Inc., formerly Paragon Bioservices, Inc. ("Catalent"). Pursuant to the terms of the Catalent Agreements, Catalent agreed to provide the Company with two dedicated clean room suites and an option to gain access to two additional dedicated clean room suites for its gene therapy programs. In September 2019, the Company exercised the option to gain access to the two additional dedicated clean room suites. The Catalent Agreements will expire on December 31, 2024, unless the Company and Catalent mutually agree to extend the term. The Company has the ability to terminate the Catalent Agreements prior to expiration, subject to potential additional financial consideration.

The Company concluded that the Catalent Agreements contain an embedded lease as the Company controls the use of the dedicated clean room suites and related equipment therein. The Company also determined that it did not control the facility or related equipment during construction and, thus, the lease did not fall in the scope of "build-to-suit" accounting.

The lease on two of the four dedicated clean room suites commenced during the quarter ended March 31, 2020, which is when the dedicated clean room suites became available for use by the Company. Accordingly, the fixed and in-substance fixed contract consideration associated with the two dedicated clean room suites was allocated to the lease and non-lease components. The lease component was determined based on the estimated standalone price of the leased clean rooms and the associated equipment based on available market and specific cost information. The non-lease component was determined using the residual estimation approach as the standalone price of the gene therapy manufacturing and supply services provided by Catalent is highly variable. Consequently, as of March 31, 2020, the Company recorded a right of use ("ROU") asset of $27.0 million and a lease liability of $24.3 million relating to these two dedicated clean room suites. The ROU asset and lease liability were based on the present value of estimated future payments associated with the lease component of the clean room suites at a discount rate of 8%, representing the rate at which the Company could borrow on a collateralized basis the amount of the lease payments in a similar term. The difference between the ROU asset and the lease liability results from certain prepayments made to Catalent by the Company prior to the commencement of the leases. The weighted average remaining lease term on the leases for the two dedicated clean room suites is approximately 4.8 years. During the first quarter of 2020, the Company recorded operating lease costs of $0.8 million and variable lease costs of $0.9 million, relating to these two dedicated clean room suits which was included in the research and development expense in the accompanying unaudited condensed consolidated statements of operations and comprehensive loss. The total lease liability payment is expected to be paid in equal installments through the five-year term of the agreement.

The lease on the remaining two clean room suites has not commenced as of March 31, 2020 because the clean room suites at the Catalent facility are not yet available for use by the Company. Accordingly, cumulative payments totaling $18.0 million made to Catalent relating to the remaining two clean room suites have been recorded as an other asset in the accompanying consolidated balance sheets, a portion of which will be considered in the initial measurement of the cost of the ROU asset at the lease commencement date, currently anticipated to occur in the fourth quarter of 2020.

Thermo Fisher Scientific, Inc.

The Company entered into a development, commercial manufacturing and supply agreement and, subsequently, entered into the first amendment (collectively, the "Thermo Agreements") with Thermo Fisher Scientific, Inc., formerly Brammer Bio MA, LLC ("Thermo") in June 2018 and May 2019, respectively. Pursuant to the terms of the Thermo Agreements, Thermo agreed to provide the Company with access to clinical and commercial manufacturing capacity for its gene therapy programs. The Thermo Agreements will continue for a period of six years following the first regulatory approval of a product manufactured under the agreements. The Company has the ability to terminate the Thermo Agreements prior to expiration but would be required to continue remitting capacity access fees to Thermo for up to eight additional quarters.

The Company determined that the Thermo Agreements contain an embedded lease because the Company controls the use of the facility and related equipment therein. The Company also determined that it does not control the facility or related equipment during construction and, thus, the lease does not fall in the scope of "build-to-suit" accounting. As of March 31, 2020, the embedded lease in the Thermo Agreements has not commenced as the clean room suites and related equipment therein at the Thermo facility have not been available for use by the Company. Accordingly, total cumulative payments made to Thermo of $86.3 million have been recorded as other assets in the accompanying unaudited condensed consolidated balance sheets and the lease portion of these

prepayments will be considered in the initial measurement of the cost of the ROU asset at the lease commencement date, currently anticipated to occur in the second quarter of 2020.

14. NET LOSS PER SHARE

Basic net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock outstanding. Diluted net loss per share is computed by dividing net loss by the weighted-average number of shares of common stock and dilutive common stock equivalents outstanding. For the three months ended March 31, 2020 and 2019, there were no differences between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive due to the net loss position and, therefore, would be excluded from the diluted net loss per share calculation.

	For the Three Months Ended March 31,	
	2020	2019
	(in thousands, except per share amounts)	
Net loss	$ (17,492)	$ (76,643)
Weighted-average common shares outstanding - basic	76,432	71,731
Effect of dilutive securities*	—	—
Weighted-average common shares outstanding - diluted	76,432	71,731
Net loss per share - basic and diluted	$ (0.23)	$ (1.07)

* For the three months ended March 31, 2020 and 2019, stock options, RSAs, RSUs, and ESPP to purchase 10.5 million and 9.8 million shares of the Company's common stock, respectively, were excluded from the diluted net loss per share calculation as their effect would have been anti-dilutive. The Company accounts for the effect of the 2024 Notes on diluted net earnings per share using the if-converted method as they may be settled in cash or shares at the Company's option. While the closing price on March 31, 2020 exceeded the conversion price of $73.42, the potential shares issuable under the 2024 Notes were excluded from the calculation of diluted loss per share as they were anti-dilutive using the if-converted method. In the period of conversion, the 2024 Notes will have no impact on diluted net earnings (loss) if they are settled in cash and will have an impact on diluted earnings per share if the Notes are settled in shares upon conversion and when the Company in an income position.

15. COMMITMENTS AND CONTINGENCIES

Manufacturing Obligations

The following table summarizes the aggregate non-cancelable contractual obligations arising from the Company's manufacturing obligations:

	As of March 31, 2020 (in thousands)
2020 (April - December)	$ 334,096
2021	192,121
2022	62,409
2023	58,553
2024	58,309
Thereafter	149,350
Total manufacturing commitments*	$ 854,838

*Total manufacturing commitments include both Catalent and Thermo Agreements. Related to the embedded lease for the two clean room suites at Catalent that have commenced as of March 31, 2020, the Company recorded an ROU asset and a lease liability. Please see *Note 13. Leases* for further details.

Additionally, should the Company obtain regulatory approval for any drug product candidate produced as a part of the Company's manufacturing obligations above, additional minimum batch requirements with the respective manufacturing parties would be required.

Litigation

In the normal course of business, the Company may from time to time be named as a party to various legal claims, actions and complaints, including matters involving securities, employment, intellectual property, effects from the use of therapeutics utilizing its technology, or others. For example, on August 30, 2019, Plaintiff Andrew Salinger filed a putative class action complaint against

the Company and two of its current officers, Douglas S. Ingram and Sandesh Mahatme (collectively, the "Defendants"), in the United States District Court for the Southern District of New York. The complaint alleged that the Defendants violated Section 10(b) of the Securities Exchange Act of 1934, as amended ("Exchange Act"), and SEC Rule 10b-5 promulgated thereunder, as well as Section 20(a) of the Exchange Act, in connection with the Company's disclosures related to golodirsen. The proposed class consisted of all persons or entities who acquired Company securities between September 6, 2017 and August 19, 2019. On December 17, 2019, the district court appointed Bernard Portnoy as lead plaintiff, and set a briefing schedule requiring the amended complaint to be filed on February 18, 2020 and requiring Defendants to answer or otherwise respond to the amended complaint on April 17, 2020. On February 13, 2020, the lead plaintiff filed a Notice of Voluntary Dismissal of his claims against all Defendants. On April 22, 2020, the Court endorsed the Notice of Voluntary Dismissal and closed the case.

On January 7, 2020, Plaintiff Al Lutzker filed a stockholder derivative complaint, purportedly on behalf of the Company, against two of the Company's current officers, Douglas S. Ingram and Sandesh Mahatme, and six current members of Company's Board of Directors, M. Kathleen Behrens, Richard J. Barry, Michael W. Bonney, Mary Ann Gray, Claude Nicaise, and Hans Wigzell (collectively, the "Defendants"), in the United States District Court for the District of Delaware. The complaint asserted claims for breach of fiduciary duty, insider selling, unjust enrichment, waste of corporate assets, and violations of Section 14(a) of the Securities Exchange Act of 1934, and Rule 14a-9 promulgated thereunder, in connection with the Company's disclosures related to golodirsen. On April 7, 2020, the plaintiff filed a Notice of Voluntary Dismissal of his claims against all Defendants, and the court closed the case.

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

This section should be read in conjunction with our unaudited condensed consolidated financial statements and related notes included in Part I, Item 1 of this Quarterly Report on Form 10-Q and the section contained in our Annual Report on Form 10-K for the year ended December 31, 2019 under the caption "Part II-Item 7 — Management's Discussion and Analysis of Financial Condition and Results of Operations". This discussion contains certain forward-looking statements, which are often identified by words such as "believe," "anticipate," "expect," "intend," "plan," "will," "may," "estimate," "could," "continue," "ongoing," "predict," "potential," "likely," "seek" and other similar expressions, as well as variations or negatives of these words. These statements relate to our future plans, objectives, expectations, intentions and financial performance and the assumptions that underlie these statements. These forward-looking statements include, but are not limited to

- *the expected or potential impact of the COVID-19 pandemic on our business, including our commercial sales, ongoing and planned clinical trials, manufacturing and operations;*
- *our expectations regarding the continued growth of our business operations due, in part, to the commercialization of new pharmaceutical products;*
- *our technologies and programs, including those with strategic partners, and their respective potential benefits, including the goal of exon skipping to create the production of an internally truncated but functional dystrophin protein and the goal of SRP-9003 to restore the dystrophin associated protein complex;*
- *our expectation that our partnerships with manufacturers will support our clinical and commercial manufacturing capacity for our micro-dystrophin Duchenne muscular dystrophy gene therapy programs and Limb-girdle muscular dystrophy programs, while also acting as a manufacturing platform for potential future gene therapy programs, and our belief that our current network of manufacturing partners are able to fulfil the requirements of our commercial plan;*
- *our plan to continue building out our network for commercial distribution in jurisdictions in which our products are approved;*
- *estimated timelines and milestones for 2020 and beyond, including having safety and dosing insights for SRP-5051 in the second half of 2020, the plan to commence a trial evaluating SRP-9001 using commercial supply in 2020, pending regulatory feedback, having the results from the second cohort of SRP-9003 and making a dose selection in the third quarter of 2020, and completing dosing in LYS-SAF 302 by the middle of 2020;*
- *our plan to expand our pipeline through internal research and development and through strategic transactions;*
- *the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for EXONDYS 51 and VYONDYS 53 in confirmatory trials;*
- *our plan to evaluate future engagement with the European Medicines Agency (the "EMA") on potential next steps for EMA approval of our products;*
- *our ability to further secure long-term supply of our commercial products and our product candidates to satisfy our planned commercial, early access programs and clinical needs;*
- *the impact of regulations and regulatory decisions by the U.S. Food and Drug Administration and other regulatory agencies on our business, as well as the development of our product candidates and our financial and contractual obligations;*
- *the possible impact of any competing products on the commercial success of EXONDYS 51, VYONDYS 53 and our product candidates and our ability to compete against such products;*
- *our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future;*
- *the extent of protection that our patents provide and our pending patent applications may provide, if patents issue from such applications, to our technologies and programs, and our ability to obtain and maintain patent protection for our technologies and programs;*
- *our plans and ability to file and progress to issue additional patent applications to enhance and protect our new and existing technologies and programs;*
- *our estimates regarding how long our currently available cash and cash equivalents will be sufficient to finance our operations and business plans and statements about our future capital needs;*
- *our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;*

- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*
- *our expectation regarding the impact of environmental laws and regulations on our business; and*
- *our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements.*

We undertake no obligation to update any of the forward-looking statements contained in this Quarterly Report on Form 10-Q after the date of this report, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission. We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Quarterly Report on Form 10-Q. The forward-looking statements contained in this Quarterly Report on Form 10-Q, and other written and oral forward-looking statements made by us from time to time, are subject to certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements, including the risks, uncertainties and assumptions identified under the heading "Risk Factors" in this Quarterly Report on Form 10-Q.

Overview

We are a commercial-stage biopharmaceutical company focused on helping patients through the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic therapeutic modalities for the treatment of rare diseases. Applying our proprietary, highly-differentiated and innovative technologies, and through collaborations with our strategic partners, we are developing potential therapeutic candidates for a broad range of diseases and disorders, including Duchenne muscular dystrophy ("DMD"), Limb-girdle muscular dystrophies ("LGMDs"), Mucopolysaccharidosis type IIIA ("MPS IIIA") and other neuromuscular and central nervous system ("CNS") related disorders.

Our first commercial product, EXONDYS 51 (eteplirsen) Injection ("EXONDYS 51"), was granted accelerated approval by the U.S. Food and Drug Administration ("FDA") on September 19, 2016. EXONDYS 51 is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 uses our phosphorodiamidate morpholino oligomer ("PMO") chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene.

Our second commercial product, VYONDYS 53 (golodirsen) Injection ("VYONDYS 53"), was granted accelerated approval by the FDA on December 12, 2019. VYONDYS 53 is indicated for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. VYONDYS 53 uses our PMO chemistry and exon-skipping technology to skip exon 53 of the dystrophin gene.

We are in the process of conducting various EXONDYS 51 and VYONDYS 53 clinical trials, including studies that are required to comply with our post-marketing FDA requirements/commitments to verify and describe the clinical benefit of EXONDYS 51 and VYONDYS 53.

A summary description of our main product candidates, including those in collaboration with our strategic partners, is as follows:

- *Casimersen* (SRP-4045) uses our PMO chemistry and exon-skipping technology to skip exon 45 of the DMD gene. Casimersen is designed to bind to exon 45 of dystrophin pre-mRNA, resulting in exclusion, or "skipping", of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 45 skipping. We are enrolling and dosing patients in ESSENCE (4045-301), our Phase 3 placebo controlled confirmatory trial in patients who have a confirmed mutation of the DMD gene that is amenable to exon 45 or 53 skipping using casimersen and golodirsen, respectively. On March 28, 2019, we announced results from our interim analysis of muscle biopsy endpoints comparing casimersen treatment to placebo in the ESSENCE study. In January 2020, we commenced a rolling submission of an NDA to the FDA seeking accelerated approval for casimersen.

- *SRP-5051* uses our next-generation chemistry platform, PPMO, and our exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051, a peptide conjugated PMO, is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein. In the fourth quarter of 2017, we commenced a first-in-human, single ascending dose, study for the treatment of DMD in patients who are amenable to exon 51 skipping. In 2019, we commenced a multiple ascending dose study for the treatment of DMD with SRP-5051 in patients who are amenable to exon 51 skipping, and we expect to have safety and dosing insights in the second half of 2020.
- *SRP-9001* (DMD, micro-dystrophin gene therapy program), aims to express micro-dystrophin – a smaller but still functional version of dystrophin. A unique, engineered micro-dystrophin is used because naturally-occurring dystrophin is too large to fit in an AAV vector. In the fourth quarter of 2017, an investigational new drug ("IND") application for the micro-dystrophin gene therapy program was cleared by the FDA, and a Phase 1/2a clinical trial in individuals with DMD was initiated. On October 3, 2018, Nationwide Children's Hospital ("Nationwide") presented what we believe to be positive results from the Phase 1/2a clinical trial in four individuals with DMD enrolled in the trial. On March 25, 2019, we presented nine-month functional and CK data from baseline from these four individuals, and twelve-month CK data from baseline from one of these individuals. In the fourth quarter of 2018, we commenced a randomized, double-blind, placebo-controlled trial of SRP-9001 with the goal to establish the functional benefits of micro-dystrophin expressions. We have dosed all 41 participants in that trial and have begun dosing participants in the crossover phase of the study. We plan to commence a trial evaluating SRP-9001 using commercial supply in 2020, pending regulatory feedback.
- *SRP-9003* (LGMD, gene therapy program). We are developing gene therapy programs for various forms of LGMDs. The most advanced of our LGMD product candidates, SRP-9003, is designed to transfer a gene that codes for and restores beta-sarcoglycan protein with the goal of restoring the dystrophin associated protein complex. It utilizes the AAVrh.74 vector system, the same vector used in the micro-dystrophin gene therapy program we are developing with Nationwide. A Phase 1/2a trial of SRP-9003 was commenced in the fourth quarter of 2018. On February 27, 2019, we announced positive two-month biopsy data from the first three-patient cohort dosed in the SRP-9003 trial, and on October 4, 2019, we announced positive nine-month functional data from these three patients. We have recently dosed one additional cohort of three patients at a higher dose per the study protocol. We expect to have the results from the second cohort and make a dose selection in the third quarter of 2020.
- *LYS-SAF 302*. We are collaborating with Lysogene S.A ("Lysogene") to develop a gene therapy, LYS-SAF302, to treat MPS IIIA. Lysogene is conducting a global Phase 2/3 clinical trial of LYS-SAF302 (AAVance) to evaluate the effectiveness of a one-time delivery of an AAVrh.10 virus carrying the N-SGSH gene. We expect to complete dosing in this trial by the middle of 2020.

Our pipeline includes more than 40 programs in various stages of pre-clinical and clinical development, reflecting our aspiration to apply our multifaceted approach and expertise in precision genetic medicine to make a profound difference in the lives of patients suffering from rare diseases.

Manufacturing, Supply and Distribution

We have developed proprietary state-of-the-art Chemistry, Manufacturing and Controls ("CMC") and manufacturing capabilities that allow synthesis and purification of our products and product candidates to support both clinical development as well as commercialization. Our current main focus in manufacturing is to continue scaling up production of our PMO-based therapies and optimizing manufacturing for PPMO and gene therapy-based product candidates. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these capabilities to support production of certain of our product candidates and their components. In 2017, we opened a facility in Andover, Massachusetts, which significantly enhanced our research and development manufacturing capabilities. However, we currently do not have internal Good Manufacturing Practices ("GMP") manufacturing capabilities. For our current and future manufacturing needs, we have entered into supply agreements with specialized contract manufacturing organizations (each a "CMO") to produce custom raw materials, the active pharmaceutical ingredients ("APIs"), drug product and finished goods for our products and product candidates for both commercial and clinical use. All of our CMO partners have extensive technical expertise, GMP experience and experience manufacturing our specific technology.

For our commercial DMD program, we have commenced work with our existing manufacturers to scale-up our process and increase our capacity. While there are a limited number of companies that can produce raw materials and APIs in the quantities and with the quality and purity that we require for our commercial products, based on our diligence to date, we believe our current network of manufacturing partners are able to fulfill these requirements, and are capable of expanding capacity as needed. Additionally, we have, and will continue to evaluate further relationships with, additional suppliers to increase overall capacity as well as further reduce risks associated with reliance on a limited number of suppliers for manufacturing.

Our commercial products are distributed in the U.S. through a limited network of home infusion specialty pharmacy providers that deliver the medication to patients and a specialty distributor that distributes our products to hospitals and hospital outpatient clinics. With respect to the pre-commercial distribution of our products to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute our products in certain countries through our ex-U.S. early access programs ("EAP"). We plan to continue building out our network for commercial distribution in jurisdictions in which our products are approved.

Our gene therapy manufacturing capabilities have been greatly enhanced through partnerships with Thermo Fisher Scientific, Inc., formerly Brammer Bio MA, LLC ("Thermo"), Catalent, Inc., formerly Paragon Bioservices, Inc. ("Catalent"), and Aldevron LLC ("Aldevron"). We have adopted a hybrid development and manufacturing strategy in which we are building internal manufacturing expertise relative to all aspects of AAV-based manufacturing, including gene therapy and gene editing supply, while closely partnering with first-in-class manufacturing partners to expedite development and commercialization of our gene therapy programs. We expect that our partnerships with Thermo and Catalent will support our clinical and commercial manufacturing capacity for our micro-dystrophin DMD gene therapy programs and LGMD programs, while also acting as a manufacturing platform for potential future gene therapy programs. The collaboration integrates process development, clinical production and testing, and commercial manufacturing. Aldevron is expected to provide GMP-grade plasmid for our SRP-9001 micro-dystrophin DMD gene therapy program and LGMD programs, as well as plasmid source material for future gene therapy programs, such as CMT, MPS IIIA and other neuromuscular and CNS related disorders.

Manufacturers and suppliers of our commercial products and product candidates are subject to the FDA's current GMP ("cGMP") requirements and other rules and regulations prescribed by foreign regulatory authorities. We depend on our third-party partners for continued compliance with cGMP requirements and applicable foreign standards.

Cash, Cash Equivalents and Investments

As of March 31, 2020, we had approximately $2,180.7 million of cash, cash equivalents and investments, consisting of $1,764.2 million of cash and cash equivalents, $406.9 of short-term investments, and $9.6 million of long-term restricted cash and investments. We believe that our balance of cash, cash equivalents and investments is sufficient to fund our current operational plan for at least the next twelve months.

The likelihood of our long-term success must be considered in light of the expenses, difficulties and delays frequently encountered in the development and commercialization of new pharmaceutical products, competitive factors in the marketplace, the risks associated with government sponsored programs and the complex regulatory environment in which we operate.

COVID-19 Pandemic

In December 2019, an outbreak of a novel strain of coronavirus ("COVID-19") originated in Wuhan, China and has since spread to a number of other countries, including the United States. On March 11, 2020, the World Health Organization characterized COVID-19 as a pandemic. The COVID-19 pandemic has presented a substantial public health and economic challenge around the world. Our business operations and financial condition and results have been impacted to varying degrees, and we expect the impact will continue in future quarters.

Commercialization: We have activated business continuity plans to minimize disruption to patients. There has been minimal disruption to our commercial and clinical supply chains and we continue to provide an uninterrupted supply of our approved or investigational therapies to patients. Although the vast majority of EXONDYS 51 patients are treated in home, and although we are working to increase the percentage of patients that can initiate treatment with VYONDYS 53 and EXONDYS 51 at home, the response to COVID-19 by healthcare providers has made it difficult for some patients to receive infusions or initiate treatment with our commercial products. For this and other reasons, such as potential delays in processing reauthorizations and modifications to program benefits by insurers, we expect that COVID-19 will impact our revenue.

Clinical Trials: With respect to our active gene therapy trials, all dosing for the 48-week analysis in our placebo controlled, blinded study to evaluate the safety and efficacy of SRP-9001 (Study 102) and in our high-dose arm for SRP-9003 to treat LGMD2E has occurred and we are working within the FDA guidance on clinical trial conduct during the pandemic to avoid or minimize delays in trial follow-up visits. For trials that involve weekly or monthly dosing, we are working to adapt protocols to allow for virtual interactions, while remaining compliant with Good Clinical Practice and continuing to advance these investigational treatments. The response to COVID-19 by healthcare providers is expected to delay site initiation, slow down enrollment, and make the ongoing collection of data for patients enrolled in studies more difficult or intermittent. For example, with respect to our planned trial evaluating SRP-9001 using commercial supply (Study 301), COVID-19 is expected to delay some necessary site initiation visits. However, we still intend to commence Study 301 in the second half of 2020.

Operations: We are operating under guidance from federal agencies, including the FDA and Centers for Disease Control and Prevention ("CDC"), which designate healthcare companies as "critical infrastructure" with a special responsibility to maintain normal work schedules. Our partners and suppliers share these special responsibilities to maintain operations and ensure the integrity of the supply chain. To protect the health of our employees, their families, and our communities, and in accordance with direction from state and local government authorities, we have taken temporary precautionary measures, including the institution of mandatory work-from-home for all employees and contingent workers, other than those who are facility-dependent. To date, our remote working arrangements have not impacted our ability to maintain critical business operations. Facility-dependent employees, including those

needed to maintain manufacturing and clinical research, are reporting to work under strict protocols designed to help those employees remain healthy.

We are continuing to assess the potential impact of the COVID-19 pandemic on our business, operations and financial condition and results. Despite careful tracking and planning, however, we are unable to accurately predict the extent of the impact of the pandemic on our business, results of operations and financial condition due to the uncertainty of future developments. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition will depend on future developments that are highly uncertain and cannot be accurately predicted, including new information that may emerge concerning COVID-19, the actions taken to contain it or treat its impact and the economic impact on local, regional, national and international markets. For additional information on the various risks posed by the COVID-19 pandemic, refer to Part II, Item 1A. Risk Factors of this Quarterly Report on Form 10-Q.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations is based upon our unaudited condensed consolidated financial statements included elsewhere in this report. The preparation of our unaudited condensed consolidated financial statements in accordance with accounting principles generally accepted in the United States requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenues and expenses and related disclosure of contingent assets and liabilities for the periods presented. Some of these judgments can be subjective and complex and, consequently, actual results may differ from these estimates. We believe that the estimates and judgments upon which we rely are reasonable based upon historical experience and information available to us at the time that we make these estimates and judgments. To the extent there are material differences between these estimates and actual results, our unaudited condensed consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates.

Due to the collaboration arrangement with F. Hoffman-La Roche Ltd. (“Roche”), we have added collaboration revenue recognition to our critical accounting policies and estimates as of March 31, 2020. As such, we believe the following accounting policies to be the most critical to the judgments and estimates used in the preparation of our unaudited condensed consolidated financial statements:

- revenue recognition;
- inventory; and
- income tax.

Revenue recognition – collaboration revenue

Our collaboration revenue is generated from our collaboration arrangement with Roche. For more information, please read *Note 3, Collaboration and License Agreements*. At the inception of a collaboration arrangement, we first assess whether the contractual arrangement is within the scope of ASC 808, *Collaborative Arrangements* (“ASC 808”), to determine whether the arrangement involves a joint operating activity and involves two (or more) parties that are both active participants in the activity and exposed to significant risks and rewards dependent on the commercial success of such activities. Then we determine whether the collaboration arrangement in its entirety represents a contract with a customer as defined by ASC 606, *Revenue from Contracts with Customers* (“ASC 606”). If only a portion of the collaboration arrangement is potentially with a customer, we apply the distinct good or service unit-of-account guidance in ASC 606 to determine whether there is a unit of account that should be accounted for under ASC 606. For the units of account in the collaboration arrangement that do not represent a vendor-customer relationship, we will (i) consider applying other GAAP, including by analogy, or (ii) if there is no appropriate analogy, consistently apply a reasonable and rational accounting policy election.

In general, by analogy to ASC 606, we identify the performance obligations within the collaboration arrangement and identify and allocate the transaction price we expect to receive on a relative standalone selling price basis to each performance obligation. The residual approach is used to determine estimated standalone selling prices when the selling price is uncertain. Variable consideration, consisting of development and regulatory milestones, will be included in the transaction price only if we expect to receive such consideration and if it is probable that the inclusion of the variable consideration will not result in a significant reversal in the cumulative amount of revenue recognized under the arrangement. Sales-based royalty and milestone payments are excluded from the transaction price we expect to receive until the underlying sales occur because the license to our intellectual property is deemed to be the predominant item to which the royalties or milestones relate as it is the primary driver of value in its collaboration arrangement.

For the recognition of revenue associated with each performance obligation, if we determine ASC 606 is not appropriate to apply by analogy, we will apply a reasonable, rational, and consistently applied accounting policy election to faithfully depict the

transfer of services to the collaboration partner over the estimated performance period. Up-front payments from a collaboration partner are recognized as deferred revenue when received and recognized as revenue over the estimated performance period. Reimbursement payments from a collaboration partner associated with research and development cost sharing provisions in a collaboration arrangement are recognized as the related expense is incurred and classified as an offset to research and development expense.

Our collaboration arrangements may contain options which provide the collaboration partner with the right to obtain additional licenses. If an arrangement contains customer options, by analogy to ASC 606, we evaluate the customer options to determine whether they represent material rights, which may include options to acquire additional goods or services for free or at a discount. If the customer options are determined to represent material rights, they are recognized as a separate performance obligation at inception of the arrangement. We allocate a portion of the transaction price of the collaboration arrangement to material rights based on the relative standalone selling price. Amounts allocated to material rights are not recognized as revenue until related options are exercised or expire.

Key assumptions to determine the standalone selling price of units of account in a collaboration arrangement include, but are not limited to, forecasted revenues, development timelines, incremental costs related to the arrangement, discount rates and probabilities of technical and regulatory success.

Other than revenue recognition, there have been no changes to our critical accounting policies and significant estimates as detailed in our Annual Report on Form 10-K for the year ended December 31, 2019.

Results of Operations for the Three Months Ended March 31, 2020 and 2019

The following table sets forth selected consolidated statements of operations data for each of the periods indicated:

	For the Three Months Ended March 31,		Change	Change
	2020	2019		
	(in thousands, except per share amounts)		$	%
Revenues:				
Products, net	$ 100,448	$ 87,011	$ 13,437	15%
Collaboration	13,226	—	13,226	NM*
Total revenues	113,674	87,011	26,663	31%
Costs and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	12,622	12,063	559	5%
Research and development	136,144	90,553	45,591	50%
Selling, general and administrative	82,768	60,566	22,202	37%
Amortization of in-licensed rights	166	216	(50)	(23)%
Total cost and expenses	231,700	163,398	68,302	42%
Operating loss	(118,026)	(76,387)	(41,639)	55%
Other income (loss):				
Gain from sale of Priority Review Voucher	108,069	—	108,069	NM*
Other expense, net	(7,420)	(172)	(7,248)	NM*
Total other income (loss)	100,649	(172)	100,821	NM*
Loss before income tax expense	(17,377)	(76,559)	59,182	(77)%
Income tax expense	115	84	31	37%
Net loss	$ (17,492)	$ (76,643)	$ 59,151	(77)%
Net loss per share - basic and diluted	$ (0.23)	$ (1.07)	$ 0.84	(78)%

* NM = Not Meaningful

Revenues

Revenues from product sales are recorded at the net sales price (transaction price), which includes estimates of variable consideration for which reserves are established and which result from Medicaid rebates, governmental chargebacks including Public Health Services chargebacks, prompt pay discounts, co-pay assistance and distribution fees. These reserves are based on the amounts earned or to be claimed on the related sales and are classified as reductions of accounts receivable (if no payments are required of us) or a current liability (if a payment is required of us). Our estimates take into consideration current contractual and statutory requirements. The amount of variable consideration included in the transaction price may be constrained and is included in the net sales price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Actual amounts of consideration ultimately received or paid may differ from our estimates. If actual results in the future vary from our estimates, we will adjust these estimates, which would affect net product revenue and net loss in the period such variances become known.

Net product revenues for our products for the three months ended March 31, 2020 increased by $13.4 million compared with the three months ended March 31, 2019. These increases primarily reflect increasing demand for our products in the U.S.

Collaboration revenue relates to our collaboration arrangement with Roche. In February 2020, we received an aggregate of approximately $1.2 billion in cash consideration from Roche, consisting of an up-front payment and an equity investment in our company. Of that amount, $342.7 million is being recognized as revenue on a straight-line basis over the performance period, estimated to be through the fourth quarter of 2023. For the three months ended March 31, 2020, we recognized $13.2 million of collaboration revenue. For more information, please read *Note 3, Collaboration and License Agreements*.

Cost of Sales (excluding amortization of in-licensed rights)

Our cost of sales (excluding amortization of in-licensed rights) primarily consists of royalty payments to BioMarin Pharmaceuticals, Inc. (“BioMarin”) and the University of Western Australia (“UWA”), inventory costs that relate to sales of our products and the related overhead costs. Prior to receiving regulatory approval for EXONDYS 51 and VYONDYS 53 by the FDA in September 2016 and December 2019, respectively, we expensed such manufacturing and material costs as research and development expenses. For VYONDYS 53 sold in the three months ended March 31, 2020, the majority of related manufacturing costs incurred had previously been expensed as research and development expenses, as such costs were incurred prior to the FDA approval of the products. For EXONDYS 51 sold in the three months ended March 31, 2020 and 2019, only part of the related manufacturing costs incurred had previously been expensed as research and development expenses. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the incremental inventory costs related to our products sold would have been approximately $5.6 million and $2.3 million for the three months ended March 31, 2020 and 2019, respectively.

The following table summarizes the components of our cost of sales for each of the periods indicated:

	For the Three Months Ended March 31,			
	2020	2019	Change	Change
	(in thousands)		$	%
Inventory costs related to products sold	$ 6,221	$ 7,608	$ (1,387)	(18)%
Royalty payments	6,401	4,455	1,946	44%
Total cost of sales	$ 12,622	$ 12,063	$ 559	5%

The cost of sales for the three months ended March 31, 2020 increased by $0.6 million, or 5%, compared with the same period in 2019. The increase was primarily driven by the following:

- $1.4 million decrease in inventory costs related to products sold primarily as a result of the increasing demand for our products, offset by write-offs of certain batches of EXONDYS 51 not meeting our quality specifications for the three months ended March 31, 2019, with no similar activity for the three months ended March 31, 2020.
- $1.9 million increase in royalty payments to BioMarin and UWA reflects increasing demand for our products.

Research and Development Expenses

Research and development expenses consist of costs associated with research activities as well as costs associated with our product development efforts, conducting pre-clinical trials, clinical trials and manufacturing activities. Direct research and development expenses associated with our programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants, up-front fees and milestones paid to third parties in connection with technologies that have not reached technological feasibility and do not have an alternative future use, and other external services, such as data management and statistical analysis support, and materials and supplies used in support of clinical programs. Indirect costs of our clinical programs include salaries, stock-based compensation and allocation of our facility- and technology-related costs.

Research and development expenses represent a substantial percentage of our total operating expenses. We do not maintain or evaluate and, therefore, do not allocate internal research and development costs on a project-by-project basis. As a result, a significant portion of our research and development expenses are not tracked on a project-by-project basis, as the costs may benefit multiple projects.

The following table summarizes our research and development expenses by project for each of the periods indicated:

	For the Three Months Ended March 31,			
	2020	2019	Change	Change
	(in thousands)		$	%
Gene therapies	$ 59,265	$ 18,322	$ 40,943	223%
Golodirsen (exon 53)	15,853	5,753	10,100	176%
Casimersen (exon 45)	9,815	6,180	3,635	59%
Up-front, milestone, and other expenses	8,533	1,122	7,411	NM*
Eteplirsen (exon 51)	5,810	8,803	(2,993)	(34)%
PPMO platform	3,528	4,927	(1,399)	(28)%
Collaboration cost-sharing	2,642	292	2,350	NM*
Other projects	969	2,047	(1,078)	(53)%
Internal research and development expenses	46,173	43,107	3,066	7%
Roche collaboration reimbursement	(16,444)	—	(16,444)	NM*
Total research and development expenses	$ 136,144	$ 90,553	$ 45,591	50%

*NM = Not Meaningful

The following table summarizes our research and development expenses by category for each of the periods indicated:

	For the Three Months Ended March 31,			
	2020	2019	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 80,820	$ 37,555	$ 43,265	115%
Compensation and other personnel expenses	26,119	19,731	6,388	32%
Facility- and technology-related expenses	13,251	11,143	2,108	19%
Stock-based compensation	9,249	5,087	4,162	82%
Up-front, milestone, and other expenses	8,533	1,122	7,411	NM*
Professional services	4,500	4,540	(40)	(1)%
Collaboration cost-sharing	2,642	292	2,350	NM*
Pre-clinical expenses	642	5,257	(4,615)	(88)%
Research and other	6,832	5,826	1,006	17%
Roche collaboration reimbursement	(16,444)	—	(16,444)	NM*
Total research and development expenses	$ 136,144	$ 90,553	$ 45,591	50%

* NM = Not Meaningful

Research and development expenses for the three months ended March 31, 2020 increased by $45.6 million, or 50%, compared with the three months ended March 31, 2019. The increase was primarily driven by the following:

- $43.3 million increase in clinical and manufacturing expenses primarily due to a continuing ramp-up of our micro-dystrophin program and our ESSENCE program. The increases were offset by a ramp-down of the PROMOVI trial in EXONDYS 51 and the Phase 1/2 trial in golodirsen;
- $6.4 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $2.1 million increase in facility- and technology-related expenses due to our continuing global expansion efforts;
- $4.2 million increase in stock-based compensation expense primarily driven by increases in headcount and stock price;
- $7.4 million increase in up-front, milestone and other expenses primarily due to $8.8 million of milestone expenses accrued to an academic institution during the three months ended March 31, 2020;
- $2.4 million increase in collaboration cost sharing with Genethon on its micro-dystrophin drug candidates and Lysogene S.A on its MPS IIIA drug candidates;
- $4.6 million decrease in pre-clinical expenses primarily due to completion of certain toxicology studies in our PPMO platform;
- $1.0 million increase in research and other primarily driven by an increase in sponsored research with academic institutions; and
- $16.4 million offset to expense associated with a collaboration reimbursement from Roche.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist of salaries, benefits, stock-based compensation and related costs for personnel in our executive, finance, legal, information technology, business development, human resources, commercial and other general and administrative functions. Other general and administrative expenses include an allocation of our facility- and technology-related costs and professional fees for legal, consulting and accounting services.

The following table summarizes selling, general and administrative expenses by category for each of the periods indicated:

	For the Three Months Ended March 31,			
	2020	2019	Change	Change
	(in thousands)		$	%
Professional services	$ 31,124	$ 16,827	$ 14,297	85%
Compensation and other personnel expenses	26,792	24,465	2,327	10%
Stock-based compensation	14,775	11,052	3,723	34%
Facility- and technology-related expenses	7,050	5,310	1,740	33%
Other	3,027	2,912	115	4%
Total selling, general and administrative expenses	$ 82,768	$ 60,566	$ 22,202	37%

Selling, general and administrative expenses for the three months ended March 31, 2020 increased by $22.2 million, or 37%, compared with the three months ended March 31, 2019. This was primarily driven by the following:

- $14.3 million increase in professional services primarily due to a transaction fee for the Roche transaction;
- $2.3 million increase in compensation and other personnel expenses primarily due to a net increase in headcount;
- $3.7 million increase in stock-based compensation primarily due to increases in headcount and stock price; and
- $1.7 million increase in facility- and technology-related expense primarily due to continuing global expansion.

Amortization of In-licensed Rights

Amortization of in-licensed rights relate to the agreements we entered into with BioMarin and UWA in July 2017 and April 2011, respectively. We recorded an in-licensed right asset of approximately $6.6 million in 2017 as a result of the settlement and license agreements with BioMarin. Additionally, following the first sale of EXONDYS 51 in September 2016 and VYONDYS 53 in December 2019, we recorded an in-licensed right asset of $1.0 million and $0.5 million, respectively, related to the license agreement with UWA. Each in-licensed right is being amortized on a straight-line basis over the life of the patent from the first commercial sale of each product. For both the three months ended March 31, 2020 and 2019, we recorded amortization of in-licensed rights of approximately $0.2 million.

Gain from Sale of Priority Review Voucher

In February 2020, we entered into an agreement with Vifor (International) Ltd. to sell the rare pediatric disease Priority Review Voucher ("PRV") we received from the FDA in connection with the approval of VYONDYS 53. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2020, we completed our sale of the PRV and received proceeds of $108.1 million, net of commission, which was recorded as a gain from sale of the PRV as it did not have a carrying value at the time of the sale. There was no similar activity during the three months ended March 31, 2019.

Other expense, net

Other expense, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense on our debt facilities, amortization of investment discount, and unrealized gain from our investment in Lysogene. Our cash equivalents and investments consist of money market funds, government and government agency debt securities, and certificates of deposit. Interest expense includes interest accrued on our convertible notes and term loan.

For the three months ended March 31, 2020, other expense, net increased by approximately $7.2 million compared with the three months ended March 31, 2019. The increase primarily reflected the interest expense on our debt facilities entered into in December 2019.

Income tax expense

Income tax expense for both the three months ended March 31, 2020 and 2019 was approximately $0.1 million, both of which relate to state taxes.

Liquidity and Capital Resources

The following table summarizes our financial condition for each of the periods indicated:

	As of March 31, 2020	As of December 31, 2019	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 1,764,212	$ 835,080	$ 929,132	111%
Short-term investments	406,940	289,668	117,272	40%
Restricted cash and investments	9,566	9,566	—	0%
Total cash, cash equivalents and investments	$ 2,180,718	$ 1,134,314	$ 1,046,404	92%
Borrowings:				
Long-term debt	$ 240,487	$ 240,004	$ 483	0%
Convertible debt	447,466	441,896	5,570	1%
Total borrowings	$ 687,953	$ 681,900	$ 6,053	1%
Working capital				
Current assets	$ 2,547,348	$ 1,468,913	$ 1,078,435	73%
Current liabilities	306,429	264,767	41,662	16%
Total working capital	$ 2,240,919	$ 1,204,146	$ 1,036,773	86%

For the period ended December 31, 2019, our principal sources of liquidity were derived from proceeds from product sales of EXONDYS 51 and debt and equity financings. For the period ended March 31, 2020, our principal sources of liquidity were primarily derived from our collaboration arrangement with Roche, net proceeds from sale of the PRV and product sales of our products. Our principal uses of cash are research and development expenses, selling, general and administrative expenses, investments, capital expenditures, business development transactions and other working capital requirements. The increase in our working capital primarily resulted from cash received from Roche offset by overall use of cash in operating activities. For more information for the Roche transaction, please read *Note 3, License and Collaboration Agreements*.

Our future expenditures and capital requirements may be substantial and will depend on many factors, including but not limited to the following:

- our ability to continue to generate revenues from sales of EXONDYS 51, VYONDYS 53, and potential future products;
- the timing and costs associated with our global expansion;
- the timing and costs of building out our manufacturing capabilities;
- the timing of advanced payments related to our future inventory commitments and manufacturing obligations;
- the timing and costs associated with our clinical trials and pre-clinical trials;
- the attainment of milestones and our obligations to make milestone payments to Myonexus' selling shareholders StrideBio, BioMarin, Lysogene, Lacerta, Nationwide, UWA and other institutions;
- repayment of outstanding debt; and
- the costs of filing, prosecuting, defending and enforcing patent claims and our other intellectual property rights.

Our cash requirements are expected to continue to increase as we advance our research, development and commercialization programs and we expect to seek additional financings primarily from, but not limited to, the sale and issuance of equity, debt securities, the licensing or sale of our technologies or additional government contracts. We cannot provide assurances that financing will be available when and as needed or that, if available, the financings will be on favorable or acceptable terms. If we are unable to obtain additional financing when and if we require, this would have a material adverse effect on our business and results of operations. To the extent we issue additional equity securities, our existing stockholders could experience substantial dilution.

Cash Flows

	For the Three Months Ended March 31,			
	2020	2019	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ 627,790	$ (146,233)	$ 774,023	NM*
Investing activities	(17,680)	135,231	(152,911)	(113)%
Financing activities	319,022	377,563	(58,541)	(16)%
Increase in cash and cash equivalents	$ 929,132	$ 366,561	$ 562,571	153%

*NM = Not Meaningful

Operating Activities.

Cash provided by operating activities was $627.8 million for the three months ended March 31, 2020. Cash used in operating activities for the three months ended March 31, 2019 was $146.2 million. The favorable change was primarily driven by the following:

- $819.8 million net increase in deferred revenue primarily as a result of the collaboration arrangement with Roche; and
- $12.1 million increase in non-cash adjustment.

The increases were partially offset by:

- $48.9 million increase in net loss, excluding the gain from the sale of the PRV, primarily driven by increases in research and development expense and selling, general, and administrative expense, offset by increases in net product revenues and collaboration revenue; and
- $8.9 million net decrease in use of operating assets and liabilities, excluding deferred revenue.

Investing Activities.

Cash used in investing activities was $17.7 million for the three months ended March 31, 2020. Cash provided by investing activities for the three months ended March 31, 2019 was $135.2 million. The unfavorable change was driven by a decrease of $396.8 million in proceeds from the sale or maturity of available-for-sale securities, which was partially offset by:

- $129.0 million decrease in purchase of available-for-sale securities;
- $108.1 million increase as a result of the sale of the PRV; and
- $7.1 million decrease in purchase of property and equipment.

Financing Activities.

Cash provided by financing activities decreased by $58.5 million for the three months ended March 31, 2020 compared with the three months ended March 31, 2019, primarily driven by the following:

- $365.3 million decrease in proceeds from the sale of common stock;
- $4.8 million decrease in proceeds from the exercise of options and the purchase of stock under our Employee Stock Purchase Program; and
- $4.8 million increase in taxes paid related to net share settlement of equity awards.

The decreases were partially offset by:

- $316.3 million increase in proceeds from the issuance of common stock to Roche.

Off-Balance Sheet Arrangements

During the periods presented, we did not have any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or for another contractually narrow or limited purpose.

Contractual Payment Obligations

In our continuing operations, we have entered into long-term contractual arrangements from time to time for our facilities, the provision of goods and services, and acquisition of technology access rights, among others. The following table presents contractual obligations arising from these arrangements as of March 31, 2020:

	Payment Due by Period				
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
	(in thousands)				
Debt obligations (1)	$ 939,762	$ 30,095	$ 60,190	$ 849,477	$ —
Lease obligations (2)	64,917	11,799	23,609	22,793	6,716
Manufacturing obligations (3)	854,838	392,636	210,567	115,862	135,773
Total contractual obligations and contingencies	$ 1,859,517	$ 434,530	$ 294,366	$ 988,132	$ 142,489

(1) Interest is included.

(2) Lease obligations only include real estate leases. The leases embedded in certain supply agreements are included in the manufacturing obligations.

(3) Manufacturing obligations include agreements to purchase goods and services that are enforceable and legally binding or subject to cancellation fees and that specify all significant terms. Manufacturing obligations relate primarily to our commercialization of EXONDYS 51 and VYONDYS 53, and clinical programs for DMD as well as our gene therapy programs.

Milestone Obligations

For product candidates that are currently in various research and development stages, we may be obligated to make up to $3.2 billion of future development, regulatory, and commercial, royalty and up-front milestone payments associated with our license and collaboration agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or commercial milestones. Because the achievement of these milestones is not probable, and payment is not required as of March 31, 2020, such contingencies have not been recorded in our unaudited condensed consolidated financial statements. Amounts related to contingent milestone payments are not yet considered contractual obligations as they are contingent on the successful achievement of certain development, regulatory approval and sales milestones.

Recent Accounting Pronouncements

For additional information, please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* of the unaudited condensed consolidated financial statements contained in Part I, Item 1 of this Quarterly Report on Form 10-Q.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, government and government agency bonds and high-grade corporate bonds with maturities of three years or less. Our cash is deposited in and invested through highly rated financial institutions in the U.S. As of March 31, 2020, we had approximately $2,180.7 million of cash, cash equivalents and investments, comprised of $1,764.2 million of cash and cash equivalents, $406.9 million of short-term investments and $9.6 million long-term restricted cash and investments. The fair value of cash equivalents and short-term investments is subject to change as a result of potential changes in market interest rates. The potential change in fair value for interest rate sensitive instruments has been assessed on a hypothetical 10 basis point adverse movement across all maturities. As of March 31, 2020, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of approximately $0.1 million to our interest rate sensitive instruments.

Item 4. Controls and Procedures.

Evaluation of Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Quarterly Report on Form 10-Q for the period ended March 31, 2020, under the supervision and with the participation of our management, including our Chief Executive Officer and our Chief Financial Officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rules 13a-15 and 15d-15 under the Securities Exchange Act of 1934, as amended (the “Exchange Act”). The purpose of this evaluation was to determine whether as of the evaluation date our disclosure controls and procedures were effective to provide reasonable assurance that the information we are required to disclose in our filings with the SEC under the Exchange Act (i) is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms and (ii) is accumulated and communicated to our management, including our Chief Executive Officer and our Chief Financial Officer, as appropriate, to allow timely decisions regarding required disclosure. Based on that evaluation, management has concluded that as of March 31, 2020, our disclosure controls and procedures were effective.

Changes in Internal Control over Financial Reporting

During the quarterly period ended March 31, 2020, there were no changes in our internal controls over financial reporting that have materially affected or are reasonably likely to materially affect our internal control over financial reporting.

PART II — OTHER INFORMATION

Item 1. Legal Proceedings

For material legal proceedings, please read *Note 15, Commitments and Contingencies - Litigation* to our unaudited condensed consolidated financial statements included in this report.

Item 1A. Risk Factors.

Set forth below and elsewhere in this report and in other documents we file with the SEC are descriptions of risks and uncertainties that could cause actual results to differ materially from the results contemplated by the forward-looking statements contained in this report. Because of the following factors, as well as other variables affecting our operating results, past financial performance should not be considered a reliable indicator of future performance and investors should not use historical trends to anticipate results or trends in future periods. The risks and uncertainties described below are not the only ones facing us. Other events that we do not currently anticipate or that we currently deem immaterial also affect our results of operations and financial condition.

Risks Related to Our Business

We are highly dependent on the commercial success of our products in the U.S. We may not be able to meet expectations with respect to sales of our products or attain profitability and positive cash-flow from operations.

On September 19, 2016 and December 12, 2019, the FDA granted accelerated approval for EXONDYS 51 and VYONDYS 53, respectively, as therapeutic treatments for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 and exon 53 skipping, respectively. EXONDYS 51 is currently commercially available in the U.S. and Israel only, and VYONDYS 53 is currently commercially available in the U.S. only, although they are available in additional countries through our EAP. The commercial success of our products continues to depend on a number of factors, including, but not limited to:

- the effectiveness of our sales, managed markets, marketing efforts and support for our products;
- the generation and dissemination of new data analyses and the consistency of any new data with prior results, whether they support a favorable safety, efficacy and effectiveness profile of our products and any potential impact on our FDA accelerated approval status and/or FDA package insert for our products;
- the effectiveness of our ongoing commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, ongoing manufacturing efforts and hiring any additional personnel as needed to support commercial efforts;
- our ability to comply with FDA post-marketing requirements and commitments, including through successfully conducting additional studies that confirm clinical efficacy, effectiveness and safety of our products and acceptance of the same by the FDA and medical community since continued approval may be contingent upon verification of a clinical benefit in confirmatory trials;
- the occurrence of any side effects, adverse reactions or misuse, or any unfavorable publicity in these areas;
- the generation of evidence describing payers, patients and/or societal value of our products;
- whether we can consistently manufacture our products and product candidates at acceptable costs;
- the rate and consistency with which our products are prescribed by physicians, which depends on physicians' views on the safety, effectiveness and efficacy of our products;
- our ability to secure and maintain adequate reimbursement for our products, including the duration of the prior-authorization as well as the number and duration of re-authorization processes required for patients who initially obtained coverage by third parties, including by government payors, managed care organizations and private health insurers;
- our ability to obtain and maintain patent protection for our products, to preserve our trade secrets, to prevent third parties from infringing on our proprietary rights and to operate without infringing on the proprietary rights of third parties;
- the development, commercialization or pricing of competing products or therapies for the treatment of DMD, or its symptoms, and the existence of competing clinical trials;

- our ability to increase awareness of the importance of genetic testing and knowing/understanding DMD mutations, and identifying and addressing procedural barriers to obtaining therapy;
- our ability to remain compliant with laws and regulations that apply to us and our commercial activities;
- the actual market-size, ability to identify patients and the demographics of patients eligible for our products, which may be different than expected;
- the sufficiency of our drug supply to meet commercial and clinical demands and standards, which are negatively impacted by various factors, including when our projections on the potential number of amenable patients and their average weight are inaccurate; if regulatory requirements increase our drug supply needs; if our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit; failure to meet cGMP requirements; or if we encounter delays expanding the number of patients on our products and portions of our products' supply expire before sale;
- our ability to obtain regulatory approvals to commercialize our product candidates, and to commercialize our products in markets outside of the U.S.;
- the process leading to a patient's first infusion of our products may be slower for certain patients. For example, the time to first infusion may take longer if a patient chooses to put in an intravenous port, which eases access to the vein. Delays in the process prior to first infusion could negatively impact the sales of our products; and
- the exercise by Roche of its option to obtain an exclusive license to commercialize one or more of our products outside of the U.S. and Roche's subsequent commercialization efforts.

In addition, the response to COVID-19 by healthcare providers has made it difficult for some patients to receive infusions or initiate treatment with our commercial products. For this and other reasons, such as delays in processing reauthorizations and modifications to program benefits by insurers, we expect that COVID-19 will reduce our revenue from commercial product sales.

We experience significant fluctuations in sales of our products from period to period and, ultimately, we may never generate sufficient revenues from our products to reach or maintain profitability or sustain our anticipated levels of operations.

Even though EXONDYS 51 and VYONDYS 53 have received accelerated approval by the FDA, they face future post-approval development and regulatory requirements, which will present additional challenges we will need to successfully navigate.

The accelerated approvals for EXONDYS 51 and VYONDYS 53 granted by the FDA were based on an increase in the surrogate biomarker of dystrophin in skeletal muscles observed in some patients treated with EXONDYS 51 and VYONDYS 53. These products will be subject to ongoing FDA requirements governing labeling, packaging, storage, advertising, promotion and recordkeeping, and we are required to submit additional safety, efficacy and other post-marketing information to the FDA.

Under the accelerated approval pathway, continued approval may be contingent upon verification of a clinical benefit in confirmatory trials. These post-approval requirements and commitments may not be feasible and/or could impose significant burdens and costs on us; could negatively impact our development, manufacturing and supply of our products; and could negatively impact our financial results. Failure to meet post-approval commitments and requirements, including completion of enrollment and in particular, any failure to obtain positive safety and efficacy data from our ongoing and planned studies of our products, would lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51 or VYONDYS 53.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP regulations. Drug product manufacturers are required to continuously monitor and report adverse events from clinical trials and commercial use of the product. If we or a regulatory agency discover previously unknown adverse events or events of unanticipated severity or frequency, a regulatory agency may require labeling changes, implementation of risk evaluation and mitigation strategy program, or additional post-marketing studies or clinical trials. If we or a regulatory agency discover previously unknown problems with a product, such as problems with a facility where the API or drug product is manufactured or tested, a regulatory agency may impose restrictions on that product and/or the manufacturer, including removal of specific product lots from the market, withdrawal of the product from the market, or suspension of manufacturing. Sponsors of drugs approved under FDA accelerated approval provisions also are required to submit to the FDA, at least 30 days before initial use, all promotional materials intended for use after the first 120 days following marketing approval. If we or the manufacturing facilities for our products fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or require us to initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

We are subject to uncertainty relating to reimbursement policies which, if not favorable, could hinder or prevent the commercial success of our products and/or product candidates.

Our ability to successfully maintain and/or increase sales of our products in the U.S. depends in part on the coverage and reimbursement levels set by governmental authorities, private health insurers and other third-party payors. Third party payors are increasingly challenging the effectiveness of and prices charged for medical products and services. We may not be able to obtain or maintain adequate third-party coverage or reimbursement for our products, and/or we may be required to provide discounts or rebates on our products in order to obtain or maintain adequate coverage.

We expect that private insurers will continue to consider the efficacy, effectiveness, cost-effectiveness and safety of our products, including any new data and analyses that we are able to collect and make available in a compliant manner, in determining whether to approve reimbursement for our products and at what levels. If there are considerable delays in the generation of new evidence or if any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of our products. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of our products from additional insurers on a satisfactory rate or basis, in which case our business would be materially adversely affected. In addition, obtaining these approvals can be a time consuming and expensive process. Our business would be materially adversely affected if we are not able to maintain favorable coverage decisions and/or fail to receive additional favorable coverage decisions from third party insurers, in particular during re-authorization processes for patients that have already initiated therapy. Our business could also be adversely affected if government health programs, private health insurers, including managed care organizations, or other reimbursement bodies or payors limit the indications for which our products will be reimbursed or fail to recognize accelerated approval and surrogate endpoints as clinically meaningful.

In addition, the impact of the ongoing COVID-19 pandemic can result in delays in processing reauthorizations and modifications to program benefits by insurers, making it difficult for patients to obtain or maintain favorable coverage decisions for our products. Furthermore, we cannot predict to what extent the COVID-19 pandemic, depending on its scale and duration, may disrupt global healthcare systems and access to our products or result in a widespread loss of individual health insurance coverage due to unemployment, a shift from commercial payor coverage to government payor coverage, or an increase in demand for patient assistance and/or free drug programs, any of which would adversely affect access to our products and our net sales.

In some foreign countries, particularly Canada and the countries of Europe, Latin America and Asia Pacific, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take 12 to 24 months or longer after the receipt of regulatory approval and product launch. In order to obtain favorable

reimbursement for the indications sought or pricing approval in some countries, we may be required to collect additional data, including conducting additional studies. Furthermore, several European countries have implemented government measures to either freeze or reduce pricing of pharmaceutical products. If reimbursement for our products is unavailable in any country in which reimbursement is sought, limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed. In addition, many foreign countries are referencing to other countries' official public list price, hence an unsatisfactory price level in one country could consequently impinge negatively upon overall revenue.

We expect to experience pricing pressures in connection with the sale of our current and future products due to a number of factors, including current and future healthcare reforms and initiatives by government health programs and private insurers (including managed care plans) to reduce healthcare costs, the scrutiny of pharmaceutical pricing, the ongoing debates on reducing government spending and additional legislative proposals. These healthcare reform efforts or any future legislation or regulatory actions aimed at controlling and reducing healthcare costs, including through measures designed to limit reimbursement, restrict access or impose unfavorable pricing modifications on pharmaceutical products, could impact our and our partners' ability to obtain or maintain reimbursement for our products at satisfactory levels, or at all, which could materially harm our business and financial results.

Additionally, our gene therapy product candidates represent novel approaches to treatment that will call for new levels of innovation in both pricing, reimbursement, payment and drug access strategies. Current reimbursement models may not accommodate the unique factors of our gene therapy product candidates, including high up-front costs, lack of long-term efficacy and safety data and fees associated with complex administration, dosing and patient monitoring requirements. Hence, it may be necessary to restructure approaches to payment, pricing strategies and traditional payment models to support these therapies.

The downward pressure on healthcare costs in general has become intense. As a result, increasingly high barriers are being erected to the entry of new products. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our products and product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our products and product candidates (e.g., for administration of our products to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

Healthcare reform and other governmental and private payor initiatives may have an adverse effect upon, and could prevent commercial success of our products and product candidates.

The U.S. government and individual states have aggressively pursued healthcare reform, as evidenced by the passing of the Healthcare Reform Act and the ongoing efforts to modify or repeal that legislation. The Healthcare Reform Act substantially changed the way healthcare is financed by both governmental and private insurers and contains a number of provisions that affect coverage and reimbursement of drug products and/or that could potentially reduce the demand for pharmaceutical products such as increasing drug rebates under state Medicaid programs for brand name prescription drugs and extending those rebates to Medicaid managed care and assessing a fee on manufacturers and importers of brand name prescription drugs reimbursed under certain government programs, including Medicare and Medicaid. Other aspects of healthcare reform, such as expanded government enforcement authority and heightened standards that could increase compliance-related costs, could also affect our business. Modifications have been implemented under the Trump Administration and additional modifications or repeal may occur. There are, and may continue to be, judicial challenges. We cannot predict the ultimate content, timing or effect of any changes to the Healthcare Reform Act or other federal and state reform efforts. There is no assurance that federal or state health care reform will not adversely affect our future business and financial results, and we cannot predict how future federal or state legislative, judicial or administrative changes relating to healthcare reform will affect our business.

The U.S. government, state legislatures and foreign governments have shown significant interest in implementing cost-containment programs to limit the growth of government-paid healthcare costs, including price controls, waiver from Medicaid drug rebate law requirements, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs and the introduction of international reference pricing in the U.S. We anticipate that the U.S. Congress, state legislatures and the private sector will continue to consider and may adopt healthcare policies intended to curb rising healthcare costs. These cost containment measures may include implementation or modification of:

- controls on government funded reimbursement for drugs;
- caps or mandatory discounts under certain government sponsored programs;
- controls on healthcare providers;
- challenges to the pricing of drugs or limits or prohibitions on reimbursement for specific products through other means;
- reform of drug importation laws;
- delegation of decision making to state Medicaid agencies and waiver of reimbursement requirements;
- expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person; and
- prohibition on direct-to-consumer advertising or drug marketing practices.

We are unable to predict what additional legislation, regulations or policies, if any, relating to the healthcare industry or third party coverage and reimbursement may be enacted in the future or what effect such legislation, regulations or policies would have on our business. Any cost containment measures, including those listed above, or other healthcare system reforms that are adopted, could significantly decrease the available coverage and the price we might establish for our products and product candidates, which would have an adverse effect on our net revenues and operating results.

Our products may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.

The commercial success of our products, particularly in the near term in the U.S., depends upon the level of market adoption by patients, payors and healthcare providers. If our products do not achieve an adequate level of market adoption for any reason, or if market adoption does not persist, our potential profitability and our future business prospects will be severely adversely impacted. The degree of market acceptance of our products depends on a number of factors, including:

- our ability to demonstrate to the medical and payor community, including specialists who may purchase or prescribe our products, the clinical efficacy, effectiveness and safety of our products as the prescription products of choice for their respective indications;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for our products in a timely manner from government and private payors;
- the ability to timely demonstrate to the satisfaction of payors real world effectiveness and the economic, humanistic and societal benefits of our products;
- the actual and perceived efficacy and safety profile of our products, particularly if unanticipated adverse events related to our products' treatment arise and create safety concerns among potential patients or prescribers or if new data and analyses we obtain for our products do not support, or are interpreted by some parties to not support, the efficacy of our products; and
- the efficacy and safety of our other exon-skipping product candidates, including our exon 45 product candidate (casimersen), and third parties' competitive therapies.

We may not be able to expand the global footprint of our products outside of the U.S.

Even though EXONDYS 51 was approved for marketing in the U.S. and in Israel, and VYONDYS 53 was approved for marketing in the U.S., we may not receive approval to commercialize these products in additional countries. In November 2016, we submitted a MAA for eteplirsen to the EMA and the application was validated in December 2016. As we announced on June 1, 2018, the CHMP of the EMA adopted a negative opinion for eteplirsen. In September 2018, the CHMP of the EMA confirmed its negative opinion for eteplirsen, and the European Commission adopted the CHMP opinion in December 2018. During 2019, we sought follow-up EMA scientific advice for eteplirsen. Once data from our ongoing studies is available, we plan to evaluate future engagement with the EMA on potential next steps.

In order to market any product in a country outside of the U.S., we must comply with numerous and varying regulatory requirements for approval in those countries regarding demonstration of evidence of the product's safety and efficacy and governing, among other things, labeling, distribution, advertising, and promotion, as well as pricing and reimbursement of the product. Obtaining marketing approval in a country outside of the U.S. is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject an application or delay, limit or deny approval of any of our products for many reasons, including:

- we may not be able to demonstrate to the satisfaction of regulatory authorities outside the U.S. the risk benefit of our products;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by regulatory authorities outside the U.S.;
- regulatory authorities outside the U.S. may disagree with the adequacy (number, design, size, controls, conduct or implementation) of our clinical trials prior to granting approval, and we may not be able to generate the required data on a timely basis, or at all;
- regulatory authorities outside the U.S. may conclude that data we submit to them fail to demonstrate an appropriate level of safety or efficacy of our products, or that our products' respective clinical benefits outweigh their safety risks;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites or require us to generate additional data or information;

- regulatory authorities outside the U.S. may impose limitations or restrictions on the approved labeling of our products, thus limiting intended users or providing an additional hurdle for market acceptance of the product;
- regulatory authorities outside the U.S. may identify deficiencies in the manufacturing processes, or may require us to change our manufacturing process or specifications; and
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

Approval procedures vary among countries and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ significantly from that required to obtain approval in the U.S. In particular, in many foreign countries, it is required that a product receives pricing and reimbursement approval before the product can be distributed commercially. Many foreign countries undertake cost-containment measures that could affect pricing or reimbursement of our products. This can result in substantial delays, and the price that is ultimately approved in some countries may be lower than the price for which we expect to offer our products.

Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the approval process in others. Failure to obtain marketing approval in other countries or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for our products and could adversely affect our business and financial condition. In addition, failure to obtain approval in one country or area may affect sales under the EAP in other countries or areas. Even if we are successful in obtaining regulatory approval of our products in additional countries, our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors.

In addition, we have granted Roche an exclusive option to obtain an exclusive license to commercialize certain products, including eteplirsen and golodirsen, outside of the U.S. If this option is exercised, Roche will have sole control over and decision-making authority with respect to the commercialization of such products outside the U.S.

We cannot predict whether historical revenues from eteplirsen through our EAP outside the U.S. will continue or whether we will be able to continue to distribute eteplirsen through our EAP.

We have initiated an EAP for eteplirsen in select countries in Europe, North America, South America and Asia where it currently has not been approved. We are also in the process of initiating an EAP for golodirsen outside of the U.S. While we generate revenue from the distribution of eteplirsen through our EAP, we cannot predict whether historical revenues from this program will continue, whether we will be able to continue to distribute our products through our EAP, or whether commercial revenues will exceed revenues historically generated from sales through our EAP. Reimbursement through national EAPs may cease to be available if authorization for an EAP expires or is terminated. For example, healthcare providers in EAP jurisdictions may not be convinced that their patients benefit from our products or may prefer to wait until such time as our products are approved by a regulatory authority in their country before prescribing any of our products. Even if a healthcare provider is interested in obtaining access to our products for its patient through the EAP, the patient will not be able to obtain access to our products if payment for the drug is not secured.

Any failure to maintain revenues from sales of eteplirsen through our EAP and/or to generate revenues from commercial sales of eteplirsen exceeding historical sales through our EAP could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

If we are unable to successfully maintain and further develop internal commercialization capabilities, sales of our products may be negatively impacted.

We have hired and trained a commercial team and put in the organizational infrastructure we believe we need to support the commercial success of our products in the U.S. Factors that may inhibit our efforts to maintain and further develop commercial capabilities include:

- an inability to retain an adequate number of effective commercial personnel;
- an inability to train sales personnel, who may have limited experience with our company or our products, to deliver a consistent message regarding our products and be effective in educating physicians on how to prescribe our products;
- an inability to equip sales personnel with compliant and effective materials, including medical and sales literature to help them educate physicians and our healthcare providers regarding our products and their proper administration and educate payors on the safety, efficacy and effectiveness profile of our products to support favorable coverage decisions;
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization; and

- restrictions on the ability of our employees to perform their jobs due to the COVID-19 pandemic, such as quarantines and self-isolations.

If we are not successful in maintaining an effective commercial, sales and marketing infrastructure, we will encounter difficulty in achieving, maintaining or increasing projected sales of our products in the U.S., which would adversely affect our business and financial condition.

If we are unable to execute effectively our sales and marketing activities outside the U.S., we may be unable to generate sufficient product revenue.

EXONDYS 51 and VYONDYS 53 are our first and second commercial products, respectively. As a result, our sales, marketing, managerial and other non-technical capabilities are relatively new in the U.S. We have built a commercial sales force in Europe and we are currently in the process of building commercial infrastructure in other key countries in order to be ready to launch our products with a relatively small specialty sales force in the event our products are ultimately approved in those jurisdictions. The establishment and development of our commercial infrastructure will continue to be expensive and time consuming, and we may not be able to successfully develop this capability in a timely manner or at all. We anticipate building sales, medical, marketing, managerial, distribution and other capabilities across multiple jurisdictions to prepare for potential approvals ex-U.S. Doing so will require a high degree of coordination and compliance with laws and regulations in such jurisdictions. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize our products in such jurisdictions will be adversely affected. Even if we are able to effectively hire a sales force and develop marketing and sales capabilities, our sales force may not be successful in commercializing our products or any product candidate that we develop. If we are unable to establish adequate manufacturing, sales, marketing, supply and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable outside of the U.S. Furthermore, we have granted Roche an exclusive option to obtain an exclusive license to commercialize certain products, including eteplirsen and golodirsen, outside of the U.S. If this option is exercised, Roche will have sole control over and decision-making authority with respect to the commercialization of such products outside the U.S.

If we fail to obtain or maintain regulatory exclusivity for our products, then we may not be able to protect our products from competition and our business may be adversely impacted. If a competitor obtains an authorization to market the same or substantially same product before a product of ours is authorized in a given country and is granted regulatory exclusivity, then our product may not be authorized for sale as a result of the competitor's regulatory exclusivity and as a result, our investment in the development of that product may not be returned.

In addition to any patent protection, we rely on various forms of regulatory exclusivity to protect our products. During the development of our products, we anticipate regulatory exclusivities available upon approval of our products. Implementation and enforcement of regulatory exclusivity, which may consist of regulatory data protection and market protection, varies widely from country to country. Failure to qualify for regulatory exclusivity, or failure to obtain or maintain the extent or duration of such protections that we expect in each of the markets for our products due to challenges, changes or interpretations in the law or otherwise, could affect our revenues for our products or our decision on whether to market our products in a particular country or countries or could otherwise have an adverse impact on our results of operations. We are not guaranteed to receive or maintain regulatory exclusivity for our current or future products, and if our products that are granted orphan status were to lose their status as orphan drugs or the data or marketing exclusivity provided for orphan drugs, our business and operations could be adversely affected.

Due to the nature of our products and product candidate pipeline, in addition to new chemical entity exclusivity and new biologic exclusivity, orphan drug exclusivity is especially important for our products that are eligible for orphan drug designation. For eligible products, we plan to rely on orphan drug exclusivity to maintain a competitive position. If we do not have adequate patent protection for our products, then the relative importance of obtaining regulatory exclusivity is even greater. While orphan status for any of our products, if granted or maintained, would provide market exclusivity for the time periods specified above upon approval, we would not be able to exclude other companies from obtaining regulatory approval of products using the same or similar active ingredient for the same indication during or beyond the exclusivity period applicable to our product on the basis of orphan drug status (e.g., seven years in the U.S.). Orphan drug designation neither shortens the development time or regulatory review time of a drug, nor gives the drug any advantage in the regulatory review or approval process.

In addition, we may face risks with maintaining regulatory exclusivities for our products, and our protection may be circumvented, even if maintained. For instance, orphan drug exclusivity in the U.S. may be rescinded if (i) an alternative, competing product demonstrates clinical superiority to our product with orphan exclusivity; or (ii) we are unable to assure the availability of sufficient quantities of our orphan products to meet the needs of patients. Moreover, competitors may receive approval of different drugs or biologics for indications for which our prior approved orphan products have exclusivity. Orphan drug exclusivity in Europe may be modified for several reasons, including a significant change to the orphan medicinal product designations or status criteria after-market authorization of the orphan product (*e.g.*, product profitability exceeds the criteria for orphan drug designation), problems with the production or supply of the orphan drug, or a competitor drug, although similar, is safer, more effective or otherwise clinically superior than the initial orphan drug. Thus, we cannot guarantee that another company will not receive approval to market a product candidate that is granted orphan drug exclusivity for the same drug or similar drug and same orphan indication as any of our product candidates for which we plan to file an NDA, BLA or MAA. If that were to happen, our prior approved orphan products may face competition and any pending NDA, BLA or MAA for our product candidate for that indication may not be approved until the

competing company's period of exclusivity has expired in the U.S. or the EU, as applicable. For example, in January 2020, the FDA issued a draft guidance to clarify its position on when gene therapy products would be considered the "same" or "different" for purposes of orphan drug exclusivity. The draft guidance notes that if the gene therapy products differ in either the gene transferred by the products ("transgene") or the vector used to deliver the transgene, then the two gene therapy products are different and could both be approved for same indication. If the transgene and the vector are the same, then the products are likely the "same," such that the first product approved would gain regulatory exclusivity over the second product. If there are other, lesser differences in the products, FDA would make a case-by-case determination as how to apply orphan exclusivity to the competing product. As illustrated by this draft guidance, orphan drug exclusivity as applied to gene therapy products is an evolving area subject to change and interpretation by the FDA and therefore we cannot be certain as to how the FDA will apply those rules to our products.

Even though we have obtained orphan drug designation for certain of our product candidates and even if we obtain orphan drug designation for these or our future product candidates, due to the uncertainties associated with developing biopharmaceutical products, we may not be the first to obtain marketing approval for any particular orphan indication, which means that we may not obtain orphan drug exclusivity and could also potentially be blocked from approval of certain product candidates until the competitor's orphan drug exclusivity period on its product expires (e.g., seven years in the U.S.). Moreover, with respect to antisense oligonucleotides and gene therapies, it is uncertain how similarity between product candidates designed to treat the same rare disease or condition may be determined on a country-by-country basis and whether the orphan drug exclusivity of a previously approved product can block the approval of a chemically distinct product candidate under regulatory review.

The patient population suffering from DMD, LGMDs, Pompe disease, CMT 1A and MPS IIIA is small and has not been established with precision. If the actual number of patients is smaller than we estimate, our revenue and ability to achieve profitability may be adversely affected.

DMD, LGMD, Pompe disease, CMT 1A and MPS IIIA are rare, fatal genetic disorders. DMD affects an estimated one in approximately every 3,500 to 5,000 males born worldwide, of which up to 13% are estimated to be amenable to exon 51 skipping and up to 8% are estimated to be amenable to exon 53. LGMDs as a class affect an estimated range of approximately one in every 14,500 to one in every 123,000 individuals. Pompe disease affects an estimated one in approximately every 40,000 individuals. CMT is a group of peripheral nerve disorders affecting approximately one in every 2,500 individuals. CMT type 1A affects approximately 50,000 patients in the U.S. MPS IIIA affects approximately 1 in 100,000 newborns. Our estimates of the size of these patient populations are based on limited number of published studies as well as internal analyses. Various factors may decrease the market size of our products and product candidates, including the severity of the disease, patient demographics and the response of patients' immune systems to our products and product candidates. If the results of these studies or our analysis of them do not accurately reflect the relevant patient population, our assessment of the market may be inaccurate, making it difficult or impossible for us to meet our revenue goals, or to obtain and maintain profitability.

We face intense competition and rapid technological change, which may result in other companies discovering, developing or commercializing competitive products.

The biotechnology and pharmaceutical industries are highly competitive and subject to significant and rapid technological change. We are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development in areas in which our products and product candidates are aimed. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with our products or product candidates. For example, we face competition in the field of DMD by third parties who are developing or who had once developed: (i) exon skipping product candidates, such as Wave Life Sciences (notably for exons 51 and 53), Nippon Shinyaku (notably for exon 53), Daiichi Sankyo (notably for exon 45) and Audentes Therapeutics, Inc. (acquired by Astellas Pharma) (notably for exons 2, 51 and 53); (ii) gene therapies that express microdystrophin or mini-dystrophin, such as Pfizer and Solid Biosciences; (iii) CRISPR/Cas 9 approaches, such as Exonics Therapeutics (acquired by Vertex Pharmaceuticals), CRISPR Therapeutics and Editas Medicine; (iv) other disease modifying approaches, such as PTC Therapeutics, which has a small molecule candidate, ataluren, that targets nonsense mutations; and (v) other approaches that may be palliative in nature or potentially complementary with our products and product candidates and that are being developed by Santhera, Catabasis, Fibrogen, ReveraGen, Capricor, BioPhytis, Mallinckrodt, Astellas Pharma, and Tivorsan. Although BioMarin announced on May 31, 2016 its intent to discontinue clinical and regulatory development of drisapersen as well as its other clinical stage candidates, BMN 044, BMN 045 and BMN 053, then-currently in Phase 2 studies for distinct forms of DMD, it further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD. In addition, while Wave announced its intention to discontinue development of suvodirsen and suspend development of WVE-N531, continued development of one or both of these candidates is possible.

In addition, we are aware of many pharmaceutical and biotechnology companies that are actively engaged in research and development using platform technologies that may be viewed as competing with ours beyond and including those companies mentioned immediately above, such as Alnylam Pharmaceuticals, Inc., Tekmira Pharmaceuticals Corp., Deciphera Pharmaceuticals, Ionis Pharmaceuticals, Inc., Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Shire plc (now Takeda), Biogen, Moderna Therapeutics, Avidity, Dyne Therapeutics, Stoke Therapeutics and Sanofi. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene therapy and

gene editing (CRIPSR and AAV, among others) and small molecule therapies that are potential competitors for therapies being developed in the muscular dystrophy, neuromuscular and rare disease space, including, but not limited to, Astellas Pharma, Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire (now Takeda), Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Akashi, Catabasis, Capricor Therapeutics, Oxford University, Exonics Therapeutics (acquired by Vertex Pharmaceuticals), and Editas Medicine.

If any of our competitors are successful in obtaining regulatory approval for any of their product candidates, it may limit our ability to enter into the market, gain market share or maintain market share in the DMD space or other diseases targeted by our platform technologies, products and product candidate pipeline.

It is possible that our competitors will succeed in developing technologies that limit the market size for our products or product candidates, impact the regulatory approval and post-marketing process for our products and product candidates, are more effective than our products or product candidates or would render our technologies obsolete or noncompetitive. Our competitors may, among other things:

- develop safer or more effective products;
- implement more effective approaches to sales and marketing;
- develop less costly products;
- obtain preferred formulary status;
- obtain regulatory approval more quickly;
- have access to more manufacturing capacity;
- develop products that are more convenient and easier to administer;
- form more advantageous strategic alliances; or
- establish superior intellectual property positions.

We have entered into multiple collaborations, including our collaboration with Roche, and may seek or engage in future collaborations, strategic alliances, acquisitions or licensing agreements that complement or expand our business. We may not be able to complete such transactions, and such transactions, if executed, may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

In order to achieve our long-term business objectives, we actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring products, technologies or businesses. We may face competition from other companies in pursuing acquisitions and similar transactions in the biotechnology industry. This competition is most intense for approved drugs and late-stage drug candidates, which have the lowest risk and would have the most immediate effect on our financial performance. Our ability to complete transactions may also be limited by applicable antitrust and trade regulation laws and regulations in the U.S. and foreign jurisdictions in which we or the operations or assets we seek to acquire carry on business.

We have entered into multiple collaborations, including with Roche, Nationwide, Lysogene, Lacerta, Duke University, Genethon, StrideBio and University of Florida. We may not realize the anticipated benefits of such collaborations, and the anticipated benefits of any future collaborations or acquisitions, each of which involves numerous risks, including:

- collaborators have significant discretion in determining the efforts and resources that they will apply to a collaboration;
- collaborators may not pursue development and commercialization of our products or product candidates based on clinical trial results, changes in their strategic focus due to the acquisition of competitive products, availability of funding, or other external factors, such as a business combination that diverts resources or creates competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial, stop a clinical trial, abandon a product candidate, repeat or conduct new clinical trials, or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our products or product candidates;
- collaborators may not properly maintain or defend our intellectual property rights or may use our intellectual property or proprietary information in a way that gives rise to actual or threatened litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential liability;

- disputes may arise between us and a collaborator that cause the delay or termination of the research, development or commercialization of our products or product candidates, or that result in costly litigation or arbitration that diverts management attention and resources;
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product or product candidate;
- failure to successfully develop the acquired or licensed drugs or technology or to achieve strategic objectives, including successfully developing and commercializing the drugs, drug candidates or technologies that we acquire or license;
- entry into markets in which we have no or limited direct prior experience or where competitors in such markets have stronger market positions;
- disruption of our ongoing business, distraction of our management and employees from other opportunities and challenges and retention of key employees;
- potential failure of the due diligence processes to identify significant problems, liabilities or other shortcomings or challenges of an acquired company, or acquired or licensed product or technology, including but not limited to, problems, liabilities or other shortcomings or challenges with respect to intellectual property, product quality, safety, accounting practices, employee, customer or third-party relations and other known and unknown liabilities;
- liability for activities of the acquired company or licensor before the acquisition or license, including intellectual property infringement claims, violations of laws, commercial disputes, tax liabilities, and other known and unknown liabilities;
- exposure to litigation or other claims in connection with, or inheritance of claims or litigation risk as a result of an acquisition or license, including but not limited to, claims from terminated employees, customers, former equity holders or other third-parties;
- difficulty in integrating the products, product candidates, technologies, business operations and personnel of an acquired asset or company; and
- difficulties in the integration of the acquired company's departments, systems, including accounting, human resource and other administrative systems, technologies, books and records, and procedures, as well as in maintaining uniform standards, controls, including internal control over financial reporting required by the Sarbanes-Oxley Act of 2002 and related procedures and policies.

For example, we will have limited influence and control over the development and commercialization activities of Roche in the territories in which it leads development and commercialization of SRP-9001, and if the exclusive option is exercised, in the territories in which it leads commercialization of certain other products or product candidates. Roche's development and commercialization activities in the territories where it is the lead party may adversely impact our own efforts in the U.S. Failure by Roche to meet its obligations under the collaboration agreement, to apply sufficient efforts at developing and commercializing collaboration products, or to comply with applicable legal or regulatory requirements, may materially adversely affect our business and our results of operations. In addition, to the extent we rely on Roche to commercialize any products for which we obtain regulatory approval, we may receive less revenues than if we commercialized these products ourselves, which could materially harm our prospects.

Even if we achieve the long-term benefits associated with strategic transactions, our expenses and short-term costs may increase materially and adversely affect our liquidity and short-term net income (loss). Future licenses or acquisitions could result in potentially dilutive issuances of equity securities, the incurrence of debt, the creation of contingent liabilities, impairment expenses related to goodwill, and impairment or amortization expenses related to other intangible assets, which could harm our financial condition. For example, in February 2020, we issued and sold 2,522,227 shares of common stock to Roche Finance in connection with the entry into the collaboration agreement with Roche.

Risks Related to the Development of our Product Candidates

We may find it difficult to enroll patients in our clinical trials, which could delay or prevent clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our clinical trials depends on the speed at which we can recruit eligible patients to participate in testing our product candidates. We have experienced delays in some of our clinical trials, and we may experience similar delays in the future. These delays could result in increased costs, delays in advancing our product development, delays in testing the effectiveness of our technology or termination of the 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 achieve diversity in a study, to complete our clinical trials in a timely manner. Patient enrollment is affected by factors including:

- design of the study protocol;
- size of the patient population;
- eligibility criteria for the study in question;
- manufacturing of product candidates;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical trial sites for prospective patients;
- availability of competing therapies and clinical trials;
- efforts to facilitate timely enrollment in clinical trials;
- patient referral practices of physicians;
- activities of patient advocacy groups;
- ability to monitor patients adequately during and after treatment; and
- severity of the disease under investigation.

In particular, each of the conditions for which we plan to evaluate our product candidates are rare genetic diseases with limited patient pools from which to draw for clinical trials. Further, because newborn screening for these diseases is not widely adopted, and it can be difficult to diagnose these diseases in the absence of a genetic screen, we may have difficulty finding patients who are eligible to participate in our studies. The eligibility criteria of our clinical trials will further limit the pool of available study participants. Additionally, the process of finding and diagnosing patients may prove costly. The treating physicians in our clinical trials may also use their medical discretion in advising patients enrolled in our clinical trials to withdraw from our studies to try alternative therapies. In addition, the COVID-19 pandemic may impact patient ability and willingness to travel to clinical trial sites as a result of quarantines and other restrictions, which may negatively impact enrollment in our clinical trials.

We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA or the EMA or other regulatory agencies. 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:

- difficulty in establishing or managing relationships with contract research organizations (“CROs”) and physicians;
- different standards for the conduct of clinical trials;
- our inability to locate 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 products and treatment.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate ongoing or planned clinical trials, any of which would have an adverse effect on our business.

Failures or delays in the commencement or completion of ongoing and planned clinical trials of our product candidates negatively impact commercialization efforts; result in increased costs; and delay, prevent or limit our ability to gain regulatory approval of product candidates and to generate revenues and continue our business.

Successful completion of clinical trials at each applicable stage of development is a prerequisite to submitting a marketing application to the regulatory agencies and, consequently, the ultimate approval and commercial marketing of any of our product candidates for the indications in which we develop them. We do not know whether any of our clinical trials will begin or be completed, and results announced, as planned or expected, if at all, as the commencement and completion of clinical trials and announcement of results is often delayed or prevented for a number of reasons, including, among others:

- denial by the regulatory agencies of permission to proceed with our planned clinical trials or any other clinical trials we may initiate, or placement of a clinical trial on hold;
- delays in filing or receiving approvals of additional INDs that may be required;
- negative results from our ongoing non-clinical trials or clinical trials;
- challenges in identifying, recruiting, enrolling and retaining patients to participate in clinical trials
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the CROs involved in the clinical trial;
- negotiate contracts and other related documents with clinical trial parties and institutional review boards, such as informed consents, CRO agreements and site agreements, which can be subject to extensive negotiations that could cause significant delays in the clinical trial process, with terms possibly varying significantly among different trial sites and CROs and possibly subjecting the Company to various risks;
- inadequate quantity or quality of supplies of a product candidate or other materials necessary to conduct clinical trials, for example as a result of delays in defining and implementing the manufacturing process for materials used in pivotal trials or for the manufacture of larger quantities or other delays or issues arising in the manufacturing of sufficient supply of finished drug product;
- difficulties obtaining institutional review board (“IRB”) approval, and equivalent approval for sites outside the U.S., to conduct a clinical trial at a prospective site or sites;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- delays or problems in analyzing data, or the need for additional analysis or data or the need to enroll additional patients;
- the occurrence of serious adverse events or unexpected drug-related side effects experienced by patients in a clinical trial or unexpected results in ongoing non-clinical trials;
- delays in validating endpoints utilized in a clinical trial;
- our inability to satisfy the requirements of the regulatory agencies to commence clinical trials, including CMC requirements, or other regulatory requirements prior to the initiation of a clinical trial;
- the regulatory agencies disagreeing with our clinical trial design and our interpretation of data from clinical trials, or changing the requirements for approval even after the regulatory authority has reviewed and commented on the design for our clinical trials;
- reports from non-clinical or clinical testing of competing therapies that raise safety or efficacy concerns; and
- the recruitment and retention of employees, consultants or contractors with the required level of expertise.

In addition, the impact of COVID-19 has caused, and may continue to cause, delays and disruptions in some of our clinical trials. The response to COVID-19 by healthcare providers can delay site initiation, slow down enrollment, and make the ongoing collection of data for patients enrolled in studies more difficult or intermittent. In addition, the pandemic may impact patient ability and willingness to travel to clinical trial sites as a result of quarantines and other restrictions, which may negatively impact the execution of clinical trials.

Any inability to complete successfully pre-clinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, manufacturing or formulation changes to our product candidates often require additional studies to demonstrate comparability of the modified product candidates to earlier versions. Clinical study delays also 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, which impairs our ability to successfully commercialize our product candidates and harms our business and results of operations.

Results from pre-clinical and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials, and pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive pre-clinical and clinical trials, that the product candidate is safe and effective in humans. Ongoing and future pre-clinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. For example, although we believe the pre-clinical data for PPMO SRP-5051 collected to date is positive, the additional data we collect, including in the clinic, may not be consistent with the pre-clinical data or show a safe benefit that warrants further development or pursuit of a regulatory approval for PPMO product candidates.

Furthermore, success in pre-clinical and early clinical trials does not ensure that the subsequent trials will be successful, nor does it predict final results of a confirmatory trial. Some of our clinical trials were conducted with small patient populations and were not blinded or placebo-controlled, making it difficult to predict whether the favorable results that we observed in such trials will be repeated in larger and more advanced clinical trials. For example, on October 3, 2018, Nationwide presented positive results from a Phase 1/2a micro-dystrophin gene therapy clinical trial in four individuals with DMD enrolled in the trial and, on March 25, 2019, we presented nine-month functional and CK data from baseline from these four individuals, and twelve-month CK data from baseline from one of these individuals. In addition, on February 27, 2019, we announced positive expression and biomarker data from the first three-patient cohort dosed in the SRP-9003 gene therapy trial to treat LGMD type 2E, or beta-sarcoglycanopathy and, on October 4, 2019, we announced positive nine-month functional data from these three patients. The data is based on small patient samples and therefore may not be predictive of future results. In addition, we cannot assure that the results of additional data or data from any future trial will yield results that are consistent with the data presented, that we will be able to demonstrate the safety and efficacy of these product candidates, that later trial results will support further development, or even if such later results are favorable, that we will be able to successfully complete the development of, obtain accelerated, conditional or standard regulatory approval for, or successfully commercialize any of such product candidates. Similarly, we cannot provide assurances that data from our ongoing and planned studies with respect to our commercially approved products and product candidates will be positive and consistent or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our products or product candidates will be consistent with our interpretations.

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

Our product candidates may cause undesirable side effects. In addition to side effects caused by our product candidates, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our trials, we may decide, or the FDA, the EMA or other regulatory authorities could order us, to halt, delay or amend pre-clinical development or clinical development of our product candidates or we may be unable to receive regulatory approval of our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Our gene therapy product candidates may be perceived as unsafe or may result in unforeseen adverse events. Failure of other gene therapy programs, negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our gene therapy product candidates and harm our ability to conduct our business or obtain regulatory approvals for our gene therapy product candidates.

Gene therapy remains a newly applied technology, with only a few gene therapy products approved to date in the U.S., the EU or elsewhere. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy 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 genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available.

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

established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

More restrictive government regulations or negative public opinion would harm our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our gene therapy product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including death. Lack of efficacy and/or serious adverse events related to clinical trials we, our strategic partners or other companies conduct, even if such adverse events are not ultimately attributable to the relevant product candidates or products, and/or failed commercialization of gene therapy products may result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

If there are significant delays in obtaining or we are unable to obtain or maintain required regulatory approvals, we will not be able to commercialize our product candidates in a timely manner or at all, which could impair our ability to generate sufficient revenue and have a successful business.

The research, testing, manufacturing, labeling, approval, commercialization, marketing, selling and distribution of drug products are subject to extensive regulation by applicable local, regional and national regulatory authorities and regulations may differ from jurisdiction to jurisdiction. In the U.S., approvals and oversight from federal (e.g., FDA), state and other regulatory authorities are required for these activities. Sale and marketing of our product candidates in the U.S. or other countries is not permitted until we obtain the required approvals from the applicable regulatory authorities. Of the large number of drugs in development in the biopharmaceutical industry, only a small percentage result in the submission of a marketing application to the FDA or an MAA to the EMA and even fewer are approved for commercialization.

Our ability to obtain the government or regulatory approvals required to commercialize any of our product candidates in any jurisdiction, including in the U.S. or the EU, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our non-clinical, clinical, chemistry, manufacturing and controls and other data and analyses from past, current and future studies for any of our product candidates may not be sufficient to meet regulatory requirements for marketing application approvals. The regulatory authorities could disagree with our interpretations and conclusions regarding data we provide in connection with NDA, BLA or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any submission we make or identify additional requirements for product approval to be submitted upon completion, if ever. In addition, in the U.S., an FDA advisory committee could determine that our data are insufficient to provide a positive recommendation for approval of any NDA or BLA we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA or BLA submission, the FDA could still disagree with the advisory committee's recommendation and deny approval of a product candidate based on their review.
- The regulatory approval process for product candidates targeting orphan diseases, such as DMD, that use new technologies and processes, such as antisense oligonucleotide therapies, gene therapy and other alternative approaches or endpoints for the determination of efficacy is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, small safety databases, varying levels of applicable expertise of regulators or their advisory committees, scientific developments, changes in the competitor landscape, shifting political priorities and changes in applicable laws, rules or regulations and interpretations of the same. As a result of uncertainty in the approval process for products intended to treat serious rare diseases, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned NDAs, BLAs and MAAs for our product candidates, in a timely manner, or at all. Examples of such requests or requirements could include, but are not limited to, conducting additional or redesigned trials and procedures (e.g., additional safety data, patient muscle biopsies, dystrophin analyses and the use of assays), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, in the U.S., an FDA advisory committee or regulators may disagree with our data analysis, interpretations and conclusions at any point in the approval process, which could negatively impact the approval of our NDA or BLA or result in a decision by the Company not to proceed with an NDA or BLA submission for a product candidate based on feedback from regulators.
- We may not have the resources required to meet regulatory requirements and successfully navigate what is generally a lengthy, expensive and extensive approval process for commercialization of drug product candidates.

Any failure on our part to respond to these requirements in a timely and satisfactory manner could significantly delay or negatively impact confirmatory study timelines and/or the development plans we have for PMO, PPMO, gene therapy-based product candidates or other product candidates. Responding to requests from regulators and meeting requirements for clinical trials, submissions and approvals may require substantial personnel, financial or other resources, which, as a small biopharmaceutical company, we may not be able to obtain in a timely manner or at all. In addition, our ability to respond to requests from regulatory

authorities that involve our agents, third party vendors and associates may be complicated by our own limitations and those of the parties we work with. It may be difficult or impossible for us to conform to regulatory guidance or successfully execute our product development plans in response to regulatory guidance, including guidance related to clinical trial design with respect to any NDA, BLA or MAA submissions.

Even if our product candidates demonstrate safety and efficacy in clinical studies, the regulatory agencies 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 advisory group or 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 agency policy during the period of product development, clinical studies and the review process. Regulatory agencies also may approve a treatment candidate for fewer or more limited indications than requested or may grant approval subject to the performance of post-marketing studies. Finally, regulatory agencies may not approve the labeling claims that are necessary or desirable for the successful commercialization of our treatment candidates. Even after approval and commercialization of a product candidate, we remain subject to ongoing regulatory compliance and oversight to maintain our approval. Conducting our confirmatory studies could take years to complete, could yield negative or uninterpretable results or could result in an FDA determination that the studies do not provide the safety and efficacy requirements to maintain regulatory approval. If we or any of our strategic partners are unable to develop, or obtain regulatory approval for, or, if approved, maintain regulatory compliance and successfully commercialize, our product candidates, our business will be materially harmed.

We are investing significant resources in the development of novel gene therapy product candidates. Only a few gene therapy products have been approved in the U.S. and EU. If we are unable to show the safety and efficacy of these product candidates, experience delays in doing so or are unable to successfully commercialize at least one of these drugs, our business would be materially harmed.

We are investing significant resources in the development of our gene therapy product candidates. We believe that a significant portion of the long-term value attributed to our company by investors is based on the commercial potential of these product candidates. There can be no assurance that any development problems we experience in the future related to our gene therapy programs will not cause significant delays or unanticipated costs, or that such development problems can be solved. Initial results from ongoing clinical trials may differ materially from final results from such clinical trials. The results from pre-clinical and early clinical studies do not always accurately predict results in later, large-scale clinical trials. We may also experience delays in developing a sustainable, reproducible and commercial-scale manufacturing process or transferring that process to commercial partners, which may prevent us from completing our clinical trials or commercializing our products on a timely or profitable basis, if at all.

In addition, the clinical trial requirements of the FDA, the EMA, and other regulatory agencies 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 the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or more extensively studied pharmaceutical or other product candidates. Currently, only a few gene therapy products have been approved in the Western world. Given the few precedents of approved gene therapy products, it is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our gene therapy product candidates in the U.S., the EU or other jurisdictions. Approvals by the EMA and the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. Within the FDA, the Center for Biologics Evaluation and Research (“CBER”) regulates gene therapy products. Within the CBER, the review of gene therapy and related products is consolidated in the Office of Cellular, Tissue and Gene Therapies, and the FDA has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its reviews. The CBER works closely with the National Institutes of Health (the “NIH”). The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. For example, on January 28, 2020, the FDA issued final guidance documents that updated draft guidance documents that were originally released in July 2018 to reflect recent advances in the field, and to set forth the framework for the development, review and approval of gene therapies. These final guidance documents pertain to the development of gene therapies for the treatment of specific disease categories, including rare diseases, and to manufacturing and long-term follow up issues relevant to gene therapy, among other topics. At the same time the FDA issued a new draft guidance document describing the FDA’s approach for determining whether two gene therapy products were the same or different for the purpose of assessing orphan drug exclusivity. In addition, the FDA can put an IND, on clinical hold if the information in an IND is not sufficient to assess the risks in pediatric patients.

These regulatory review agencies, committees and advisory groups and the new requirements and guidelines they promulgate may lengthen the regulatory review process, require us to perform additional or larger studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval studies, limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups and comply with applicable requirements and guidelines. If we fail to do so, we may be required to delay or discontinue development of our product candidates.

If the anticipated or actual timing of marketing approvals for our gene therapy product candidates, or the market acceptance of these product candidates, if approved, including treatment reimbursement levels agreed to by third-party payors, do not meet the expectations of investors or public market analysts, the market price of our common stock would likely decline.

Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to determine the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, such as gene therapy, there is heightened risk that the FDA, the EMA or other regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. Different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Pre-clinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent full or accelerated regulatory approval.

If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn.

Fast track product, breakthrough therapy, priority review, or Regenerative Medicine Advanced Therapy ("RMAT") designation by the FDA, or access to the PRIME scheme by the EMA, for our product candidates may not lead to 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 fast track, breakthrough therapy designation, RMAT designation, PRIME scheme access or priority review designation for our product candidates if supported by the results of clinical trials. A fast track product designation is designed to facilitate the clinical development and expedite the review of drugs intended to treat a serious or life-threatening condition which demonstrate the potential to address an unmet medical need. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. A RMAT designation is designed to accelerate approval for regenerative advanced therapies such as our gene therapy product candidates. Priority review designation is intended to speed the FDA marketing application review timeframe for drugs that treat a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. PRIME is a scheme provided by the EMA to enhance support for the development of medicines that target an unmet medical need.

For drugs and biologics that have been designated as fast track products or breakthrough therapies, or granted access to the PRIME scheme, interaction and communication between the regulatory agency and the sponsor of the trial can help to identify the most efficient path for clinical development. Sponsors of drugs with fast track products or breakthrough therapies may also be able to submit marketing applications on a rolling basis, meaning that the FDA may review portions of a marketing application before the sponsor submits the complete application to the FDA, if the sponsor pays the user fee upon submission of the first portion of the marketing application. For products that receive a priority review designation, the FDA's marketing application review goal is shortened to six months, as opposed to ten months under standard review. This review goal is based on the date the FDA accepts the marketing application for review, this application validation period typically adds approximately two months to the timeline for review and decision from the date of submission. RMAT designations will accelerate approval but the exact mechanisms have not yet been announced by FDA.

Designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product is within the discretion of the regulatory agency. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a fast track product, breakthrough therapy, RMAT, PRIME, or priority review product, the agency may disagree and instead determine not to make such designation. In any event, the receipt of such a designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional regulatory procedures and does not assure ultimate marketing approval by the agency. In addition, regarding fast track products and breakthrough therapies, the FDA may later decide that the products no longer meet the conditions for qualification as either a fast track product, RMAT, or a breakthrough therapy or, for priority review products, decide that period for FDA review or approval will not be shortened.

We may not be able to advance all of our programs, and we may use our financial and human resources to pursue particular programs and fail to capitalize on programs that may be more profitable or for which there is a greater likelihood of success.

Our pipeline includes more than 40 programs in various stages of development for a broad range of diseases and disorders. We plan to expand our pipeline through internal research and development and through strategic transactions. Because we have limited resources, we may not be able to advance all of our programs. We may also forego or delay pursuit of opportunities with certain programs or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or 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.

Risks Related to Third Parties

If we are unable to maintain our agreements with third parties to distribute our products to patients, our results of operations and business could be adversely affected.

We rely on third parties to commercially distribute our products to patients in the U.S. We have contracted with a third-party logistics company to warehouse our products and with distributors and specialty pharmacies to sell and distribute our products to patients. A specialty pharmacy is a pharmacy that specializes in the dispensing of medications for complex or chronic conditions that require a high level of patient education and ongoing management.

This distribution network requires significant coordination with our sales and marketing and finance organizations. In addition, failure to coordinate financial systems could negatively impact our ability to accurately report product revenue from our products. If we are unable to effectively manage the distribution process, the sales of our products, as well as any future products we may commercialize, could be delayed or severely compromised and our results of operations may be harmed.

In addition, the use of third parties involves certain risks, including, but not limited to, risks that these organizations will:

- not provide us with accurate or timely information regarding their inventories, the number of patients who are using our products or serious adverse events and/or product complaints regarding our products;
- not effectively sell or support our products;
- reduce or discontinue their efforts to sell or support our products;
- not devote the resources necessary to sell our products in the volumes and within the time frame we expect;
- be unable to satisfy financial obligations to us or others; or
- cease operations.

Any such events may result in decreased product sales, lower product revenue, loss of revenue, and/or reputational damage, which would harm our results of operations and business.

With respect to the pre-commercial distribution of our products to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute our products in certain countries through our EAP. We will need to continue building out our network for commercial distribution in jurisdictions in which our products are approved, which will also require third party contracts. The use of distributors and service providers involves certain risks, including, but not limited to, risks that these organizations will not comply with applicable laws and regulations, or not provide us with accurate or timely information regarding serious adverse events and/or product complaints regarding our products. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of our products in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

We rely on third parties to conduct some aspects of our early stage research and pre-clinical and clinical development. The inadequate performance by or loss of any of these third parties could affect the development and commercialization of our product candidate development.

We have relied upon, and plan to continue to rely upon, third parties to conduct some aspects of our early stage research and pre-clinical and clinical development with respect to certain of our product candidates, including our follow-on exon-skipping product candidates, PPMO, gene therapy and gene editing product candidates. Our third-party collaborators may not commit sufficient resources or adequately develop our programs for these candidates. If our third-party collaborators fail to commit sufficient resources to any of our product candidates or to carry out their contractual duties or obligations, our programs related to any particular product candidate could be delayed, terminated, or unsuccessful. Furthermore, if we fail to make required payments to these third-party collaborators, including up-front, milestone, reimbursement or royalty payments, or to observe other obligations in our agreements with them, these third parties may not be required to perform their obligations under our respective agreements with them and may have the right to terminate such agreements.

We also have relied upon and plan to continue to rely upon third-party CROs to monitor and manage data for our ongoing pre-clinical and clinical programs. We rely on these parties for execution of our pre-clinical and clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol and legal, regulatory and scientific standards, and our reliance on collaborators and CROs does not relieve us of our regulatory responsibilities.

The individuals at our third-party collaborators and CROs who conduct work on our behalf, including their sub-contractors, are not always our employees, and although we participate in the planning of our early stage research and pre-clinical and clinical programs, we cannot control whether or not they devote sufficient time and resources or exercise appropriate oversight of these programs, except for remedies available to us under our agreements with such third parties. If our collaborators and CROs do not successfully carry out their contractual duties or obligations or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our pre-clinical and clinical protocols, regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, our results of operations and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenues could be delayed.

Our reliance on third parties requires us to share our proprietary information, which increases the possibility that a competitor will discover them or that our proprietary information will be misappropriated or inadvertently disclosed.

Our reliance on third-party collaborators requires us to disclose our proprietary information to these parties, which could increase the risk that a competitor will discover this information or that this information will be misappropriated or disclosed without our intent to do so. Furthermore, if these third parties cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our products or product candidates, our development programs may be delayed. Although we carefully manage our relationships with our third-party collaborators and CROs, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not have a material adverse impact on our business, financial condition and prospects.

Risks Related to Manufacturing

We currently rely on third parties to manufacture our products and to produce our product candidates; our dependence on these parties, including failure on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial, EAP, clinical and pre-clinical product demand may impair the availability of product to successfully support various programs, including research and development and the potential commercialization of our product candidates.

We currently do not have the internal ability to undertake the manufacturing process for our products or product candidates in the quantities needed to meet commercial, clinical or EAPs demand for our products, or to conduct our research and development programs and conduct clinical trials. Therefore, we rely on, and expect to continue relying on for the foreseeable future, a limited number of third parties to manufacture and supply materials (including raw materials and subunits), API and drug product, as well as to perform additional steps in the manufacturing process, such as labeling and packaging of vials and storage of our products and product candidates. The limited number of third parties with facilities and capabilities suited for the manufacturing process of our products and product candidates creates a heightened risk that we may not be able to obtain materials and APIs in the quantity and purity that we require.

In addition, the process for adding new manufacturing capacity is lengthy and often causes delays in development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as the ongoing COVID-19 pandemic, order delays for equipment or materials, equipment malfunctions, quality control and quality assurance issues, regulatory delays and possible negative effects of such delays on supply chains and expected timelines for product availability,

production yield issues, shortages of qualified personnel, discontinuation of a facility or business or failure or damage to a facility by natural disasters, such as earthquakes or fires, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in supply of our products, product candidates or materials.

If these third parties cease providing quality manufacturing and related services to us, and we are not able to engage appropriate replacements in a timely manner, our ability to manufacture our products or product candidates in sufficient quality and quantity required for our planned commercial, pre-clinical and clinical or EAPs, our various product research, development and commercialization efforts would be adversely affected.

Furthermore, any problems in our manufacturing process or the facilities with which we contract 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 third-party manufacturing processes or facilities also restrict our ability to meet market demand.

We, through our third-party manufacturers, seek to produce or produce supply of our products and product candidates. In light of the limited number of third parties with the expertise to produce our products and product candidates, the lead time needed to manufacture them, and the availability of underlying materials, we may not be able to, in a timely manner or at all, establish or maintain sufficient commercial and other manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of our products and product candidates. Furthermore, we may not be able to obtain the significant financial capital that may be required in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for our products and product candidates, such parties may not comply with the terms and timelines they have agreed to for various reasons, some of which may be out of their or our control, which impacts our ability to execute our business plans on expected or required timelines in connection with the commercialization of our products and the continued development of our product candidates. When we enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, we constrain our operational flexibility.

The third parties we use in the manufacturing process for our products and product candidates may fail to comply with cGMP regulations.

Our contract manufacturers are required to produce our materials, APIs and drug products under cGMP. We and our contract manufacturers are subject to periodic inspections by the FDA, EMA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations. In addition, before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA, which includes a review of the manufacturing process and facility. A manufacturing authorization also must be obtained from the appropriate EU regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. In order to obtain approval, we need to demonstrate that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. In complying with cGMP, we are obligated to expend time, money and effort in production, record keeping and quality control to seek to assure that the product meets applicable specifications and other requirements.

We do not have direct operational control over a third-party manufacturer’s compliance with regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of our products and product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. Failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, results in product recalls, clinical holds, delayed or withheld approvals, patient injury or death.

This risk is particularly heightened as we optimize manufacturing for our product candidates. For example, we were notified by the Research Institute at Nationwide that they received a letter from the FDA on July 24, 2018, stating that their Phase 1/2a DMD micro-dystrophin gene therapy trial had been placed on clinical hold due to the presence of a trace amount of DNA fragment in research-grade third-party supplied plasmid (the “Clinical Hold”). The Research Institute, working with us, developed an action plan with immediate plans to submit for review by the FDA, which included the use of cGMP plasmid for the program. On September 24, 2018, we announced that the FDA had lifted the Clinical Hold.

Failure by our contract manufacturers to adhere to applicable cGMP and other applicable government regulations, or our contract manufacturers experiencing manufacturing problems, may result in significant negative consequences, including product seizures or recalls, postponement or cancellation of clinical trials, loss or delay of product approval, fines and sanctions, loss of revenue, termination of the development of a product candidate, reputational damage, shipment delays, inventory shortages, inventory write-offs and other product-related charges and increased manufacturing costs. If we experience any of these consequences, the success of our commercialization of our products and/or our development efforts for our product candidates could be significantly delayed, fail or otherwise be negatively impacted.

We may not be able to successfully scale up manufacturing of our products or product candidates in sufficient quality and quantity or within targeted timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact the commercial success of our products and/or the development of our product candidates.

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. Our focus remains on (i) achieving larger-scale manufacturing capacity for our products and product candidates throughout the manufacturing supply chain, (ii) continuing to increase material and API production capacity to provide the anticipated amounts of drug product needed for our planned studies for our product candidates and (iii) optimizing manufacturing for our follow-on exon skipping product candidates and other programs, including PPMO and gene therapy. We may not be able to successfully increase manufacturing capacity or scale up the production of materials, APIs and drug products, whether in collaboration with third party manufacturers or on our own, in a manner that is safe, compliant with cGMP conditions or other applicable legal or regulatory requirements, in a cost-effective manner, in a time frame required to meet our timeline for commercialization, clinical trials and other business plans, or at all.

Challenges complying with cGMP requirements and other quality issues arise during efforts to increase manufacturing capacity and scale up production. We experience such issues in connection with manufacturing, packaging and storage of our products and product candidates, and during shipping and storage of the APIs or finished drug product. In addition, in order to release our products for commercial use and demonstrate stability of product candidates for use in clinical trials (and any subsequent drug products for commercial use), our manufacturing processes and analytical methods must be validated in accordance with regulatory guidelines. Failure to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of our products or product candidates in a timely or cost-effective manner, or at all, may undermine our commercial efforts. Failure to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, will negatively impact the commercial availability of our products and the continued development and/or regulatory approval of our product candidates, which could significantly harm our business.

During our work with our third-party manufacturers to increase and optimize manufacturing capacity and scale up production, they may make proprietary improvements in the manufacturing and scale-up processes for our products or product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, we may need additional processes, technologies and validation studies, which could be costly and which we may not be able to develop or acquire from third parties. Failure to secure the intellectual property rights required for the manufacturing process needed for large-scale clinical trials or commercialization of our products or the continued development of our product candidates could cause significant delays in our business plans or otherwise negatively impact the commercialization of our products or the continued development of our product candidates.

Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization of gene therapy programs, limit the supply of our products or otherwise harm our business.

We currently have development, manufacturing and testing agreements with third parties to manufacture supplies of our gene therapy product candidates. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers.

The physical and chemical properties of biologics 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 the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical and/or commercial-grade materials that meet FDA, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs.

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 competent authority authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability and deviations among different sites, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

As our product candidates advance to later stage clinical trials, it is customary that various aspects of the development program, such as manufacturing, formulation and other processes, and methods of administration, may be altered to optimize the candidates and processes for scale-up necessary for later stage clinical trials and potential approval and commercialization. These changes may not produce the intended optimization, including production of drug substance and drug product of a quality and in a quantity sufficient for Phase 3 clinical stage development or for commercialization, which may cause delays in the initiation or completion of clinical trials and greater costs. We may also need to conduct additional studies to demonstrate comparability between newly manufactured drug substance and/or drug product for commercialization relative to previously manufactured drug substance and/or drug product for clinical trials. Demonstrating comparability may require us to incur additional costs or delay initiation or completion of clinical trials and, if unsuccessful, could require us to complete additional preclinical studies or clinical trials.

We also may encounter problems hiring and retaining the experienced 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.

Furthermore, no manufacturer currently has the capacity and scalability to produce our vectors or gene therapy product candidates at commercial levels. Even if we timely develop a manufacturing process and successfully transfer it to the third-party vector and product manufacturers or successfully and timely develop our internal capacity, if we or such third-party manufacturers are unable to produce the necessary quantities of viral vectors and our product candidates, or in compliance with GMP or other pertinent regulatory requirements, and within our planned time frame and cost parameters, it may result in delays in our development plans or increased capital expenditures, and the development and sales of our products, if approved, may be materially harmed.

Risks Related to our Intellectual Property

Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors and other collaborators to obtain, maintain and defend the patent protection for our products, product candidates, and platform technologies, to preserve our trade secrets, and to prevent third parties from infringing on our proprietary rights.

We currently directly hold various issued patents and patent applications, or have exclusive license or option rights to issued patents and patent applications, in each case in the U.S. as well as other countries that protect our products, product candidates and platform technologies. We anticipate filing additional patent applications both in the U.S. and in other countries. Our success will depend, in significant part, on our ability to obtain, maintain and defend our U.S. and foreign patents covering our products, product candidates and platform technologies as well as preserving our trade secrets for these assets. The patent process is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining, maintaining, or defending our patents. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect our products, product candidates or platform technologies.

The patent positions of pharmaceutical, biotechnology and other life sciences companies can be highly uncertain and involve complex legal and factual questions for which important legal principles remain unresolved. This uncertainty is heightened for our PMO-based products and product candidates and gene therapy-based product candidates for which there has been little patent litigation involving such technologies. No consistent policy regarding the breadth of claims allowed in biotechnology patents has emerged to date in the U.S. and tests used for determining the patentability of patent claims in all technologies are in flux. The USPTO and patent offices in other jurisdictions have often required that patent applications concerning pharmaceutical and/or biotechnology-related inventions be limited or narrowed substantially to cover only the specific innovations exemplified in the patent application, thereby limiting the scope of protection against competitive challenges. Accordingly, even if we or our licensors are able to obtain patents, the patents might be substantially narrower than anticipated. Thus, there is no assurance as to the degree and range of protections any of our patents, if issued, may afford us or whether patents will be issued. Patents which may be issued to us may be subjected to further governmental review that may ultimately result in the reduction of their scope of protection, and pending patent applications may have their requested breadth of protection significantly limited before being issued, if issued at all. The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain.

As a matter of public policy, there might be significant pressure on governmental bodies to limit the scope of patent protection or impose compulsory licenses for disease treatments that prove successful, particularly as a tactic to impose a price control. Additionally, competitors may leverage such pressure to enhance their ability to exploit these laws to create, develop and market competing products.

We may be able to assert that certain activities engaged in by our competitors infringe on our current or future patent rights. To the extent that we enforce our patents, an alleged infringer may deny infringement and/or counter-claim that our patents are not valid, and if successful, could negatively impact our patent estate. We may not be able to successfully defend patents necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated,

circumvented or otherwise not provide any competitive advantage. Defending our patent positions may require significant financial resources and could negatively impact other Company objectives.

Under the Hatch-Waxman Act, one or more motivated third parties may file an ANDA, seeking approval of a generic copy of an innovator product approved under the NDA pathway such as our PMO products, or a NDA under Section 505(b)(2), which may be for a new or improved version of the original innovator products. The third parties are allowed to rely on the safety and efficacy data of the innovator's product, may not need to conduct clinical trials and can market a competing version of a product after the expiration or loss of patent exclusivity or the expiration or loss of regulatory exclusivity and often charge significantly lower prices. Upon the expiration or loss of patent protection or the expiration or loss of regulatory exclusivity for a product, the major portion of revenues for that product may be dramatically reduced in a very short period of time. If we are not successful in defending our patents and regulatory exclusivities, we will not derive the expected benefit from them. As such, a third party could be positioned to market an ANDA or Section 505(b)(2) product that competes with one of our products prior to the expiry of our patents if the third party successfully challenged the validity of our patents protecting the product.

The DMD patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe our current or future patent rights. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights. As such, the patents and patent applications that we own, license, have optioned, and rely on for exclusivity for our product candidates may be challenged.

Uncertainty over intellectual property in the pharmaceutical and biotechnology industry has been the source of litigation and other disputes, which is inherently costly and unpredictable.

Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are, have been and may in the future be necessary in some instances to determine the validity and scope of certain of our proprietary rights, and in other instances to determine the validity, scope or non-infringement of certain patent rights claimed by third parties to be pertinent to the manufacture, use or sale of our products. We may also face challenges to our patent and regulatory exclusivities covering our products by third parties, including manufacturers of generics and biosimilars that may choose to launch or attempt to launch their products before the expiration of our patent or regulatory exclusivity. Litigation, interferences, oppositions, inter partes reviews, administrative challenges or other similar types of proceedings are unpredictable and may be protracted, expensive and distracting to management. The outcomes of such proceedings could adversely affect the validity and scope of our patents or other proprietary rights, hinder our ability to manufacture and market our products, require us to seek a license for the infringed products or technology or result in the assessment of significant monetary damages against us that may exceed amounts, if any, accrued in our financial statements. An adverse determination in a judicial or administrative proceeding or a failure to obtain necessary licenses could prevent us from manufacturing or selling our products. Furthermore, payments under any licenses that we are able to obtain would reduce our profits derived from our products. Any of these circumstances could result in financial, business or reputational harm to us or could cause a decline or volatility in our stock price.

On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act"), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted, and may also affect patent litigation. The USPTO has issued regulations and procedures to govern administration of the Leahy-Smith Act. In view of the long timelines for interpreting legal provisions in the court system and the evolving nature of our laws, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. 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, all of which could have a material adverse effect on our business and financial condition. For instance, a third party may petition the Patent Trial and Appeal Board ("PTAB") seeking to challenge the validity of some or all of the claims in any of our patents through an inter partes review or other post-grant proceeding. Should the PTAB institute an inter partes review or other proceeding and decide that some or all of the claims in the challenged patent are invalid, such a decision, if upheld on appeal, could have a material adverse effect on our business and financial condition.

Our business prospects will be impaired if third parties successfully assert that our products, product candidates, or platform technologies infringe proprietary rights of such third parties.

Similar to us, competitors continually seek intellectual property protection for their technology. Several of our development programs, particularly gene therapy programs, focus on therapeutic areas that have been the subject of extensive research and development by third parties for many years and have been protected with third party patent rights. Due to the amount of intellectual property in our various fields of technology, we cannot be certain that we do not infringe intellectual property rights of competitors or other third parties or that we will not infringe intellectual property rights of competitors or other third parties granted or created in the future. Our competitors or other third parties might have obtained, or could obtain in the future, patents that limit, interfere with or eliminate our ability to make, use and sell our products, product candidates or platform technologies in important commercial markets.

In order to maintain or obtain freedom to operate for our products and product candidates, we may incur significant expenses, including those associated with entering into agreements with third parties that require milestone and royalty payments. Additionally, if we were to challenge the patent rights of our competitors, we could incur substantial costs and ultimately might not be successful.

If our products, product candidates, or platform technologies are alleged to infringe or are determined to infringe enforceable proprietary rights of others, we could incur substantial costs and may have to:

- obtain rights or licenses from others, which might not be available on commercially reasonable terms or at all;
- abandon development of an infringing product candidate, or cease commercialization of an infringing product;
- redesign our products, product candidates or processes to avoid infringement;
- pay damages; and/or
- defend litigation or administrative proceedings which might be costly whether we win or lose, and which could result in a substantial diversion of financial and management resources.

Any of these events could result in product and product candidate development delays or cessation, and as such substantially harm our potential earnings, financial condition and operations. The patent landscape of our product candidates is continually evolving and multiple parties, including both commercial entities and academic institutions, may have rights to claims or may be pursuing additional claims that could provide these parties a basis to assert that our products, product candidates or platform technologies infringe on the intellectual property rights of such parties. There has been, and we believe that there will continue to be, significant litigation in the biopharmaceutical and pharmaceutical industries regarding patent and other intellectual property rights.

Risks Related to our Business Operations

The outbreak of COVID-19 may result in disruptions to our commercialization, clinical trials, manufacturing and other business operations, which could have a material adverse effect on our business, financial condition, operating results, cash flows and prospects.

In December 2019, an outbreak of a novel strain of coronavirus ("*COVID*-19") originated in Wuhan, China and has since spread to a number of other countries, including the United States. On March 11, 2020, the World Health Organization characterized *COVID*-19 as a pandemic. The rapid spread of COVID-19 has led to the implementation of various responses, including government-imposed quarantines, shelter-in-place mandates, sweeping restrictions on travel, mandatory shutdowns for non-essential businesses, requirements regarding social distancing, and other public health safety measures, as well as reported adverse impacts on healthcare resources, facilities and providers across the United States and in other countries. In response to the pandemic, healthcare providers have, and may need to further, reallocate resources, such as physicians, staff, hospital beds, and intensive care unit facilities, as they prioritize limited resources and personnel capacity to focus on the treatment of patients with COVID-19 and implement limitations on access to hospitals and other medical institutions due to concerns about the potential spread of COVID-19 in such settings. These actions may negatively impact commercialization, clinical trials, manufacturing and other business operations, including:

- **Commercial:** The response to COVID-19 by healthcare providers has made it difficult for some patients, especially those dependent on a hospital setting, to receive infusions or initiate treatment with our commercial products. In addition, as a result of the pandemic, some patients may choose to delay or stop treatment to avoid a visit to a hospital or a visit of a third party in their homes to minimize the risk of infection. The impact of COVID-19 may also result in delays in processing reauthorizations and modifications to program benefits by insurers, making it difficult for patients to obtain or maintain favorable coverage decisions for our products. These challenges may continue for the duration of the COVID-19 pandemic, which is uncertain, and are expected to reduce our revenue and cash flows.
- **Clinical trials:** The impact of COVID-19 has caused, and may continue to cause, delays and disruptions to some of our clinical trials. The response to COVID-19 by healthcare providers is expected to delay site initiation, slow down enrollment, and make the ongoing collection of data for patients enrolled in studies more difficult or intermittent. In

addition, patients may be unable or unwilling to travel to clinical trial sites as a result of quarantines and other restrictions, which may negatively impact the execution of clinical trials. Significant delays or disruptions to our clinical trials could adversely affect our ability to timely initiate studies, conduct successful studies, obtain or maintain regulatory approvals, or commercialize our product candidates.

- **Manufacturing:** A prolonged outbreak could lead to delays in the manufacturing of our products and product candidates.
- **Operations:** On March 13, 2020, to protect the health of our employees and their families, and our communities, and in accordance with direction from state and local government authorities, we instituted mandatory work-from-home for all employees and contingent workers other than those who are facility-dependent. Our increased reliance on personnel working from home may negatively impact productivity or disrupt, delay or otherwise adversely impact our business. In addition, remote working could increase our cyber security risk. General protective measures put into place at various governmental levels, including quarantines, travel restrictions and business shutdowns, may also negatively affect our operations.
- **Share Price:** The extent and duration of the impact of the COVID-19 pandemic on our stock price is uncertain. The COVID-19 pandemic may cause our stock price to be more volatile, and our ability to raise capital could be impaired.

Any of the foregoing factors could have a material adverse impact on our business, financial condition, operating results, cash flows and prospects. The extent to which COVID-19 impacts our operations and those of our third-party partners will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration of the pandemic, additional or modified government actions, new information which emerges concerning the severity of COVID-19 and the actions taken to contain the virus or treat its impact, among others. In particular, the speed of the continued spread of COVID-19 globally, and the magnitude of interventions to contain the spread of the virus, will determine the impact of the pandemic on our operations.

If we fail to comply with healthcare and other regulations, we could face substantial penalties and our business, operations and financial condition could be adversely affected.

As a manufacturer of pharmaceuticals, within the U.S., certain federal and state healthcare laws and regulations will apply to or affect our business. The laws and regulations include:

- federal healthcare program anti-kickback laws, which prohibit, among other things, persons from soliciting, receiving or providing remuneration, directly or indirectly, to induce either the referral of an individual, for an item or service or the purchasing or ordering of a good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- federal false claims laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, information or claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent;
- the Federal Food, Drug and Cosmetic Act, which among other things, strictly regulates drug product and medical device marketing, prohibits manufacturers from marketing such products for off-label use and regulates the distribution of samples;
- federal laws that require pharmaceutical manufacturers to report certain calculated product prices to the government or provide certain discounts or rebates to government authorities or private entities, often as a condition of reimbursement under government healthcare programs;
- the so-called “federal sunshine” law, which requires pharmaceutical and medical device companies to monitor and report certain financial interactions with physicians and teaching hospitals to the federal government for re-disclosure to the public; and
- state law equivalents of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third party payor, including commercial insurers, state laws regulating interactions between pharmaceutical manufactures and health care providers, 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 often are not preempted by federal laws, thus complicating compliance efforts.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being used to enforce these laws and to prosecute companies and individuals who are believed to be violating them. We anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions.

In connection with the commercial launch of our products, we have enhanced our compliance program, which is based on industry best practices and is designed to ensure that the commercialization of our products complies with all applicable laws, regulations and industry standards. As the requirements in this area are constantly evolving, we cannot be certain that our program will eliminate all areas of potential exposure. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. If our operations are found to be in violation of any of the laws described above or any other laws, rules or regulations that apply to us, we will be subject to penalties, including civil and criminal penalties, damages, fines and the curtailment or restructuring of our operations. Any penalties, damages, fines, curtailment or restructuring of our operations could adversely affect our ability to operate our business and our financial results. Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business. Even if we successfully defend against an action against us for violation of a law, the action and our defense could nonetheless cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security, fraud and reporting laws may prove costly.

We may be subject to product liability claims and our insurance may not be adequate to cover damages.

The current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of our products, or the use of our products under emergency use vehicles may expose us to liability claims inherent to the manufacture, clinical testing, marketing and sale of medical products. These claims might be made directly by consumers or healthcare providers or indirectly by pharmaceutical companies, our collaborators or others selling such products. Regardless of merit or eventual outcome, we may experience financial losses in the future due to such product liability claims. We have obtained commercial general liability insurance coverage for our clinical trials and the sale of commercial products. However, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against all losses. If a successful product liability claim or series of claims is brought against us for uninsured liabilities or in excess of insured liabilities, our assets may not be sufficient to cover such claims and our business operations could be impaired.

If we, our collaborators, or any third-party manufacturers engaged by us or our collaborators fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We, our collaborators, and any third-party manufacturers we engage 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, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. Our operations involve the use of hazardous materials, including organic and inorganic solvents and reagents. Although we believe that our activities conform in all material respects with such environmental laws, there can be no assurance that violations of these laws will not occur in the future as a result of human error, accident, equipment failure or other causes. Liability under environmental, health and safety laws can be joint and several and without regard to fault or negligence. The failure to comply with past, present or future laws could result in the imposition of substantial fines and penalties, remediation costs, property damage and personal injury claims, loss of permits or a cessation of operations, and any of these events could harm our business and financial condition. We expect that our operations will be affected by other new environmental, health and workplace safety laws on an ongoing basis, and although we cannot predict the ultimate impact of any such new laws, they may impose greater compliance costs or result in increased risks or penalties, which could harm our business.

Further, with respect to the operations of any current or future collaborators or third party contract manufacturers, it is possible that if they fail to operate in compliance with applicable environmental, health and safety laws and regulations or properly dispose of wastes associated with our product or product candidates, we could be held liable for any resulting damages, suffer reputational harm or experience a disruption in the manufacture and supply of our product or product candidates.

Violation of the General Data Protection Regulation could subject us to significant fines.

The GDPR increases our obligations with respect to clinical trials conducted in the member states of 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 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 U.S. The GDPR imposes substantial fines for breaches of data protection requirements, which can be up to four percent of global revenue or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects for breaches of data protection requirements. Compliance with these directives will be a rigorous and time-intensive process that may increase our cost of doing business, and despite those efforts, there is a risk that we may be subject to fines and penalties, litigation and reputational harm in connection with our European activities.

If we fail to retain our key personnel or are unable to attract and retain additional qualified personnel, our future growth and our ability to compete would suffer.

We are highly dependent on the efforts and abilities of the principal members of our senior management. Additionally, we have scientific personnel with significant and unique expertise in RNA-targeted therapeutics and gene therapy technologies. The loss of the services of any one of the principal members of our managerial team or staff may prevent us from achieving our business objectives.

The competition for qualified personnel in the biotechnology field is intense, and our future success depends upon our ability to attract, retain and motivate such personnel. In order to develop and commercialize our products successfully, we will be required to retain key management and scientific employees. In certain instances, we may also need to expand or replace our workforce and our management ranks. In addition, we rely on certain consultants and advisors, including scientific and clinical advisors, to assist us in the formulation and advancement of our research and development programs. Our consultants and advisors may be employed by other entities or have commitments under consulting or advisory contracts with third parties that limit their availability to us, or both. If we are unable to attract, assimilate or retain such key personnel, our ability to advance our programs would be adversely affected.

We expect to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.

As our business activities expand, we expect to expand our full-time employee base and to hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities and devote a substantial amount of time to managing these growth activities. Our ability to manage our growth properly and maintain compliance with all applicable rules and regulations will require us to continue to improve our operational, legal, financial and management controls, as well as our reporting systems and procedures. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced, and we may not be able to implement our business strategy.

Our sales and operations are subject to the risks of doing business internationally.

We are increasing our presence in international markets, including emerging markets, subjecting us to many risks that could adversely affect our business and revenues, such as:

- the inability to obtain necessary foreign regulatory or pricing approvals of products in a timely manner;
- uncertainties regarding the collectability of accounts receivable;
- fluctuations in foreign currency exchange rates that may adversely impact our revenues, net income and value of certain of our investments;
- difficulties in staffing and managing international operations;
- the imposition of governmental controls;
- less favorable intellectual property or other applicable laws;
- increasingly complex standards for complying with foreign laws and regulations that may differ substantially from country to country and may conflict with corresponding U.S. laws and regulations;
- the far-reaching anti-bribery and anti-corruption legislation in the U.K., including the U.K. Bribery Act 2010, and elsewhere and escalation of investigations and prosecutions pursuant to such laws;
- compliance with complex import and export control laws;
- restrictions on direct investments by foreign entities and trade restrictions; and
- changes in tax laws and tariffs.

In addition, our international operations are subject to regulation under U.S. law. For example, the Foreign Corrupt Practices Act (“FCPA”) prohibits U.S. companies and their representatives from paying, offering to pay, promising to pay or authorizing the payment of anything of value to any foreign government official, government staff member, political party or political candidate for the purpose of obtaining or retaining business or to otherwise obtain favorable treatment or influence a person working in an official capacity. In many countries, the health care professionals we regularly interact with may meet the FCPA's definition of a foreign

government official. Failure to comply with domestic or foreign laws could result in various adverse consequences, including: possible delay in approval or refusal to approve a product, recalls, seizures or withdrawal of an approved product from the market, disruption in the supply or availability of our products or suspension of export or import privileges, the imposition of civil or criminal sanctions, the prosecution of executives overseeing our international operations and damage to our reputation. Any significant impairment of our ability to sell products outside of the U.S. could adversely impact our business and financial results.

Unfavorable global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. A severe or prolonged economic downturn could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could strain our manufacturers, possibly resulting in manufacturing disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

Additionally, in June 2016, a majority of United Kingdom ("UK") voters voted for the UK to exit the EU (Brexit) and, on January 31, 2020, the UK's withdrawal became effective. A transition period will apply until the end of 2020 (or later, if extended) during which the pre-Brexit legal regime will continue to apply with the UK and the EU negotiate rules that will apply to their future relationship. The economic effects of Brexit will depend on any agreements the UK makes to retain access to EU markets either during a transitional period or more permanently. Brexit could adversely affect European and worldwide economic or market conditions and could contribute to instability in global financial markets. Brexit is likely to lead to legal uncertainty and potentially divergent national laws and regulations as the UK determines which EU laws to replace or replicate. Any of these effects of Brexit, and any other effects we cannot anticipate, could adversely affect our business, business opportunities, results of operations, financial condition and cash flows.

Moreover, the COVID-19 pandemic is impacting the global economy, and the U.S. economy in particular, with the potential for the economic downturn to be severe and prolonged. A severe or prolonged economic downturn as a result of the COVID-19 pandemic could result in a variety of risks to our business, including disruptions in the financial markets, which could adversely impact our ability to raise additional capital when needed or on acceptable terms, if at all.

We rely significantly on information technology and any failure, inadequacy, interruption or security lapse of that technology, including any cyber security incidents, could harm our ability to operate our business effectively.

In the ordinary course of our business, we collect and store sensitive data, including intellectual property, our proprietary business information and that of our suppliers, as well as personally identifiable information of the patients using our commercially approved products, clinical trial participants and employees. Similarly, our third-party providers possess certain of our sensitive data. The secure maintenance of this information is critical to our operations and business strategy. Despite our security measures, our information technology and infrastructure may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. Any such breach could compromise our networks and the information stored there could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure or other loss of information, including our data being breached at third party providers, could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations and damage our reputation, which could adversely affect our business.

We may incur substantial costs in connection with litigation and other disputes.

In the ordinary course of business we may, and in some cases have, become involved in lawsuits and other disputes such as securities claims, intellectual property challenges, including interferences declared by the USPTO, and employee matters. It is possible that we may not prevail in claims made against us in such disputes even after expending significant amounts of money and company resources in defending our positions in such lawsuits and disputes. The outcome of such lawsuits and disputes is inherently uncertain and may have a negative impact on our business, financial condition and results of operations.

Comprehensive tax reform in the U.S. and future guidance could adversely affect our business and financial condition.

The Tax Cuts and Jobs Act (the "TCJA") was enacted on December 22, 2017 in the U.S. The TCJA contains significant changes to corporate taxation, including reduction of the U.S. corporate tax rate from 35% to 21%, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, limitation of the tax deduction for interest expense, immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits.

We continue to monitor for legislative developments, issuance of regulations and technical memorandum to provide further clarification and/or interpretations of the TCJA.

Our ability to use net operating loss carryforwards and other tax attributes to offset future taxable income may be limited as a result of future transactions involving our common stock.

In general, under Section 382 of the Internal Revenue Code, a corporation that undergoes an ‘‘ownership change’’ is subject to limitations on its ability to utilize its pre-change net operating losses and certain other tax assets to offset future taxable income. In general, an ownership change occurs if the aggregate stock ownership of certain stockholders increases by more than 50 percentage points over such stockholders’ lowest percentage ownership during the testing period, which is generally three years. An ownership change could limit our ability to utilize our net operating loss and tax credit carryforwards for taxable years including or following such “ownership change.” Limitations imposed on the ability to use net operating losses and tax credits to offset future taxable income could require us to pay U.S. federal income taxes earlier than we estimated or than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes.

We are winding down our expired U.S. government contracts, and the U.S. government may deny payment of some or all of the currently outstanding amounts owed to us. In addition, further development of our infectious disease programs may be limited by the intellectual property and other rights retained by the U.S. government.

We have historically relied on U.S. government contracts and awards to fund and support certain infectious disease development programs. These contracts are expired and we are currently involved in contract close-out activities. The U.S. government has the right to perform additional audits prior to making final payment of costs and fees. If we are not able to adequately support costs incurred or other government requirements, the government may deny payment of some or all of the currently outstanding amounts owed to us. In addition, the U.S. government may have the right to develop all or some parts of product candidates that we have developed under a U.S. government contract after such contract has terminated or expired.

Our employees, principal investigators, consultants and strategic partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and strategic partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the U.S. and abroad, report financial information or data accurately or disclose unauthorized activities to us. We adopted a code of conduct applicable to all of our employees, but 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 comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

The increasing use of social media platforms presents new risks and challenges.

Social media is increasingly being used to communicate about our products, technologies and programs, and the diseases our product and product candidates are designed to treat. Social media practices in the biopharmaceutical industry continue to evolve and regulations relating to such use are not always clear. This evolution creates uncertainty and risk of noncompliance with regulations applicable to our business. For example, patients may use social media channels to comment on the effectiveness of a product or to report an alleged adverse event. When such disclosures occur, there is a risk that we fail to monitor and comply with applicable adverse event reporting obligations or we may not be able to defend ourselves or the public's legitimate interests in the face of the political and market pressures generated by social media due to restrictions on what we may say about our product and/or product candidates. There is also a risk of inappropriate disclosure of sensitive information or negative or inaccurate posts or comments about us on any social networking website. If any of these events were to occur or we otherwise fail to comply with applicable regulations, we could incur liability, face overly restrictive regulatory actions or incur other harm to our business.

We or the third parties upon whom we depend may be adversely affected by natural disasters and/or terrorism attacks, and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations, and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, power outage, terrorism attack or other event occurred that prevented us from using all or a significant portion of our office, manufacturing and/or lab spaces, that damaged critical infrastructure, such as the manufacturing facilities of our third-party contract manufacturers, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Risks Related to our Financial Condition and Capital Requirements

We have incurred operating losses since our inception and we may not achieve or sustain profitability.

We incurred an operating loss of $118.0 million for the three months ended March 31, 2020. Our accumulated deficit was $2.3 billion as of March 31, 2020. Although we currently have two commercially approved products in the U.S., we believe that it will take us some time to attain profitability and positive cash flow from operations. Since our products and product candidates target small patient populations, the per-patient drug pricing must be high in order to recover our development and manufacturing costs, fund adequate patient support programs, fund additional research and achieve profitability. We may be unable to maintain or obtain sufficient sales volumes at a price high enough to justify our product development efforts and our sales, marketing and manufacturing expenses.

We have generally incurred expenses related to research and development of our technologies and product candidates and from general and administrative expenses that we have incurred while building our business infrastructure. We anticipate that our expenses will increase substantially if and/or as we:

- continue the commercialization of our products in the U.S.;
- expand the global footprint of our products outside of the U.S.;
- establish our sales, marketing and distribution capabilities;
- continue our research, pre-clinical and clinical development of our product candidates;
- respond to and satisfy requests and requirements from regulatory authorities in connection with development and potential approval of our product candidates;
- initiate additional clinical trials for our product candidates;
- seek marketing approvals for our product candidates that successfully complete clinical trials;
- acquire or in-license other product candidates;
- maintain, expand and protect our intellectual property portfolio;
- increase manufacturing capabilities, including capital expenditures related to our real estate facilities and entering into manufacturing agreements;
- hire additional clinical, quality control and scientific personnel; and
- add operational, financial and management information systems and personnel, including personnel to support our product development and planned future commercialization efforts.

As a result, we expect to continue to incur significant operating losses at least through 2020. Because of the numerous risks and uncertainties associated with developing biopharmaceutical products, we are unable to predict the extent of any future losses or when, or if, we will become profitable.

We will need to raise 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 will likely require additional capital from time to time in the future in order to meet FDA post-marketing approval requirements and market and sell our products as well as to continue the development of product candidates in our pipeline, to prepare for potential commercialization of our product candidates, to expand our product portfolio and to continue or enhance our business development efforts. The actual amount of funds that we may need and the sufficiency of the capital we have or are able to raise will be determined by many factors, some of which are in our control and others that are beyond our control.

While we are currently well capitalized, we may use available capital resources sooner than we expect under our current operating plan. In addition, our operating plan may change. We may need or choose to seek additional funds sooner than planned, through equity or debt financings, government or other third-party funding, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. In any event, we expect to require additional capital to expand future development efforts, obtain regulatory approval for, and to commercialize, our product candidates. Raising funds in the current economic environment may present additional challenges. Even if we believe we have sufficient funds for

our current or future operating plans, we may seek additional capital if market conditions are favorable or in light of specific strategic considerations.

Any additional fundraising efforts may divert our management from their day-to-day activities, which may adversely affect our ability to develop and commercialize our product candidates. In addition, we cannot guarantee that future financing will be available in sufficient amounts or on terms acceptable to us, if at all. In the event we receive negative data from our key clinical programs or encounter other major setbacks in our development, manufacturing or regulatory activities or in our commercialization efforts, our stock price is likely to decline, which would make a future financing more difficult. Moreover, the terms of any financing may adversely affect the holdings or the rights of our stockholders. The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our shares to decline. The sale of additional equity or convertible securities would dilute all of our stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and we may be required to agree to certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire, sell or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. We could also be required to seek funds through arrangements with collaborative partners or otherwise at an earlier stage than otherwise would be desirable and we may be required to relinquish rights to some of our technologies or product candidates or otherwise agree to terms unfavorable to us, any of which may have a material adverse effect on our business, operating results and prospects.

Moreover, the COVID-19 pandemic is impacting the global economy, and the U.S. economy in particular, with the potential for the economic downturn to be severe and prolonged. A severe or prolonged economic downturn as a result of the COVID-19 pandemic could result in a variety of risks to our business, including disruptions in the financial markets, which could adversely impact our ability to raise additional capital when needed or on acceptable terms, if at all.

If we are unable to obtain funding on a timely basis, we may be required to significantly curtail, delay or discontinue one or more of our research or development programs or the commercialization of any product, if approved, or be unable to expand our operations or otherwise capitalize on our business opportunities, as desired, which could materially affect our business, financial condition and results of operations.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights.

We may seek additional capital through a combination of private and public equity offerings, debt financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, the ownership interest of our stockholders in our company will be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect the rights of our stockholders. Debt financing, if available, would increase our fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to our product candidates, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us.

The estimates and judgments we make, or the assumptions on which we rely, in preparing our consolidated financial statements could prove inaccurate.

Our consolidated financial statements have been prepared in accordance with accounting principles generally accepted in the U.S. The preparation of these consolidated financial statements requires us to make estimates and judgments that affect the reported amounts of our assets, liabilities, revenues and expenses, the amounts of charges accrued by us and related disclosure of contingent assets and liabilities. Such estimates and judgments include revenue recognition, inventory, valuation of stock-based awards, research and development expenses and income tax. We base our estimates on historical experience, facts and circumstances known to us and on various other assumptions that we believe to be reasonable under the circumstances. We cannot provide assurances, however, that our estimates, or the assumptions underlying them, will not change over time or otherwise prove inaccurate. If this is the case, we may be required to restate our consolidated financial statements, which could, in turn, subject us to securities class action litigation. Defending against such potential litigation relating to a restatement of our consolidated financial statements would be expensive and would require significant attention and resources of our management. Moreover, our insurance to cover our obligations with respect to the ultimate resolution of any such litigation may be inadequate. As a result of these factors, any such potential litigation could have a material adverse effect on our financial results and cause our stock price to decline, which could in turn subject us to securities class action litigation.

Risks Related to Our Common Stock

Our stock price is volatile and may fluctuate due to factors beyond our control.

The market prices for and trading volumes of securities of biotechnology companies, including our securities, has historically been volatile. Our stock has had significant swings in trading prices, in particular in connection with our public communications regarding feedback received from regulatory authorities. For example, over the last thirty-six months, our stock has increased as much as 37% in a single day or decreased as much as 15% in a single day. The market has from time to time experienced significant price and volume fluctuations unrelated to the operating performance of particular companies. The market price of our common stock may fluctuate significantly due to a variety of factors, including but not limited to:

- the commercial performance of our products in the U.S.;
- the timing of our submissions to regulatory authorities and regulatory decisions and developments;
- positive or negative clinical trial results or regulatory interpretations of data collected in clinical trials conducted by us, our strategic partners, our competitors or other companies with investigational drugs targeting the same, similar or related diseases to those targeted by us;
- delays in beginning and completing pre-clinical and clinical trials for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of our products or product candidates or entry into strategic relationships on terms that are not deemed to be favorable to us;
- technological innovations, product development or additional commercial product introductions by ourselves or competitors;
- changes in applicable government regulations or regulatory requirements in the approval process;
- developments concerning proprietary rights, including patents and patent litigation matters, such as developments in the interferences declared by the USPTO, including in the near term any outcomes of ongoing interference proceedings and over the longer term the outcomes from any related appeals;
- public concern relating to the commercial value, efficacy or safety of any of our products;
- our ability to obtain funds, through the issuance of equity or equity linked securities or incurrence of debt, or other corporate transactions;
- comments by securities analysts;
- developments in litigation against us;
- changes in senior management; or
- general market conditions in our industry or in the economy as a whole.

Broad market and industry factors may seriously affect the market price of a company’s stock, including ours, regardless of actual operating performance. For example, the trading prices of biopharmaceutical companies have been highly volatile as a result of the COVID-19 pandemic, which continues to rapidly evolve. In addition, in the past, following periods of volatility in the overall market and the market price of a particular company’s securities, securities class action litigation has often been instituted against these companies. Such litigation could result in substantial costs and a diversion of our management’s attention and resources.

Our revenues and operating results could fluctuate significantly, which may adversely affect our stock price.

Our revenues and operating results may vary significantly from year-to-year and quarter-to-quarter as well as in comparison to the corresponding quarter of the preceding year. Variations my result from one or more factors, including, without limitation:

- timing of purchase orders;
- changes in coverage and reimbursement policies of health plans and other health insurers, especially in relation to those products that are currently manufactured, under development or identified for future development by us;
- re-authorizations processes that may be required for patients who initially obtained coverage by third parties, including government payors, managed care organizations and private health insurers;
- transition from temporary billing codes established by the CMS to permanent medical codes;
- timing of approval of applications filed with the FDA;

- timing of product launches and market acceptance of products launched;
- changes in the amounts spent to research, develop, acquire, license or promote new and existing products;
- results of clinical trial programs;
- serious or unexpected health or safety concerns with our product or product candidates and any resulting clinical holds;
- introduction of new products by others that render one or more of our products obsolete or noncompetitive;
- the ability to maintain selling prices and gross margins on our products;
- increases in the cost of raw materials contained within our products and product candidates;
- manufacturing and supply interruptions, including product rejections or recalls due to failure to comply with manufacturing specifications;
- timing of revenue recognition relating to our distribution agreements;
- the ability to protect our intellectual property from being acquired by other entities;
- the ability to avoid infringing the intellectual property of others;
- the continued impact of the ongoing COVID-19 pandemic; and
- the addition or loss of customers.

In addition, in one or more future periods, our results of operations may fall below the expectations of securities analysts and investors. In that event, the market price of our common stock could decline.

Provisions of our certificate of incorporation, bylaws and Delaware law might deter acquisition bids for us that might be considered favorable and prevent or frustrate any attempt to replace or remove the then-current management and board of directors.

Certain provisions of our certificate of incorporation and bylaws may make it more difficult for a third party to acquire control of us or effect a change in our board of directors and management. These provisions include:

- when the board is comprised of six or more directors, classification of our board of directors into two classes, with one class elected each year;
- directors may only be removed for cause by the affirmative vote of a majority of the voting power of all the then-outstanding shares of voting stock;
- prohibition of cumulative voting of shares in the election of directors;
- right of the board of directors to elect directors to fill a vacancy created by the expansion of the board of directors or the resignation, death, disqualification or removal of a director;
- express authorization of the board of directors to make, alter or repeal our bylaws;
- prohibition on stockholder action by written consent;
- advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at stockholder meetings;
- the ability of our board of directors to authorize the issuance of undesignated preferred stock, the terms and rights of which may be established and shares of which may be issued without stockholder approval, including rights superior to the rights of the holders of common stock; and
- a super-majority (66 2/3%) of the voting power of all of the then-outstanding shares of capital stock are required to amend, rescind, alter or repeal our bylaws and certain provisions of our certificate of incorporation.

In addition, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These and other provisions in our certificate of incorporation and our bylaws and in the Delaware General Corporation Law could make it more difficult for stockholders or potential acquirers to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors.

A significant number of shares of our common stock are issuable pursuant to outstanding stock awards, and we expect to issue additional stock awards and shares of common stock to attract and retain employees, directors and consultants. We may also issue shares of common stock to finance our operations and in connection with our strategic goals. Exercise of these awards and sales of shares will dilute the interests of existing security holders and may depress the price of our common stock.

Currently, our Amended and Restated Certificate of Incorporation authorizes the issuance of up to 99.0 million shares of common stock. As of March 31, 2020, there were approximately 78.0 million shares of common stock outstanding and outstanding awards to purchase 10.5 million shares of common stock under various incentive stock plans. Additionally, as of March 31 2020, there were approximately 1.8 million shares of common stock available for future issuance under our 2018 Equity Incentive Plan, approximately 0.5 million shares of common stock available for issuance under our Amended and Restated 2013 Employee Stock Purchase Plan, and approximately 1.6 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan.

We may issue additional shares to grant equity awards to our employees, officers, directors and consultants under our 2018 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. We may also issue additional common stock and warrants from time to time to finance our operations and in connection with strategic transactions, such as acquisitions and licensing. For example, in February 2020, we issued and sold 2,522,227 shares of common stock to Roche Finance in connection with the entry into the collaboration agreement with Roche. We will need to increase our authorized shares of common stock under our Amended and Restated Certificate of Incorporation to support these strategic goals. There can be no assurance that we will be able to obtain shareholder approval to increase the number of authorized shares.

The issuance of additional shares of common stock or warrants to purchase common stock and the perception that such issuances may occur or exercise of outstanding warrants or stock options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Future sales of our common stock in the public market could cause our share price to fall.

Sales of a substantial number of our common stock in the public market, including sales by members of our management or board of directors, or the perception that these sales might occur, could depress the market price of our common stock and could impair our ability to raise capital through the sale of additional equity or equity-related securities.

Risks Related to Our Credit Agreement and Convertible Senior Notes

Our indebtedness resulting from our credit agreement could adversely affect our financial condition or restrict our future operations.

On December 13, 2019, we entered into a loan agreement (the "Credit Agreement") with BioPharma Credit PLC, as the collateral agent and a lender ("BioPharma"), and BioPharma Credit Investments V (Master) LP, as a lender (together with BioPharma in its capacity as a lender, and each of their respective successors and assigns at any time party to the Credit Agreement, the "Lenders" and each a "Lender") that provides for a senior secured term loan facility of up to $500.0 million to be funded in two tranches: (i) a Tranche A Loan in an aggregate principal amount of $250.0 million (the "Tranche A Loan"), which was funded on December 20, 2019; and (ii) a Tranche B Loan in an aggregate principal amount of up to $250.0 million (the "Tranche B Loan", and together with the Tranche A Loan, the "Term Loans"), to be funded at our option in increments of $50.0 million, which proposed funding date shall be 75 days following the delivery of notice and in no event later than December 31, 2020. There is no assurance that the Lenders will fund the Tranche B Loan if and when requested.

All obligations under the Credit Agreement are secured pursuant to the terms of a security agreement and subject to certain exceptions, by security interests in certain collateral (collectively, the "Collateral"), which includes the following: (1) any and all U.S. intellectual property owned by, and rights to U.S. intellectual property licensed to, us relating to any pharmaceutical composition in which eteplirsen or golodirsen is indicated to be administered for use in the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 or 53 skipping, respectively, or for any other use approved by the FDA (the "Loan Products"), (2) 100% of the equity interests directly held by us in certain wholly owned domestic subsidiaries and 65% of the equity interests in certain other wholly owned domestic subsidiaries, and (3) all of our personal property, including, without limitation, cash held in all our deposit accounts. Any non-U.S. intellectual property related to the Loan Products and intellectual property unrelated in any way to the Loan Products anywhere are not part of the Collateral.

The Credit Agreement contains negative covenants that, among other things and subject to certain exceptions, restrict our ability to:

- sell or dispose of assets, including certain intellectual property;
- amend, modify or waive certain material agreements or organizational documents;
- consolidate or merge;
- incur additional indebtedness;
- incur additional liens on the Collateral;

- pay dividends or make any distribution or payment on or redeem, retire or purchase any equity interests; and
- make payments of certain subordinated indebtedness.

The Credit Agreement requires us to have consolidated liquidity of at least $100.0 million as of the last day of each month. Additionally, the Credit Agreement contains certain representations and warranties, affirmative covenants and provisions relating to events of default, which include, but are not limited to, the following: (i) nonpayment of principal, interest and other amounts; (ii) failure to comply with covenants; (iii) the occurrence of a material adverse change in (A) our ability to fulfill the payment or performance obligations under the Credit Agreement and related documents or (B) the binding nature of the Credit Agreement and related documents; (iv) the rendering of judgments or orders or the acceleration or payment default by us in respect of other indebtedness in excess of $10.0 million; and (v) certain insolvency and ERISA events. A change of control triggers a mandatory prepayment of the Term Loans, and we may not have sufficient funds or the ability to raise the funds necessary to prepay them.

Servicing our Credit Agreement and 1.50% notes due 2024 (the "Notes") requires a significant amount of cash, and we may not have sufficient cash flow to pay our debt.

In 2017, we issued $570.0 million aggregate principal amount of Notes, pursuant to that certain indenture, dated as of November 14, 2019, between us, as issuer, and U.S. Bank National Association, as trustee. Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including the Credit Agreement and the Notes, depends on our future performance, which is subject to many factors, including, economic, financial, competitive and other, beyond our control. We do not expect our business to be able to generate cash flow from operations in the foreseeable future, sufficient to service our debt and make necessary capital expenditures and we may therefore be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be onerous or highly dilutive. Our ability to refinance the Credit Agreement, which matures in 2023, and the Notes, which are non-callable and mature in 2024, will depend on the capital markets and our financial condition at such time. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations, and limit our flexibility in planning for and reacting to changes in our business.

We may not have the ability to raise the funds necessary to repurchase the Notes as required upon a fundamental change, and our future debt may contain limitations on our ability to repurchase the Notes.

Holders of the Notes will have the right to require us to repurchase their Notes for cash upon the occurrence of a fundamental change at a fundamental change repurchase price equal to 100% of the principal amount of the Notes to be repurchased, plus accrued and unpaid interest, if any. A fundamental change may also constitute an event of default or prepayment under, and result in the acceleration of the maturity of, our then-existing indebtedness. We cannot assure you that we will have sufficient financial resources, or will be able to arrange financing, to pay the fundamental change repurchase price in cash with respect to any Notes surrendered by holders for repurchase upon a fundamental change. In addition, restrictions under our then existing credit facilities or other indebtedness, if any, may not allow us to repurchase the Notes upon a fundamental change. Our failure to repurchase the Notes upon a fundamental change when required would result in an event of default with respect to the Notes which could, in turn, constitute a default under the terms of our other indebtedness, if any. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Notes.

Capped call transactions entered into in connection with the Notes may impact the value of our common stock.

In connection with the Notes, we entered into capped call transactions (the "Capped Call Transactions") with certain financial institutions. The Capped Call Transactions are expected to generally reduce the potential dilution upon conversion of the Notes into shares of our common stock.

In connection with establishing their initial hedges of the Capped Call Transactions, these financial institutions or their respective affiliates entered into various derivative transactions with respect to our common stock and/or to purchase our common stock. The financial institutions, or their respective affiliates, may modify their hedge positions by entering into or unwinding various derivatives with respect to our common stock and/or purchasing or selling our common stock or other securities of ours in secondary market transactions prior to the maturity of the Notes. This activity could also cause or avoid an increase or a decrease in the market price of our common stock or the Notes, which could affect the value of our common stock.

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

On February 14, 2020, pursuant to a Stock Purchase Agreement dated as of December 23, 2019 between Sarepta and Roche Finance, we issued and sold 2,522,227 shares (the "Roche Shares") of common stock to Roche Finance for an aggregate purchase price of approximately $400.0 million, or $158.59 per share. We relied on the exemption from the registration requirements of the Securities Act under Section 4(a)(2) thereof, for a transaction by an issuer not involving any public offering.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

None.

Item 5. Other Information.

None.

Item 6. Exhibits.

The exhibits listed on the Exhibit Index immediately preceding such exhibits, which is incorporated herein by reference, are filed or furnished as part of this Quarterly Report on Form 10-Q.

EXHIBIT INDEX

Exhibit Number	Exhibit Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
31.1	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1**	Certification of the Company's Chief Executive Officer, Douglas S. Ingram, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2**	Certification of the Company's Executive Vice President, Chief Financial Officer and Chief Business Officer, Sandesh Mahatme, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	Inline eXtensible Business Reporting Language (XBRL) Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.					X
101.SCH	Inline XBRL Taxonomy Extension Schema Document.					X
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document.					X
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)					X

† Indicates management contract or compensatory plan, contract or arrangement.

* Identified information has been excluded from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

** The Certifications attached as Exhibits 32.1 and 32.2 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the SEC and are not to be incorporated by reference into any filings of Sarepta Therapeutics, Inc. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date of this Form 10-Q, irrespective of any general incorporation language contained in such filing.

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.

SAREPTA THERAPEUTICS, INC.
(Registrant)

Date: May 6, 2020

By: /s/ DOUGLAS S. INGRAM
Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

Date: May 6, 2020

By: /s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)

EXHIBIT 31.1

CERTIFICATION

I, Douglas S. Ingram, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

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.

May 6, 2020

/s/ DOUGLAS S. INGRAM

Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

EXHIBIT 31.2

CERTIFICATION

I, Sandesh Mahatme, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Sarepta Therapeutics, Inc., (the "Registrant");

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.

May 6, 2020

/s/ SANDESH MAHATME

Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)

EXHIBIT 32.1

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Douglas S. Ingram, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended March 31, 2020, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

May 6, 2020

/s/ DOUGLAS S. INGRAM
Douglas S. Ingram
President and Chief Executive Officer
(Principal Executive Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.

EXHIBIT 32.2

CERTIFICATION PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002
(18 U.S.C. SECTION 1350)

I, Sandesh Mahatme, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that this Quarterly Report of Sarepta Therapeutics, Inc. on Form 10-Q for the quarterly period ended March 31, 2020, fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934 and that information contained in such Quarterly Report on Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

May 6, 2020

/s/ SANDESH MAHATME
Sandesh Mahatme
Executive Vice President,
Chief Financial Officer and
Chief Business Officer
(Principal Financial and Accounting Officer)

A signed original of this written statement required by Section 906 of the Sarbanes-Oxley Act of 2002 has been provided to Sarepta Therapeutics, Inc. and will be retained by Sarepta Therapeutics, Inc. and furnished to the Securities and Exchange Commission or its staff upon request.

This certification accompanies this Quarterly Report on Form 10-Q pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 and shall not, except to the extent required by such Act, be deemed filed by Sarepta Therapeutics, Inc. for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). Such certification will not be deemed to be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, except to the extent that Sarepta Therapeutics, Inc. specifically incorporates it by reference.