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

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2017

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 Number)**
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:

Tile of Each Class	**Name of Exchange on Which Registered**
Common Stock, $0.0001 par value	**The NASDAQ Stock Market LLC (The NASDAQ Global Select Market)**

Securities registered pursuant to Section 12(g) of the Act:
None

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

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

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

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

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§ 229.405 of this chapter) is not contained herein, and will not be contained, to the best of the registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ☐

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	☐ (Do not check if a smaller reporting company)	Smaller reporting company	☐
Emerging growth company	☐		

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2017 was approximately $1,865,548,919.

The number of outstanding shares of the registrant's common stock as of the close of business on February 23, 2018 was 64,978,369.

DOCUMENTS INCORPORATED BY REFERENCE

The registrant has incorporated by reference into Part III of this Annual Report on Form 10-K, portions of its definitive Proxy Statement for its 2018 annual meeting to be filed with the Commission no later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K.

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Forward-Looking Information

This Annual Report on Form 10-K, including the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section in Item 7, and other materials accompanying this Annual Report on Form 10-K contain forward-looking statements or incorporate by reference forward-looking statements. Statements that are not purely historical are forward-looking statements. Forward-looking statements 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 address expectations, projections of future results of operations or financial condition, or other "forward-looking" information. 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:

- *our belief that our proprietary technology platforms can be used to develop novel pharmaceutical products to treat a broad range of diseases and address key currently unmet medical needs and our intention to;*
- *our intent to leverage our technology platforms, organizational capabilities and resources to become a leading developer and marketer of precision genetic medicines, including the treatment of rare, neuromuscular and other diseases, with a diversified portfolio of product candidates;*
- *our intent to continue engaging in certain activities to achieve our business strategy, including executing on our strategic initiatives designed to achieve a successful commercialization of EXONDYS 51® (eteplirsen) Injection ("EXONDYS 51"), enhancing access to EXONDYS 51, expanding the global footprint of eteplirsen, advancing the development of our technology platforms, including phosphorodiamidate morpholino oligomer ("PMO"), peptide-conjugated PMO ("PPMO") and gene therapy, and identifying product candidates to target additional therapeutic areas, expanding our portfolio through internal research, strategic partnerships, collaborations and other potential opportunities; and ensuring we have the appropriate capitalization;*
- *our belief that Duchenne muscular dystrophy ("DMD") represents a significant market opportunity;*
- *our expectations regarding the continued growth of our business operations due, in part, to the commercialization of EXONDYS 51;*
- *our pipeline, technologies and next-generation approaches and their respective potential benefits, including our PMO based compounds' potential to be designed to create more, less, or none of certain proteins, or produce analogues of endogenous proteins; the potential of our PPMO to be tailored to reach other organs beyond muscle; PMOplus®'s potentially having broad therapeutic applications; PMO-X®'s potential to provide enhanced in vivo potency and efficacy, as well as greater flexibility in the modulation of selective tissue targeting and cellular delivery; the potential of micro-dystrophin and GALGT2 to treat all or nearly all DMD patients regardless of mutation; and CRSPR/Cas9's potential to be used to fix stop codon mutations in the dystrophin gene so that dystrophin can be translated to a function protein.*
- *our belief that the novel characteristics intrinsic to our new PMO-based platforms significantly reduce the potential for off-target effects and will allow for the development of drug candidates with favorable safety and efficacy characteristics;*
- *our belief that golodirsen and casimersen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping;*
- *our key milestones and inflection points for 2018, including a meeting with the United States Food and Drug Administration ("FDA") on golodirsen in the first quarter, the Committee for Medicinal Products for Human Use ("CHMP") decision for eteplirsen in mid-2018 and submitting an investigational new drug ("IND") for SRP-5053 and other PPMOs in the second half of 2018;*
- *the timely completion and satisfactory outcome of our post-marketing requirements and commitments, including verification of a clinical benefit for EXONDYS 51 in confirmatory trials;*

- *our ability to successfully expand the global footprint of eteplirsen in jurisdictions in which we have yet to obtain or do not have any near term ability or plans to obtain a full regulatory approval, including through obtaining an approval from the European Medicines Agency in the EU ("EMA"), establishing compliant and successful managed access programs ("MAP"), expanding our MAPs to include more countries over time, entering into any additional distribution, service and other contracts and building out the commercial, medical and other company infrastructure necessary to support the launch and support the distribution of*

  eteplirsen in jurisdictions outside of the U.S.;

- *the potential acceptance of EXONDYS 51, and our product candidates if they receive regulatory approval, in the marketplace and the accuracy of our projections regarding the market size in each of the jurisdictions that we target;*
- *our ability to further secure long term supply of EXONDYS 51 and our product candidates, including our PPMO, to satisfy our planned commercial, MAP, named-patient program and clinical needs;*
- *our belief that our current network of manufacturing partners is able to produce raw materials and active pharmaceutical Ingredients ("APIs") in the quantities that we require, and are capable of continuing to expand capacity as needed;*
- *our expectations regarding our ability to successfully conduct or accelerate research, development, pre-clinical, clinical and post-approval trials, and our expectations regarding the timing, design and results of such trials, including the potential consistency of data produced by these trials with prior results, as well as any new data and analyses relating to the safety profile and potential clinical benefits of EXONDYS 51 and our product candidates, including golodirsen, casimersen, SRP-5051 and gene therapy;*
- *the impact of regulations and regulatory decisions by the FDA 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 and our product candidates and our ability to compete against such products;*
- *our expectation that private insurers will continue to consider the efficacy, cost-effectiveness and safety of EXONDYS 51, in determining whether to approve reimbursement for EXONDYS 51 and at what levels;*
-
- *our ability to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications and our ability to selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements;*
- *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 ability to invalidate some or all of the claims of patents issued to competitors and pending patent applications if issued to competitors, and the potential impact of those claims on the potential commercialization and continued commercialization, where authorized, of EXONDYS 51 and the potential commercialization of our product candidates, including golodirsen, casimersen and SRP-5051;*
- *our ability to operate our business without infringing the intellectual property rights of others;*
- *our intention to expand our insurance coverage to include the sale of commercial products in connection with the FDA's approval of EXONDYS 51;*
- *our belief that our current facilities in Cambridge, Massachusetts and Andover, Massachusetts are suitable and will provide sufficient capacity to meet the projected needs of our business for the next 12 months;*
- *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 ability to raise additional funds to support our business plans and strategies, including business development, and the impact of our amended and restated credit and security agreement with MidCap Financial Trust, a Delaware statutory trust ("MidCap"), as administrative agent and new revolving credit and security agreement with MidCap, on our financial condition and future operations;*
- *our expectations relating to potential funding from government and other sources for the development of some of our product candidates;*
-
- *our ability to attract and retain key employees needed to execute our business plans and strategies and our expectations regarding our ability to manage the impact of any loss of key employees;*
- *our ability to comply with applicable environmental laws and regulations;*
- *the impact of the potential achievement of performance conditions and milestones relating to our stock awards; 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 Annual Report on Form 10-K after the date of this report, except as required by law. We caution readers not to place undue reliance on forward-looking statements. Our actual results could differ materially from those discussed in this Annual Report on Form 10-K. The forward-looking statements contained in this Annual Report on Form 10-K, and other written and oral forward-looking statements made by us from time to time, are subject to 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 Annual Report on Form 10-K.

PART I

Item 1. Business.

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 medicine approaches for the treatment of rare neuromuscular diseases.

Applying our proprietary, highly-differentiated and innovative RNA-targeted platform technologies, we are able to develop candidate therapies for a broad range of diseases and disorders.

Our first commercial product in the U.S., EXONDYS 51, was granted accelerated approval by the 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.

A summary description of our product and main product candidates is as follows:

- *EXONDYS 51*, our first product, uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS51 is designed to bind to exon 51 of dystrophin pre- messenger RNA ("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.
- *Golodirsen*, one of our main product candidates, uses our PMO chemistry and exon-skipping technology to skip exon 53 of the DMD gene. Golodirsen is designed to bind to exon 53 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 53 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.
- *Casimersen*, one of our main product candidates, 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. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.
- *SRP-5051*, one of our main product candidates, 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 addition to SRP-5051, our 2018 plans currently include IND-enabling pre-clinical work on 5 additional PPMOs.

In addition to advancing our exon-skipping product candidates for DMD, we are working with several strategic partners under various agreements to research and develop multiple treatment approaches to DMD. These strategic partners include:

- Nationwide Children's Hospital, with whom we are collaborating on the advancement of (1) their micro-dystrophin gene therapy program under a research and exclusive license option agreement and (2) their Galgt2 gene therapy program under an exclusive license agreement;
- Genethon, with whom we are collaborating on the advancement of their micro-dystrophin gene therapy program under a sponsored research and exclusive license option agreement;
- Duke University, with whom we are collaborating on the advancement of gene editing CRISPR/Cas9 technology for muscular dystrophy under a sponsored research and exclusive license option agreement that grants us rights to certain of Duke University's intellectual property for CRISPR/Cas9; and

- Summit (Oxford) Ltd. ("Summit"), with whom we are collaborating under an exclusive License and Collaboration Agreement that grants us exclusive rights to Summit's utrophin modulator pipeline, including ezutromid, in Europe, Turkey and the Commonwealth of Independent States and an option to acquire rights in Latin America.

Objectives and Business Strategy

We believe that our proprietary technology platforms and collaborations can be used to develop novel pharmaceutical products to treat a broad range of diseases and address key currently-unmet medical needs. We intend to leverage our technology platforms, organizational capabilities, collaborations and resources to become a leading developer and marketer of precision genetic medicines, including the treatment of rare, neuromuscular and other diseases, with a diversified portfolio of product candidates. In pursuit of this objective, we intend to continue engaging in the following activities:

- executing on strategic initiatives designed to achieve a successful commercialization of and access to EXONDYS 51 in the U.S.;
- expanding the global footprint of eteplirsen by pursuing regulatory approval or establishing early access programs in jurisdictions where regulatory approval has not yet been obtained;
- advancing the development of exon-skipping drug and gene therapy candidates targeting DMD;
- advancing the research and development of our technology platforms, including PMO, PPMO and gene therapy, and identifying product candidates to target additional therapeutic areas;
- expanding our portfolio through internal research, strategic partnerships, collaborations and other potential opportunities; and
- ensuring we have the appropriate capitalization to fund our business objectives and strategies, including by raising additional capital through licensing, collaborations and offerings of Company equity and / or debt.

Our Proprietary Platform Technologies

We have developed the following platform technologies, which can potentially be applied to various disease areas.

PMO. The original PMO structure and variations of this structure that are so-called PMO-based (collectively "PMO-based") are central to our proprietary chemistry platform. PMO-based therapeutics have been safely dosed in over 400 patients. PMO-based compounds are synthetic compounds that bind to complementary sequences of RNA by standard Watson-Crick nucleobase pairing. The two key structural differences between PMO-based compounds and naturally occurring RNA are that the PMO nucleobases are bound to synthetic morpholino rings instead of ribose rings, and the morpholino rings are linked by phosphorodiamidate groups instead of phosphodiester groups. Replacement of the negatively charged phosphodiester in RNA with the uncharged phosphorodiamidate group in PMO eliminates linkage ionization at physiological pH. Due to these modifications, PMO-based compounds are resistant to degradation by plasma and intracellular enzymes. Unlike the RNA-targeted technologies such as siRNAs and DNA gapmers, PMO-based compounds operate by steric blockade rather than by cellular enzymatic degradation to achieve their biological effects. Thus, PMOs use a fundamentally different mechanism from other RNA-targeted technologies.

PMO technologies can be used to selectively up-regulate or down-regulate the production of a target protein through pre-mRNA splice alteration. This mechanism can be used to correct disease-causing genetic errors by inducing the targeted expression of novel proteins. Thus, PMO-based compounds have the potential to be designed to create more, less, or none of certain proteins, or produce analogues of endogenous proteins.

The safety of therapeutic agents is paramount. We believe that our PMO-based compounds significantly reduce the potential for off-target effects specifically because of their demonstrated inactivity with key molecular mechanisms that are known to be toxicologically active when stimulated. Additionally, consistent with our research and development to date, we believe that PMO-based compounds do not exhibit coagulation and immune stimulatory effects, do not stimulate toll-like receptors or receptors of the RIG-I-like receptor family, and do not sequester metal ions away from the catalytic centers of polymerases.

In addition to our original PMO technology, we have also developed three new PMO-based chemistry platforms. We believe that the novel characteristics intrinsic to these new platforms will allow for the development of drug candidates with favorable safety and efficacy characteristics.

PPMO. The first of these novel chemistries is based on cell-penetrating PPMOs. This technology features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced delivery into the cell. In pre-clinical research, our proprietary class of PPMO compounds demonstrated an increase in dystrophin production and a more durable response compared to PMO. In addition, PPMO treatment in non-human primates is well tolerated and results in high levels of exon-skipping in skeletal, cardiac and smooth muscle tissues. Preclinical trials also indicate that PPMOs may require less frequent dosing than PMOs, and that PPMOs could potentially be tailored to reach other organs beyond muscle.

PMOplus®. The second of these chemistries, PMO*plus*®, features the selective introduction of positive charges to the PMO backbone. We believe that PMO*plus*® has potentially broad therapeutic applications.

PMO-X®. The third of these chemistries, PMO-X®, incorporates novel and proprietary chemical modifications to the PMO backbone linkages. We believe PMO-X® may provide enhanced *in vivo* potency and efficacy, as well as greater flexibility in the modulation of selective tissue targeting and cellular delivery.

Emerging Technologies – Gene Therapy and Gene Editing

As further described under *"Strategic Alliances"* below, we collaborate with different partners to explore a gene therapy approach to DMD. The programs in collaboration with Nationwide Children's Hospital and Genethon look to express a smaller but still functional version of dystrophin ("micro-dystrophin"). Micro-dystrophin is used because normal-sized dystrophin is too large to fit in an adeno-associated virus (AAV). An additional program, also in collaboration with Nationwide Children's Hospital, aims to express the enzyme GALGT2 from an AAV vector. We believe that GALGT2 modifies the dystrophin associated protein complex (DAPC) and up-regulates utrophin (a protein significantly homologous to dystrophin) to protect muscle from damage in the absence of dystrophin. The micro-dystrophin and GALGT2 technologies have the potential to treat all or nearly all DMD patients regardless of mutation.

We are also exploring, in collaboration with Duke University, the gene-editing technology CRSPR/Cas9 that aims to restore dystrophin expression by removing or "excising" exons directly from the dystrophin gene to correct out-of-frame mutations. CRSPR/Cas9 technology can also potentially be used to fix stop codon mutations in the dystrophin gene so that dystrophin can be translated to a function protein.

DMD Focus

We primarily focus on rapidly advancing the development of our potentially disease-modifying pipeline of exon-skipping drug candidates targeting DMD. DMD is a rare x-linked recessive genetic disorder affecting children (primarily males) that is characterized by progressive muscle deterioration and weakness. It is the most common type of muscular dystrophy. DMD is caused by an absence of dystrophin, a protein that protects muscle cells. The absence of dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and fibrotic replacement. In the absence of dystrophin protein, affected individuals generally experience the following symptoms, although disease severity and life expectancy vary:

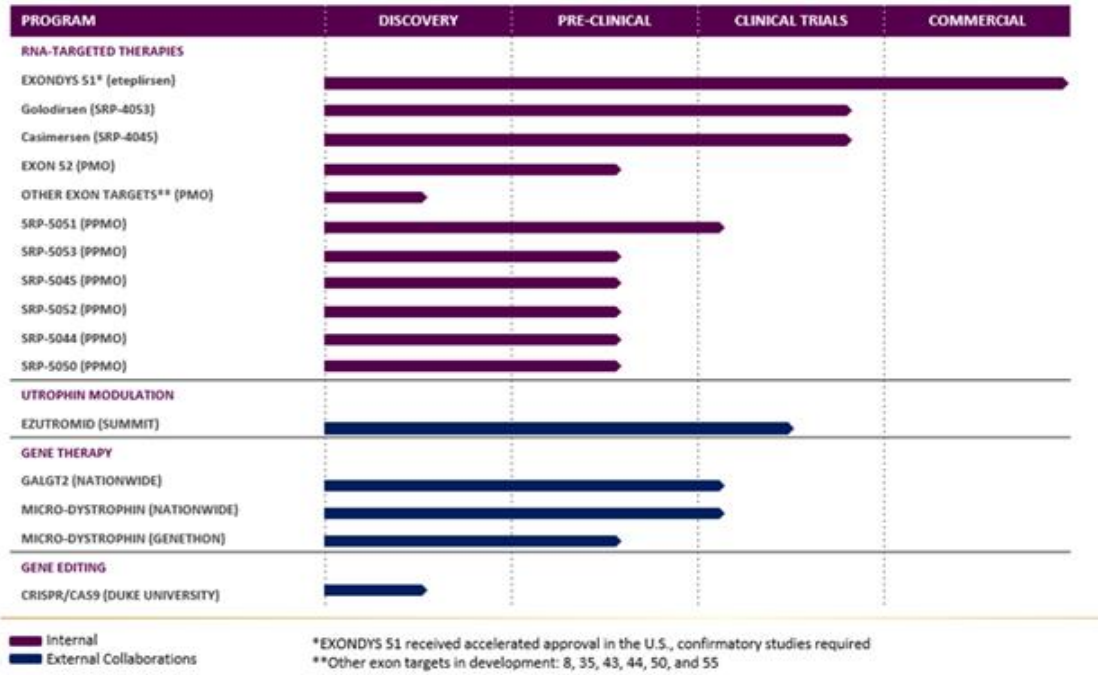
- muscle damage characterized by inflammation, fibrosis and loss of myofibers beginning at an early age;
- muscle weakness and progressive loss of muscle function beginning in the first few years of life;
- decline of ambulation and respiratory function after the age of seven;
- total loss of ambulation in the pre-teenage or early teenage years;
- progressive loss of upper extremity function during mid- to late-teens; and
- respiratory and/or cardiac failure, resulting in death before the age of 30.

EXONDYS 51: Our First Commercial Product

EXONDYS 51, our first commercial product, approved by the FDA on September 19, 2016, 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 targets the most frequent series of mutations that cause DMD. Approximately 13% of DMD patients are amenable to exon 51 skipping. We are in the process of conducting, starting or planning various EXONDYS 51 clinical trials, including studies that are required to comply with regulatory new drug application ("NDA") and/or marketing authorization application ("MAA") filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements/commitments to verify and describe clinical benefit of EXONDYS 51.

Our Pipeline: A Comprehensive Approach to DMD

In addition to EXONDYS 51, our DMD pipeline includes other product candidates, which are at various stages of development. The chart below summarizes the status of our more advanced programs as of the date of this report, including those with our strategic partners:



Golodirsen (SRP-4053). We are enrolling and dosing patients in ESSENCE (Study 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. Golodirsen, an exon 53-skipping product candidate, is currently in the clinic as part of a Phase 1/2 study. Part I has been completed, and Part II, an open-label portion of this study, is ongoing (Study 4053-101). In September 2017, we announced positive results of an analysis that included biopsies of the bicep muscle at baseline and on-treatment at the Part II, Week 48 time point. The study results demonstrated statistical significance on all primary and secondary biological endpoints. Golodirsen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping. We have recently announced that we are targeting a meeting with the FDA in the first quarter of 2018 to discuss golodirsen.

Casimersen (SRP-4045). We are enrolling and dosing patients in ESSENCE, further described above. Casimersen is an exon 45-skipping product candidate. Pursuant to an ongoing Sarepta-sponsored Phase 1/2 clinical trial studying casimersen (Study 4045-101), we have completed a dose titration portion (Phase 1) and are currently conducting the open-label portion of the study (Phase 2). Casimersen will potentially address one of the most prevalent sets of mutations in DMD that are amenable to exon-skipping.

SRP-5051. In the fourth quarter of 2017, we received clearance from the FDA and commenced a first-in-human, single ascending dose, study for our PPMO for the treatment of DMD in patients who are amenable to exon 51 skipping (SRP-5051). We expect to have data regarding safety and future dosing for SRP-5051 in the second half of 2018. We also plan to submit an IND for our next PPMO candidate, SRP-5053, in the second half of 2018.

Micro-Dystrophin. In the fourth quarter of 2017, the IND application for the micro-dystrophin gene therapy program, in collaboration with Nationwide Children's Hospital, was cleared by the FDA, and a Phase 1/2a clinical trial in individuals with DMD was initiated.

GALGT2. In the fourth quarter of 2017, the IND application for the GALGT2 gene therapy program, in collaboration with Nationwide Children's Hospital, was cleared by the FDA, and a Phase 1/2a clinical trial in individuals with DMD was initiated.

Our DMD pipeline includes additional product candidates, which are at various stages of development. Our pipeline reflects our multifaceted approach to DMD and our aspiration to apply our expertise in precision genetic medicine to address a variety of neuromuscular conditions.

Manufacturing, Supply and Distribution

We have developed proprietary state-of-the-art manufacturing techniques that allow synthesis and purification of our product and product candidates to support clinical development as well as commercialization. Our current main focus in manufacturing is to continue scaling up production of our PMO-based products and optimizing manufacturing for PPMO. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. In 2017, we opened a facility in Andover, Massachusetts, which we believe should significantly enhance our research and development manufacturing capabilities. However, we currently do not have internal manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

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 APIs and finished goods for our product candidates. All of our CMO partners have extensive technical expertise, Good Manufacturing Practices ("GMP") experience and experience manufacturing our specific technology. For our commercial DMD program, we have commenced work with our existing manufacturers to increase product capacity from mid-scale to large-scale.

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 product, based on our diligence to date, we believe our current network of manufacturing partners are able to fulfill these requirements, and are capable of continuing to expand 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.

Manufacturers and suppliers of 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.

EXONDYS 51 is 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 EXONDYS 51 to hospitals and hospital outpatient clinics. With respect to the pre-commercial distribution of eteplirsen to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute eteplirsen in certain countries on a named patient basis and through our ex-U.S. MAP. We plan to continue building out our network for commercial distribution in jurisdictions in which eteplirsen is approved.

Material Agreements

We believe that our RNA-targeted technologies could be broadly applicable for the potential development of pharmaceutical products in many therapeutic areas. To further exploit our core technologies, we have and may continue to enter into research, development or commercialization alliances with universities, hospitals, independent research centers, non-profit organizations, pharmaceutical and biotechnology companies and other entities for specific molecular targets or selected disease indications. We may also selectively pursue opportunities to access certain intellectual property rights that complement our internal portfolio through license agreements or other arrangements.

University of Western Australia

In April 2013, we entered into an agreement with University of Western Australia ("UWA") under which an existing exclusive license agreement between the two parties was amended and restated (the "Amended and Restated UWA License Agreement"). The Amended and Restated UWA License Agreement grants us specific rights to the treatment of DMD by inducing the skipping of certain exons. EXONDYS 51, golodirsen and casimersen fall under the scope of the license agreement. Under the Amended and Restated UWA License Agreement, we may be required to make payments of up to $6.0 million in aggregate to UWA based on the successful achievement of certain development and regulatory milestones relating to EXONDYS 51 and up to five additional product candidates. As of the date of this report, a portion of the $6.0 million development and regulatory milestone payments has been made. We may also be obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, we may be required to pay a low-single-digit percentage royalty on net sales of products covered by issued patents licensed from UWA during the term of the Amended and Restated UWA License Agreement. However, we have the option to purchase future royalties up-front. Under this option, prior to the First Amendment (defined below), we could be required to make a one-time royalty payment of $30.0 million to UWA.

In June 2016, we entered into the first amendment to the Amended and Restated UWA License Agreement (the "First Amendment") with UWA. Under the First Amendment, we were obligated to make an up-front payment of $7.0 million to UWA upon execution of the First Amendment. Under the terms of the First Amendment, UWA has waived rights to certain royalties and amended the timing of certain other royalty payments under the Amended and Restated UWA License Agreement, including lowering the one-time royalty payment that is due by us upon exercise of the option to purchase future royalties up-front. Upon exercise of the option to purchase future royalties up-front, we will be obligated to make a $23.0 million payment to UWA. Additionally, we would still be obligated to make up to $20.0 million in payments to UWA upon achievement of certain sales milestones.

Currently, the latest date on which an issued patent covered by our agreement with UWA expires is November 2030 (excluding any patent term extension, supplemental protection certificate or pediatric extensions that may be available); however, patents granted from pending patent applications could result in a later expiration date.

Summit (Oxford) Ltd.

On October 3, 2016, we entered into the exclusive Collaboration and License Agreement (the "Collaboration Agreement") with Summit which grants us the exclusive right to commercialize products in Summit's utrophin modulator pipeline in the E.U., Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States (the "Licensed Territory").

Under the terms of the Collaboration Agreement, we made an up-front payment of $40.0 million to Summit, with the potential for additional payments of up to $192.0 million based on achievement of certain development and regulatory milestones for ezutromid. For each of Summit's future generation small molecule utrophin modulators, we may be required to make up to $290.0 million in development and regulatory milestone payments. Additionally, on a product-by-product basis, we may be required to make up to $330.0 million in sales milestone payments.

The Collaboration Agreement also grants us an option to expand the Licensed Territory. If we exercise this option, then we will be liable for a one-time $10.0 million option fee as well as up to $7.0 million in regulatory milestone payments. For each of Summit's lead product candidate, ezutromid, for the treatment of DMD, and its second generation and future generation small molecule utrophin modulators (collectively, the "Licensed Product"), we may be liable for up to $82.5 million in sales milestone payments.

Additionally, we may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory.

Under the Collaboration Agreement, Summit was solely responsible for all research and development costs for the Licensed Products until December 31, 2017. Currently, Summit is responsible for 55.0% of the budgeted research and development costs related to the Licensed Products in the Licensed Territory, and we are be responsible for 45.0% of such costs. Any costs in excess of 110.0% of the budgeted amount will be borne by the party that incurred such costs. Summit is also obligated to spend a specified minimum amount on the research and development of certain Licensed Products prior to the end of 2019.

Manufacture and Supply of Licensed Products

Under the Collaboration Agreement, Summit has agreed to use commercially reasonable efforts to supply to us API, finished drug product and placebo so that we may conduct research, development and commercialization activities for the Licensed Products. We will also have the right to establish back up and second source suppliers under certain circumstances.

Commercialization

Under the Collaboration Agreement, we will be solely responsible for all commercialization activities and associated costs relating to the Licensed Products in the Licensed Territory. We have agreed to use commercially reasonable efforts to commercialize the Licensed Products in specified countries within the Licensed Territory and, if the exclusive option to expand the Licensed Territory to include specified countries in South and Central America (the "Option Territory") is exercised, to use commercially reasonable efforts to commercialize the Licensed Products in certain specified countries within the Option Territory.

BioMarin

License Agreement

On July 17, 2017, Sarepta Therapeutics, Inc. and Sarepta International C.V. (collectively, "Sarepta") and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV (collectively, "BioMarin") executed a License Agreement (the "License Agreement"), pursuant to which BioMarin granted Sarepta a royalty-bearing, worldwide license under patent rights ("Licensed Patents") and know-how ("Licensed Know-How") controlled by BioMarin with respect to BioMarin's DMD program, which are potentially necessary or useful for the treatment of DMD, to practice and exploit the Licensed Patents and Licensed Know-How in all fields of use and for all purposes, including to develop and commercialize antisense oligonucleotide products that target one or more exons of the dystrophin gene to induce exon skipping, including eteplirsen (collectively, the "Products").

The license granted by BioMarin to Sarepta under the terms of the License Agreement is exclusive, even as to BioMarin, with respect to the Licensed Patents, and is non-exclusive with respect to Licensed Know-How. Under the License Agreement, BioMarin has the option to convert the exclusive license under the Licensed Patents into a co-exclusive license (co-exclusive with BioMarin) ("BioMarin Co-Exclusive Option").

Under the terms of the License Agreement, Sarepta is required to pay BioMarin an upfront payment of $15 million, and BioMarin will be eligible to receive up to $20 million from Sarepta per dystrophin gene exon (other than exon 51) targeted by one or more Products in specified regulatory milestones, as well as an additional $10 million milestone, payable following the regulatory approval of eteplirsen by the EMA. BioMarin will also be eligible to receive $15 million from Sarepta upon the achievement of $650 million in sales, as well as royalties segmented by specified geographic markets, in some jurisdictions dependent on the existence of a patent, ranging from four (4) to eight (8) percentages of net sales on a product-by-product and country-by-country basis.

Milestones and royalties are payable with respect to eteplirsen (an exon 51 skipping Product), casimersen (an exon 45 skipping Product), golodirsen (an exon 53 skipping Product) and other Products. For eteplirsen, casimersen and golodirsen, the royalty term will expire upon the end of 2023 in the U.S., upon September 30, 2024 in the EU and no later than September 30, 2024 in other countries provided certain conditions are met. For Products other than exon 45 skipping Products, exon 51 skipping Products and exon 53 skipping Products, the royalty term will end on a country-by country basis upon expiration of granted Licensed Patents covering the applicable Product. The royalties for all Products are subject to reduction upon BioMarin's exercise of the BioMarin Co-Exclusive Option. All royalties are subject to further potential reductions, including for generic competition and, under specified conditions, for a specified portion of payments that Sarepta may become required to pay under third-party license agreements, subject to a maximum royalty reduction.

Unless earlier terminated, the License Agreement will expire upon the expiration of the last-to-expire royalty term. Either party may terminate the License Agreement in the event of the other party's uncured material breach. BioMarin may also terminate the License Agreement on a Licensed Patent-by-Licensed Patent basis under specified circumstances relating to patent challenges by Sarepta.

Settlement Agreement

On July 17, 2017, Sarepta and The University of Western Australia on the one hand, and the BioMarin Parties and Academisch Ziekenhuis Leiden ("AZL") on the other hand (collectively, the "Settlement Parties"), executed a Settlement Agreement pursuant to which all legal actions in the U.S. and certain legal actions in Europe (the "Actions") would be stopped or withdrawn as between the Settlement Parties. Specifically, the terms of the Settlement Agreement require that existing efforts pursuing ongoing litigation and opposition proceedings would be stopped as between the Settlement Parties, and the Settlement Parties would cooperate to withdraw the Actions before the European Patent Office (except for actions involving third parties), the U.S. Patent and Trademark Office, the U.S. Court of Appeals for the Federal Circuit and the High Court of Justice of England and Wales, except for the cross-appeal of the Interlocutory Decision of the Opposition Division dated April 15, 2013 of the European Patent Office of EP 1619249B1 ("EP '249 Appeal") in which Sarepta will withdraw its appeal and BioMarin/AZL will continue with its appeal with Sarepta having oversight of the continued appeal by BioMarin/AZL.

Additionally, under the terms of the Settlement Agreement, the Settlement Parties agree to release each other and the customers, end-users, agents, suppliers, distributors, resellers, contractors, consultants, services and partners of Sarepta or BioMarin (as applicable) from claims and damages related to (i) the patent rights controlled by the releasing party that are involved in the Actions, (ii) with respect to Sarepta and UWA, its patent rights related to the patent rights involved in the Actions, and (iii) with respect to BioMarin and AZL, all of the Licensed Patents and Licensed Know-How.

Under the terms of the Settlement Agreement, Sarepta made an upfront payment of $20 million to BioMarin.

Strategic Alliances

In connection with our multi-front battle against DMD, we have entered into a number of partnering opportunities. We believe these collaborations, taken along with our own programs, represent the most comprehensive approach to treating DMD.

Duke University

In October 2017, we entered into a sponsored research and exclusive license option agreement with Duke University, granting us an option to an exclusive license to intellectual property and technology related to certain CRISPR/Cas9 technology developed in the laboratory of Charles A. Gersbach, Ph.D. The underlying premise of Dr. Gersbach's approach is to restore dystrophin expression by removing or "excising" exons from the dystrophin gene. This includes a strategy to excise exons potentially enabling treatment for a majority of the DMD patient population.

Genethon

In May 2017, we entered into a gene therapy exclusive license option agreement with Genethon to jointly develop micro-dystrophin gene therapy products for the treatment of DMD. Under the terms of the collaboration, Genethon is responsible for the early development work, and we have the option to co-develop Genethon's micro-dystrophin program, which includes exclusive U.S. commercial rights.

Nationwide Children's Hospital

In December 2015, we entered into an exclusive license agreement with Nationwide Children's Hospital to acquire exclusive rights to their Galgt2 gene therapy program. This program explores the potential surrogate gene therapy approach to DMD. Under this approach, the gene therapy looks to induce genes that make proteins that can perform a similar function as dystrophin, with the goal of producing a muscle cell that can function normally even when dystrophin is absent. This approach has the potential to be used broadly in several muscular dystrophies, regardless of their mutation. In the fourth quarter of 2017, the IND application for the Galgt2 gene therapy program was cleared by the FDA, and a Phase 1/2a clinical trial in individuals with DMD was initiated.

In addition, in December 2016, we entered into a research and exclusive license option agreement with Nationwide Children's Hospital for their micro-dystrophin gene therapy program. In the fourth quarter of 2017, the 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 at Nationwide Children's Hospital. Parent Project Muscular Dystrophy has committed $2.2 million to the trial, with support from additional Duchenne foundations and families. We have committed to the trial through a research agreement with Nationwide Children's Hospital, and we have an exclusive option to exclusively license the program.

Charley's Fund Agreement

In October 2007, Charley's Fund, Inc. ("Charley's Fund"), a nonprofit organization that funds drug development and discovery initiatives specific to DMD, awarded us a research grant of approximately $2.5 million and, in May 2009, the grant authorization was increased to a total of $5.0 million. Pursuant to the related sponsored research agreement, the grant was provided to support the development of product candidates related to exon 50 skipping using our proprietary exon-skipping technologies. As of December 31, 2017, Charley's Fund had made payments of approximately $3.4 million to us. Revenue associated with this research and development arrangement is recognized based on the proportional performance method. To date, we have recognized approximately $0.1 million as revenue. We have deferred $3.3 million of previous receipts, which are anticipated to be recognized as revenue upon resolution of outstanding performance obligations.

Previously, we noted unexpected toxicology findings in the kidney as part of our series of preclinical trials for AVI-5038, our PMO-based candidate designed for the treatment of individuals with DMD who have an error in the gene coding for dystrophin that can be treated by skipping exon 50. We have conducted additional preclinical trials and have not alleviated the toxicity problem. Pursuant to the terms of our agreement with Charley's Fund, the receipt of additional funds is tied to the satisfaction of certain clinical milestones. Because of the toxicity issues with AVI-5038, satisfaction of the additional milestones under the agreement is unlikely and we do not expect to receive any additional funds from Charley's Fund.

Patents and Proprietary Rights

Our success depends in part upon our ability to protect our core technologies and intellectual property. To accomplish this, we rely on a combination of intellectual property rights, including patents, trade secrets, copyrights and trademarks, as well as regulatory exclusivity and contractual protections.

Our patents and patent applications are directed to our product candidates as well as to our PMO-based technologies platform. We seek patent protection for certain of our product candidates and proprietary technologies by filing patent applications in the U.S. and other countries as appropriate.

Our product candidates and our technologies are primarily protected by composition of matter and use patents and patent applications. Currently, our exon skipping clinical product candidates for DMD include EXONDYS 51 (eteplirsen), casimersen and golodirsen.

We own patents and have exclusively licensed patents from UWA that provide primary patent protection for eteplirsen, casimersen and golodirsen as follows:

Eteplirsen

Patent Number	Country/Region*	Patent Type	Expiration Date**	Owner
U.S. 9,416,361	United States	Composition of Matter	May 4, 2021	Sarepta
U.S. 7,960,541[1]	United States	Composition of Matter	June 28, 2025	UWA
U.S. 8,486,907[2]	United States	Methods of Use	June 28, 2025	UWA
U.S. 9,018,368	United States	Composition of Matter	June 28, 2025	UWA
U.S. 7,807,816[1]	United States	Composition of Matter	February 23, 2026	UWA
U.S. 9,243,245	United States	Methods of Use	October 27, 2028	BioMarin/AZL
U.S. 9,506,058	United States	Methods of Use	March 14, 2034	Sarepta

Patent Number	Country/Region*	Patent Type	Expiration Date**	Owner
EP 1 619 249 B1[3]	Europe	Methods of Use	September 21, 2021	BioMarin/AZL
EP 2 284 264 B1	Europe	Composition of Matter & Methods of Use	September 21, 2021	BioMarin/AZL
EP 2 801 618 B1	Europe	Composition of Matter & Methods of Use	September 21, 2021	BioMarin/AZL
EP 1 766 010 B1	Europe	Composition of Matter & Methods of Use	June 28, 2025	UWA
EP 2 203 173 B1[4]	Europe	Methods of Use	October 27, 2028	BioMarin/AZL

Casimersen

Patent Number	Country/Region**	Patent Type	Expiration Date**	Owner
U.S. 9,416,361	United States	Composition of Matter	May 4, 2021	Sarepta
U.S. 9,447,415	United States	Composition of Matter	June 28, 2025	UWA
U.S. 8,524,880	United States	Composition of Matter & Methods of Use	April 2, 2026	UWA
U.S. 9,228,187	United States	Composition of Matter	November 12, 2030	UWA
U.S. 9,758,783	United States	Methods of Use	November 12, 2030	UWA

Golodirsen

Patent Number	Country/Region**	Patent Type	Expiration Date**	Owner
U.S. 9,416,361	United States	Composition of Matter	May 4, 2021	Sarepta
U.S. 8,455,636[5]	United States	Composition of Matter & Methods of Use	June 28, 2025	UWA
U.S. 9,024,007	United States	Composition of Matter	June 28, 2025	UWA

Patent Number	Country/Region*	Patent Type	Expiration Date**	Owner
EP 1 619 249 B1[3]	Europe	Methods of Use	September 21, 2021	BioMarin/AZL
EP 2 602 322 B1[6]	Europe	Composition of Matter & Methods of Use	September 21, 2021	BioMarin/AZL
EP 2 206 781 B1[7]	Europe	Composition of Matter & Methods of Use	June 28, 2025	UWA

* Granted patents in the United States and Europe (EP) are shown here. Additional patent protection in the U.S., Europe (EP) or other countries or regions through pending or granted foreign counterparts may be available.

****** Stated expiration dates do not account for any patent term extension, supplemental protection certificate or pediatric extensions that may be available.

[1] Previously involved in U.S. Patent Interference No. 106,008. Judgment dated September 20, 2016 ordered cancellation of all claims of U.S. Application No. 13/550,210 to BioMarin (AZL). Appeal by BioMarin (AZL) to the Court of Appeals for the Federal Circuit (Case No. 2017-1078) voluntarily dismissed July 27, 2017. Reissue of U.S. 7,807,816 (U.S. Application No. 15/349,535) filed November 11, 2016.

[2] Previously involved in U.S. Patent Interference No. 106,013. Judgment dated September 29, 2015 ordered cancellation of U.S. 8,486,907 to us (UWA). Decision dated December 29, 2015 denied our Request for Rehearing. Appeal by us (UWA) to the Court of Appeals for the Federal Circuit (Case Nos. 2016-1937, 2016-2086 (consolidated)) voluntarily dismissed July 27, 2017. Reissue of U.S. 8,486,907 (U.S. Application No. 15/655,646) filed July 20, 2017.
[3] Involved in EPO Opposition. Cross-appeal of Interlocutory Decision dated April 15, 2013 pending.
[4] Involved in EPO Opposition proceedings initiated on September 22, 2016.
[5] Previously involved in U.S. Patent Interference No. 106,007. Judgment dated April 29, 2016 ordered cancellation of (i) all claims, except claim 77, of U.S. Application No. 11/233,495 to BioMarin (AZL); and (ii) U.S. 8,455,636 to us (UWA). Appeal by BioMarin (AZL) to the Court of Appeals for the Federal Circuit (Case No. 2016-2262) voluntarily dismissed July 27, 2017. Reissue of U.S. 8,455,636 (U.S. Application No. 15/645,842) filed July 10, 2017.
[6] Involved in Opposition proceedings initiated on November 28, 2016.
[7] Involved in Opposition proceedings initiated on August 25, 2016. EPO Opposition Division Decision dated December 19, 2017 ordered that the patent be revoked in its entirety. The Decision is open for appeal.

In addition to the foregoing patents that protect eteplirsen, casimersen and golodirsen, we either solely own or exclusively license from UWA, BioMarin or AZL patents and patent applications in the U.S. and in major foreign markets that provide additional protection for eteplirsen as well as our DMD follow-on exon-skipping candidates (e.g., casimersen and golodirsen), which cover the composition of matter, preparation and/or uses of these drug candidates. These patents, and patent applications, if granted, expire between 2025 and 2037, such expiration dates not accounting for any patent term extension, supplemental protection certificate or pediatric extensions that may be available.

We separately own patents and patent applications in the U.S. and in major foreign markets that cover our proprietary PMO-based technologies (e.g., PPMO, PMOplus®, PMO-X®). These patents, and patent applications, if granted, expire between 2024 and 2037, such expiration dates not accounting for any patent term extension, supplemental protection certificate or pediatric extensions that may be available. We are the owner of multiple federal trademark registrations in the U.S. including, but not limited to, Sarepta®, Sarepta Therapeutics®, PMO*plus*®, PMO-X® the Sarepta Therapeutics logo, EXONDYS®, and EXONDYS 51®. In addition, we have multiple pending trademark applications in the U.S. and in major foreign markets.

We do not have patents or patent applications in every jurisdiction where there is a potential commercial market for our product candidates. For each of our programs, our decision to seek patent protection in specific foreign markets, in addition to the U.S., is based on many factors, including:

- our available resources;
- the number and types of patents already filed or pending;
- the likelihood of success of the product candidate;
- the size of the commercial market;
- the presence of a potential competitor in the market; and
- whether the legal authorities in the market effectively enforce patent rights.

We continually evaluate our patent portfolio and patent strategy and believe our owned and licensed patents and patent applications provide us with a competitive advantage; however, if markets where we do not have patents or patent applications become commercially important, our business may be adversely affected.

Government Regulation

The testing, manufacturing, labeling, advertising, promotion, distribution, exportation and marketing of our products are subject to extensive regulation by governmental authorities in the U.S. and in other countries. In the U.S., the FDA, under the Federal Food, Drug and Cosmetic Act and its implementing regulations, regulates pharmaceutical products. Failure to comply with applicable U.S. requirements may subject us to administrative or judicial sanctions, such as FDA refusal to approve pending NDAs, withdrawal of approval of approved products, warning letters, untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, civil penalties and/or criminal prosecution.

U.S. Drug Approval Process

To obtain FDA approval of a product candidate, we must, among other things, submit clinical data providing substantial evidence of safety and efficacy of the product for its intended use, as well as detailed information on product composition, its manufacture and controls and proposed labeling. The testing and collection of data and the preparation of necessary applications are expensive and time-consuming. The FDA may not act quickly or favorably in reviewing these applications, and we may encounter significant difficulties or costs in our efforts to obtain FDA approvals that could delay or preclude us from marketing our products.

The steps required before a drug may be approved for marketing in the U.S. generally include the following:

- preclinical laboratory tests and animal toxicity testing;
- submission of an IND for conducting human clinical testing to the FDA, which must become effective before human clinical trials commence;
- adequate and well-controlled human clinical trials to establish the safety and efficacy of the drug product for each indication, including controlled studies or comparison of treated group from clinical trials to data from natural history data or studies;
- submission of a complete and compliant NDA containing chemistry, manufacturing and control information for the drug substance and drug product, reports of nonclinical and clinical trials, product labeling and administrative information;
- satisfactory completion of an FDA inspection of the commercial manufacturing facilities at which the drug substance and drug product are made to assess compliance with cGMP;
- satisfactory FDA audit of the clinical trial site(s) that generated the pivotal safety and efficacy data included in the NDA and also potentially the nonclinical trial site(s) in the form of pre-approval inspections; and
- FDA review and approval of the NDA.

Preclinical trials may include laboratory evaluations of the product chemistry, pharmacology, toxicity and formulation, as well as animal studies to assess the pharmacokinetics, metabolism, bio-distribution, elimination and toxicity of the product candidate. The conduct of the preclinical tests and formulation of the compounds for testing must comply with federal regulations and requirements. The results of the preclinical trials, manufacturing information, analytical data and a proposed first in human clinical trial protocol are submitted to the FDA as part of the IND, which must become effective before clinical trials may be initiated. The IND will become effective approximately 30 days after receipt by the FDA, unless the FDA raises concerns or questions about the supportive data, or the study design, particularly regarding potential safety issues with conducting the clinical trial as described in the protocol. In this situation, the trials are placed on clinical hold and the IND sponsor must resolve any outstanding FDA concerns before clinical trials can proceed.

Clinical trials involve the administration of the product candidate to healthy volunteers or patient participants under the supervision of a qualified principal investigator. Clinical trials are conducted under protocols detailing the objectives of the study, the administration of the investigational product, study procedures, parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol must be submitted to the FDA as a submission to the IND. Clinical trials must be conducted in accordance with the FDA's Good Clinical Practice ("GCP") requirements and federal and state laws and regulations protecting study subjects. Further, each clinical trial must be reviewed and approved by the Institutional Review Board ("IRB") at or servicing each institution in which the clinical trial will be conducted. The IRB will consider, among other things, rationale for conducting the trial, clinical trial design, participant informed consent, ethical factors, the safety and rights of human subjects and the possible liability of the institution. The FDA can temporarily or permanently halt a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial subjects. The IRB may also require the clinical trial at a particular site be halted, either temporarily or permanently, for failure to comply with GCP or the IRB's requirements, or may impose other conditions.

Clinical trials typically are conducted in three sequential drug development phases (Phases 1, 2 and 3) prior to approval, and a portion of these phases may overlap. A fourth post-approval phase (Phase 4) may include additional clinical trials. A general description of clinical trials conducted in each phase of development is provided below. However, the number of study subjects involved in each phase of drug development for rare diseases can be significantly less than typically expected for more common diseases with larger patient populations:

- Phase 1. Phase 1 clinical trials involve the initial introduction of the drug into human subjects. These studies are usually designed to determine the safety of single and multiple doses of the compound and determine any dose limiting toxicities or intolerance, as well as the metabolism and pharmacokinetics of the drug in humans. Phase 1 studies usually involve less than 100 subjects and are conducted in healthy adult volunteers, unless it is unethical to administer the study drug to healthy volunteers, in which case they are tested in patients.
- Phase 2. Phase 2 clinical trials are usually conducted in a limited patient population to evaluate the safety and efficacy of the drug for a specific indication to determine optimal dosage and to identify possible adverse effects and safety risks. Phase 2 studies usually involve patients with the disease under investigation and may vary in size from several dozen to several hundred.
- Phase 3. If an investigational drug is found to be potentially effective and to have an acceptable safety profile in early phase studies, larger Phase 3 clinical trials are conducted to confirm clinical efficacy, dosage and safety in the intended patient population, which may involve geographically dispersed clinical trial sites. Generally, two adequate and well-controlled Phase 3 clinical trials which establish the safety and efficacy of the drug for a specific indication are required for approval of an NDA. Phase 3 studies usually include several hundred to several thousand patients for larger, non-orphan drug indications/diseases. However, clinical trials for rare or orphan diseases generally have fewer patients due to their lower prevalence. For these orphan diseases, a company may also try to demonstrate efficacy and safety by comparing treated patients in clinical trials to untreated patients participating in placebo-controlled clinical trials or to observational natural history studies.
- Phase 4. Phase 4 trials are clinical trials conducted after the FDA has approved a product for marketing. Typically there are two forms of Phase 4 trials: those that are conducted to fulfill mandatory conditions of product approval and those that are voluntarily conducted to gain additional experience from the treatment of patients in the intended therapeutic indication. The mandatory studies are used to confirm clinical benefit in the case of drugs approved under the accelerated approval regulations or to provide additional clinical safety or efficacy data for "full" approvals. Failure to promptly conduct and complete mandatory Phase 4 clinical trials could result in withdrawal of approval for products approved under accelerated approval regulations.

A company seeking marketing approval for a new drug in the U.S. must submit the results of the preclinical and clinical trials to the FDA in the form of an NDA, together with, among other things, detailed information on the manufacture and composition of the product candidate and proposed labeling, including payment of a user fee for FDA review of the application. The user fee is waived for an application for a product intended to treat an Orphan Indication. The FDA assesses all submitted NDAs for completeness before it accepts them for filing. In some cases, the FDA may request additional information before accepting an NDA for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. Applications receive either standard or priority review. Under the current goals mandated under the Prescription Drug User Fee Act (the "PDUFA"), the FDA has ten months in which to complete its initial review of a standard NDA and respond to the applicant, and six months for a priority NDA. The FDA does not always meet its PDUFA goal dates for standard or priority NDAs. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the NDA sponsor otherwise provides additional information or clarification regarding information already provided in the submission within the last three months before the PDUFA goal date. The FDA may refer an application to an advisory committee for review, evaluation and recommendation as to whether the application should be approved. Though the FDA is not bound by such recommendations, it considers them carefully when making decisions. If the FDA's evaluations of the NDA and the clinical and manufacturing procedures and facilities are favorable, the FDA may issue an approval letter. If the FDA finds deficiencies in the NDA, it may issue a complete response letter, which defines the conditions that must be met in order to secure final approval of the NDA. If and when those conditions have been met to the FDA's satisfaction, the FDA will issue an approval letter, authorizing commercial marketing of the drug. Sponsors that receive a complete response letter may submit to the FDA information that represents a complete response to the issues identified by the FDA. Resubmissions by the NDA sponsor in response to a complete response letter trigger new review periods of varying length (typically two to six months) based on the content of the resubmission. If the FDA's evaluation of the NDA and the commercial manufacturing procedures and facilities is not favorable, the FDA may not approve the NDA.

A sponsor may also seek designation of its drug candidates under programs designed to accelerate the FDA's review and potential approval of NDAs. For instance, a sponsor may seek FDA designation of a drug candidate as a "fast track product." Fast track products are those products intended for the treatment of a serious or life-threatening disease or condition and which demonstrate the potential to address unmet medical needs for such disease or condition. If fast

track designation is obtained, the FDA may initiate early and frequent communication and begin reviewing sections of an NDA before the application is complete. This “rolling review” is available if the applicant provides, and the FDA approves, a schedule for the remaining information. Eteplirsen was granted fast track status in 2007.

The Food and Drug Administration Safety and Innovation Act (“FDASIA”) enacted and signed into law in 2012 amended the criteria for the fast track and accelerated approval pathways and, as a result, the pathways now share many common eligibility criteria. FDASIA provides both the sponsor companies and the FDA with greater flexibility and expedited regulatory mechanisms. The statute clarifies that a fast track product may be approved pursuant to an accelerated approval (Subpart – H) or under the traditional approval process. In addition, FDASIA codified the accelerated approval pathway as separate and apart from the fast track pathway, meaning that for drugs to be eligible for accelerated approval, they do not need to be designated under the fast track pathway. FDASIA reinforces the FDA’s authority to grant accelerated approval of a drug that treats a serious condition and generally provides a meaningful advantage over available therapies and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality (“IMM”) that is reasonably likely to predict an effect on IMM or other clinical benefit (i.e., an intermediate clinical endpoint). Approvals of this kind typically include requirements for appropriate post-approval Phase 4 clinical trials to confirm clinical benefit. FDASIA retains this requirement and further requires those studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit.

Additionally, FDASIA established a new, expedited regulatory mechanism referred to as breakthrough therapy designation. Breakthrough therapy designation, fast track, and accelerated approval are not mutually exclusive and are meant to serve different purposes. The breakthrough therapy designation is focused on expediting the development and review process and by itself does not create an alternate ground for product approval. A sponsor may seek FDA designation of a drug candidate as a breakthrough therapy if the drug is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and 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. The FDA issued guidance entitled “Expedited Programs for Serious Conditions—Drugs and Biologics” in May 2014.

Finally, if a drug candidate demonstrates a significant benefit over existing therapy, it may be eligible for priority review, which means it will be reviewed within a six-month timeframe from the date a complete NDA is accepted for filing.

We cannot be sure that any of our drug candidates will qualify for any of these expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review.

Holders of an approved NDA are required to:

- report serious adverse drug reactions to the FDA;
- submit annual and periodic reports summarizing product information and safety data;
- comply with requirements concerning advertising and promotional labeling;
- continue to have quality control and manufacturing procedures conform to cGMP after approval; and
- conduct any post-marketing study designated as a required condition of the NDA approval.

The FDA periodically inspects the sponsor’s records related to safety reporting and/or manufacturing; this latter effort includes assessment of compliance with cGMP. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved NDA, including withdrawal of the product from the market.

Orphan Drug Designation and Exclusivity in the U.S.

In the U.S., the FDA may grant orphan drug designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the U.S., or more than 200,000 individuals in the U.S. for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or

condition will be recovered from sales in the U.S. for that drug. An orphan drug designation must be requested before submitting an application for marketing approval. An orphan drug designation does not shorten the duration of the regulatory review and approval process. The approval of an orphan designation request does not alter the regulatory requirements and process for obtaining marketing approval. Safety and efficacy of a compound must be established through adequate and well-controlled studies. If a product which has an orphan drug designation subsequently receives FDA approval for the indication for which it has such designation, the product is generally entitled to an orphan drug exclusivity period, which means the FDA may not grant approval to any other application to market the same chemical entity for the same indication for a period of seven years, except in limited circumstances, such as where an alternative product demonstrates clinical superiority to the product with orphan exclusivity. In addition, holders of exclusivity for orphan drugs are expected to assure the availability of sufficient quantities of their orphan drugs to meet the needs of patients. Failure to do so could result in the withdrawal of orphan exclusivity for the drug. Competitors may receive approval of different drugs or biologics for the indications for which a prior approved orphan drug has exclusivity. The FDA granted orphan drug exclusivity to EXONDYS 51 through September 19, 2023, which is seven years from its approval date, September19, 2016, for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping.

Distinct from orphan drug exclusivity, the FDA may provide six months of pediatric exclusivity to a sponsor of an NDA, if the sponsor conducted a pediatric study or studies of such product. This process is applied to products developed for adult use and is initiated by the FDA as a written request for pediatric studies that applies to a sponsor's product. If the sponsor conducts qualifying studies and the studies are accepted by the FDA, then an additional six months of pediatric exclusivity will be added to previously granted exclusivity, such as orphan drug exclusivity and new chemical entity exclusivity.

Foreign Regulatory Requirements

We are pursuing regulatory approval of eteplirsen in jurisdictions outside of the U.S. On December 19, 2016, we announced that the EMA validated for centralized assessment our previously submitted MAA for eteplirsen to treat DMD amenable to exon 51 skipping. The review process is underway, and we expect to receive a response from the EMA's Committee for Medicinal Products for Human Use on our application in mid-2018. We have also initiated key activities in support of the potential launch of eteplirsen in the EU, such as building out commercial infrastructure and scaling-up manufacturing. As of the date of this report, EXONDYS 51 has not been approved for sale or marketing by any regulatory agency or authority outside of the U.S.

Thus, in addition to regulations in the U.S., our business is subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. Irrespective of whether it is an FDA approved drug or an investigational drug, approvals by the comparable regulatory authorities of foreign countries are required before we can commence clinical trials or marketing of the product in those countries. For example, in the EU, the conduct of clinical trials is governed by the currently applicable Clinical Trials Directive 2001/20/EC concerning conduct of clinical trials in the EU and the Directive 2005/28/EC laying down the principles and guidelines on GCP, a system for the approval of clinical trials that has been implemented through national legislation in the member states in the EU. Under this system, a sponsor must obtain approval from the competent national authority of an EU member state in which the clinical trial is to be conducted, or in multiple member states if the clinical trial is to be conducted in a number of countries. Furthermore, the sponsor may only start a clinical trial at a specific study site after the competent ethics committee has issued a favorable opinion. The Clinical Trials Application ("CTA") must include the supporting information prescribed by Directive 2001/20/EC and Directive 2005/28/EC, corresponding national laws of the member states, and as further detailed in the applicable guidance documents.

In April 2014, the EU adopted a new Clinical Trials Regulation (EU) No 536/2014 to replace the current Clinical Trials Directive. The new Clinical Trials Regulation has come into force, but will come into application in all EU Member States in October 2018 without the need for any national implementing legislation. The new regulation provides an overhaul of the system to ensure greater consistency in the approval of clinical trials with the highest standards of patient safety in the EU. Specifically, the new legislation seeks to simplify and streamline approval. Under the new coordinated procedure for the approval, the sponsor of a clinical trial is required to submit a single application to a reporting EU Member State. The reporting Member State will consult and coordinate with all other Member States in which the clinical trial is planned to be conducted (concerned Member States). If the application is rejected, it can be amended and resubmitted through a central EU Portal. If an approval is issued, the sponsor can start the clinical trial in all concerned Member States. However, a concerned Member State can in certain cases declare an "opt-out" from the

approval. In such a case, the clinical trial cannot be conducted in those Member State(s). The Clinical Trials Regulation also aims to streamline and simplify the rules on safety reporting for clinical trials.

In order to obtain marketing authorization for a medicinal product in the EU, applicants are required to submit an MAA to either the national Competent Authorities or the EMA for the Centralized authorization procedures, using the ICH Common Technical Document (“CTD”). Applicants are required to demonstrate the quality, safety and efficacy of the medicinal product in the application for marketing authorization. This includes the requirement to conduct human clinical trials to generate the necessary clinical data. Submission of data in compliance with an agreed Pediatric Investigation Plan (“PIP”) is essential for the validation or acceptance of an MAA for review. Medicinal products are authorized in the EU through one of several different procedures, either by the national competent authorities of the EU Member States (through the decentralized, mutual recognition, or national procedures), or through the centralized authorization procedure administered by the EMA. Regulation (EC) No 726/2004 of the European Parliament and of the Council lays down the centralized procedure for the authorization of medicinal products, for which there is a single application, a single evaluation and a single approval allowing direct access to the single market of the EU. Approval via the Centralized Procedure is a two-step process whereby the CHMP first adopts an “Opinion” recommending grant of a marketing authorization following a review of the submitted data to inform an assessment of benefit/risk. The adopted Opinion can be positive or negative. A positive CHMP opinion is followed by European Commission (“EC”) binding decision to grant a marketing authorization. The marketing authorization is valid throughout the EU and is automatically recognized in three of the four European Free Trade Association states (Iceland, Liechtenstein and Norway). These countries collectively belong to the European Economic Area. The timeframe for the evaluation of an MAA leading to the CHMP opinion is 210 days (discounting procedural clock-stops) from receipt of a valid application for marketing authorization. This time period to complete the scientific review is generally longer than the 210 days as “clock stops” are required to respond to additional written or oral information requested by the EMA. Following a positive CHMP opinion, the EU Commission has 67 days to issue the EC Decision (i.e. the marketing authorization).

Article 3 of Regulation (EC) No 726/2004 defines the scope and eligibility of applications for evaluation under the centralized procedure through which medicinal products must ("mandatory scope") or may ("optional scope" or "Generic/Hybrid") be authorized by the Community. The centralized procedure is compulsory for certain medicinal products, including medicinal products derived from biotechnological processes, orphan medicinal products, advanced therapy medicinal products and products indicated for the treatment of certain diseases including treatment of neurodegenerative diseases. It is optional for new active substances and products that can demonstrate a significant therapeutic, scientific or technical innovation, where approval would be in the interest of public health. Our portfolio of innovative orphan products for neurodegenerative diseases is subject to the mandatory centralized procedure.

Accelerated evaluation may be granted in exceptional cases, following a justified request from the applicant, when a medicinal product is of a major public health interest, particularly from the point of view of therapeutic innovation. CHMP determines what constitutes a major public interest on a case by case basis. Justifications must include the major benefits expected and present the arguments to support the claim that the medicinal product introduces new methods of therapy or improves on existing methods, thereby addressing, to a significant extent, the greater unmet needs for maintaining and improving public health. During an accelerated assessment, the opinion of the CHMP is given, in principle, within 150 days. The EU Commission Decision is then issued according to the timetable described above.

Innovative medicinal products authorized in the EU on the basis of a full stand-alone MAA consisting of pharmaceutical and preclinical testing results and clinical trial data (as opposed to an application for a generic marketing authorization that relies on the results of pre-clinical and clinical trials available in the marketing authorization dossier for another, previously approved, reference medicinal product) are treated as reference medicinal products and accordingly entitled to eight years’ data exclusivity. During this period, applicants for approval of generics of these innovative products cannot reference or rely upon data contained in the marketing authorization dossier submitted for the innovative medicinal product. Even if the generic product is approved, it cannot be placed on the market until the full 10-year period of market protection has elapsed from the initial authorization of the reference medicinal product. This period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the MA holder for the innovative product obtains an authorization for new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies or as the result of significant preclinical or clinical trials.

In the EU, orphan medicinal product designation is considered by the EMA for drugs intended to diagnose, prevent or treat a life-threatening or very serious condition with a prevalence of no more than 5 in 10,000 people in the EU. In addition, the sponsor is required to demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition that has been authorized in the EU or if such method exists, the medicinal product is of significant benefit to those affected by the condition as compared to approved methods. Medicinal products developed for treating serious and chronic conditions would likely not be marketed without incentives due to low market return on the sponsor's development investment. As such, they may also be eligible for an EU orphan drug designation. Benefits of being granted orphan designation are significant, including up to ten years of market exclusivity. During this ten-year period, the EMA may not accept a new marketing application for a similar medicinal product for the same therapeutic indication as the approved orphan medicinal product. Pursuant to Regulation (EC) 1901/2006 on medicinal products for pediatric use, the 10-year orphan market exclusivity can be extended to a maximum period of 12 years on the satisfactory completion of all the key elements of the agreed PIP. We have been granted orphan drug designation for eteplirsen in the EU.

Similarly to the U.S., marketing authorization holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA and/or the national competent authorities of the EU Member States. This oversight applies both before and after the granting of manufacturing and marketing authorizations. It includes compliance with EU GMP and GDP rules in relation to such activities as distribution, importing and exporting of medicinal products, rules governing conduct of pharmacovigilance and requirements governing advertising, promotion and sale of medicinal products.

Failure to comply with the EU Member State laws implementing the EU Community Code on medicinal products, and EU rules governing the promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices, with the EU Member State laws that apply to the promotion of medicinal products, statutory health insurance, bribery and anti-corruption or with other applicable regulatory requirements can result in enforcement action by the relevant EU Member State authorities. This may include any of the following: fines, imprisonment, orders forfeiting products or prohibiting or suspending their supply to the market, orders to suspend, vary, or withdraw the marketing authorization or requiring the manufacturer to issue public warnings, or to conduct a product recall.

The collection and use of personal health data and other personal information in the EU is governed by the provisions of the Data Protection Directive as implemented into national laws by the EU Member States. This Directive imposes a number of strict obligations and restrictions on the ability to collect, analyze and transfer patient data, including sensitive health data from clinical trials and adverse event reporting. There is, moreover, a growing trend towards imposition of an obligation of public disclosure of clinical trial data in the EU, which adds to the complexity of obligations relating to the processing of health data from clinical trials. Such public disclosure obligations are provided in the new EU Clinical Trials Regulation, EMA disclosure initiatives, and voluntary commitments by industry. The Data Protection Directive also includes requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals prior to processing their personal data or personal health data, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive also prohibits the transfer of personal data to countries outside of the EU Member States that are not considered by the EU to provide an adequate level of data protection. These countries include the U.S. Failure to comply with the requirements of the Data Protection Directive and the related national data protection laws of the EU Member States may result in fines and other administrative penalties. Data protection authorities from the different EU Member States may interpret the EU Data Protection Directive and national laws differently, which adds to the complexity of processing personal data in the EU Guidance developed at both EU level and at national level in individual EU Member States concerning implementation and compliance practices are often updated or otherwise revised.

In December 2015, a proposal for an EU General Data Protection Regulation, intended to replace the current EU Data Protection Directive, was agreed between the European Parliament, the Council of the European Union and the European Commission. The EU General Data Protection Regulation, which was officially adopted in April 2016 and will be applicable in May 2018, will introduce new data protection requirements in the EU, as well as substantial fines for breaches of the data protection rules. The EU General Data Protection Regulation will increase our responsibility and liability in relation to any personal data that we process, and we may be required to put in place additional mechanisms to ensure compliance with the new EU data protection rules.

The approval process in other countries outside the U.S. and the EU varies from country to country, and the time may be longer or shorter than that required for the FDA approval. In addition, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement for market access vary greatly from country to country. In all cases, clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

Expanded / Early Access

In certain countries, drug products approved in the U.S. or the EU can be accessed by patients before the drug has obtained marketing approval in such country. There are various forms of this access including, but not limited to, the actual purchase of product by the purchaser, which is often times the government for patients, on a named patient basis, and providing the product free of charge on a named patient basis for compassionate use. Each country has its own laws and regulations that apply to these forms of access and the extent and nature of such laws and regulations vary by country.

We have initiated a MAP for eteplirsen in select countries in Europe, North America, South America and Asia where it currently has not been approved. The MAP provides a mechanism through which physicians can prescribe eteplirsen, within their professional responsibility, to patients who meet pre-specified medical and other criteria and can secure funding. We have commenced shipments through the MAP and continue to expand the MAP to include more countries. In addition, we contracted with third party distributors and service providers to distribute eteplirsen in certain areas outside the U.S., such as Israel and certain countries in the Middle East, on a named patient basis.

Other Regulatory Requirements

In addition to regulations enforced by the FDA and foreign authorities relating to the clinical development and marketing of products, we are or may become subject to regulation under the Occupational Safety and Health Act, the Toxic Substances Control Act, the Resource Conservation and Recovery Act and other present and potential future foreign, federal, state and local laws and regulations. Although we believe that we are in material compliance with applicable environmental laws that apply to us, we cannot predict whether new regulatory restrictions will be imposed by state or federal regulators and agencies or whether existing laws and regulations will adversely affect us in the future.

Healthcare Fraud and Abuse Laws

We are subject to various federal, state and local laws targeting fraud and abuse in the healthcare industry, including anti-kickback and false claims laws. Violations of fraud and abuse laws may be punishable by crime or civil sanctions, including fines and civil monetary penalties, and/or exclusion from federal health care programs (including Medicare and Medicaid). Federal and state authorities are paying increased attention to enforcement of these laws within the pharmaceutical industry, and private individuals have been active in alleging violations of the laws and brining suits on behalf of the government under the federal False Claims Act (“FCA”). Violations of international fraud and abuse laws could result in similar penalties, including exclusion from participation in health programs outside the U.S. If we were subject to allegations concerning, or were convicted of violating, these laws, our business could be harmed.

The federal Anti-Kickback Statute generally prohibits, among other things, a pharmaceutical manufacturer from directly or indirectly soliciting, offering, receiving, or paying any remuneration in cash or in kind where one purpose is either to induce the referral of an individual for, or the purchase or prescription of a particular drug that is payable by a federal health care program, including Medicare or Medicaid. The Healthcare Reform Act clarifies the intent requirements of the federal Anti-Kickback Statute, providing that a person or entity does not need to have actual knowledge of the statute or a specific intent to violate the statute. Violations of the federal Anti-Kickback Statute can result in exclusion from Medicare, Medicaid or other governmental programs as well as civil and criminal fines and penalties of up to $50,000 per violation and three times the amount of the unlawful remuneration. In addition, the Healthcare Reform Act revised the FCA to provide that a claim arising from a violation of the Federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the FCA. The majority of states also have anti-kickback, false claims, and similar fraud and abuse laws and although the specific provisions of these laws vary, their scope is generally broad, and there may not be regulations, guidance or court decisions that apply the laws to particular industry practices. There is therefore a possibility that our practices might be challenged under the anti-kickback statutes or similar laws.

Federal and state false claims laws generally prohibit anyone from knowingly and willfully, among other activities, presenting, or causing to be presented for payment to third party payors (including Medicare and Medicaid) claims for drugs or services that are false or fraudulent (which may include claims for services not provided as claimed or claims for medically unnecessary services). False or fraudulent claims for purposes of the FCA carry fines and civil penalties for violations ranging from $10,957 to $21,916 for each false claim, plus up to three times the amount of damages sustained by the federal government and, most critically, may provide the basis for exclusion from federally funded healthcare programs. There is also a criminal FCA statute by which individuals or entities that submit false claims can face criminal penalties. In addition, under the federal Civil Monetary Penalty Law, the Department of Health and Human Services Office of Inspector General has the authority to exclude from participation in federal health care programs or to impose civil penalties against any person who, among other things, knowingly presents, or causes to be presented, certain false or otherwise improper claims. Our activities relating to the sale and marketing of our products may be subject to scrutiny under these laws.

Laws and regulations have also been enacted by the federal government and various states to regulate the sales and marketing practices of pharmaceutical manufacturers. The laws and regulations generally limit financial interactions between manufacturers and health care providers; require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the U.S. federal government; and/or require disclosure to the government and/or public of financial interactions (so-called "sunshine laws"). The Healthcare Reform Act requires manufacturers to submit information to the FDA on the identity and quantity of drug samples requested and distributed by a manufacturer during each year. Recent scrutiny of pharmaceutical pricing practices by certain companies may lead to changes in laws that currently allow substantial flexibility in pricing decisions by pharmaceutical manufacturers. Such changes could occur at the federal level or state level and may be adopted by statute, rule, or sub-regulatory policies. State laws may also require disclosure of pharmaceutical pricing information and marketing expenditures. Many of these laws and regulations contain ambiguous requirements or require administrative guidance for implementation. Given the lack of clarity in laws and their implementation, our activities could be subject to the penalty provisions of the pertinent federal and state laws and regulations.

Pharmaceutical Pricing and Reimbursement

Our first commercial product in the U.S., EXONDYS 51, was granted accelerated approval by the FDA on September 19, 2016. We have an ongoing dialogue with payors with the goal of obtaining broad coverage for EXONDYS 51. To date, payors' policies on coverage for EXONDYS 51 have varied on their approach, including policies that allow broad coverage per the EXONDYS 51 prescribing information, policies that provide limited coverage, to policies that have denied coverage. The majority of payors have policies that provide for case-by-case coverage or restricted coverage. Our revenue depends, in part, upon the price that payors, including government authorities or programs, private health insurers and other organizations, reimburse on behalf of patients and physicians for the cost of EXONDYS 51.

Third Party Reimbursement and Pricing in the U.S.

Commercial Insurance. Coverage and reimbursement of our EXONDYS 51 varies from commercial payor to commercial payor. Many commercial payors, such as managed care plans, manage access to FDA approved products' coverage partly to control costs to their plans, and may use drug formularies and medical policies to limit their exposure. Exclusion from policies can directly reduce product usage in the payor's patient population and may negatively impact utilization in other payor plans, as well.

Medicaid. Our product EXONDYS 51 is eligible to be reimbursed by Medicaid. Medicaid is a joint federal and state program that is administered by the states for low income and disabled beneficiaries. Under the Medicaid Drug Rebate Program, participating manufacturers are required to pay a rebate for each unit of product reimbursed under the state Medicaid programs. The amount of the rebate for each product is set by law and may be subject to an additional discount if certain pricing increases more than inflation. State Medicaid programs and Medicaid managed care plans can seek additional "supplemental" rebates from manufacturers in connections with favorable positioning on formularies.

Medicare. Medicare is a federal program that is administered by the federal government that covers individuals age 65 and over as well as those with certain disabilities. Our product EXONDYS 51 is eligible for reimbursement under Medicare Part B. Medicare Part B generally covers drugs that must be administered by physicians. Medicare Part B pays for such drugs under a payment methodology based on the average sales price ("ASP") of the drugs. Reimbursement

levels and reimbursement methodologies have come under scrutiny and may be subject to change. The Centers for Medicare & Medicaid Services ("CMS") are also increasingly bundling drug reimbursement into procedure costs, which can severely decrease the reimbursement rates for some manufacturers' drugs.

Federal Purchasers. Drug products are subject to discounted pricing when purchased by federal agencies via the Federal Supply Schedule ("FSS"). FSS participation is required for a drug product to be covered and reimbursed by certain federal agencies and for coverage under Medicaid, Medicare Part B and the Public Health Service ("PHS") 340B drug pricing program. FSS pricing is negotiated periodically with the Department of Veterans Affairs. FSS pricing is intended not to exceed the price that a manufacturer charges its most-favored non-federal customer for its product. In addition, prices for drugs purchased by the Veterans Administration, Department of Defense (including drugs purchased by military personnel and dependents through the TRICARE retail pharmacy program), Coast Guard, and PHS are subject to a cap on pricing (known as the "federal ceiling price") and may be subject to an additional discount if pricing increases more than the rate of inflation.

PHS 340B Drug Pricing Program. To maintain coverage of drugs under the Medicaid Drug Rebate Program and Medicare Part B, manufacturers are required to extend discounts to certain purchasers under the PHS 340B drug pricing program. Purchasers eligible for discounts include hospitals that serve a disproportionate share of financially needy patients, community health clinics and other entities that receive health services grants from the PHS.

Healthcare and Other Reform. In the U.S., federal and state governments continue to propose and pass legislation designed to reform delivery of, or payment for, health care, which include initiatives to reduce the cost of healthcare. For example, in March 2010, the U.S. Congress enacted the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act, or the Healthcare Reform Act, which expanded health care coverage through Medicaid expansion and the implementation of the individual mandate for health insurance coverage and which included changes to the coverage and reimbursement of drug products under government healthcare programs. Under the Trump administration, there have been ongoing efforts to modify or repeal all or certain provisions of the Healthcare Reform Act. The Trump administration may also take executive action in the absence of legislative action. For example, in October 2017, the President announced that his administration will withhold the cost-sharing subsidies paid to health insurance exchange plans serving low-income enrollees. Actions by the administration are widely expected to lead to fewer Americans having more comprehensive health insurance compliant with the Healthcare Reform Act, even in the absence of a legislative repeal. Tax reform legislation was also enacted at the end of 2017 that includes provisions that will affect healthcare insurance coverage and payment, such as the elimination of the tax penalty for individuals who do not maintain sufficient health insurance coverage beginning in 2019. In a November 2017 report, the Congressional Budget Office estimates that the elimination will increase the number of uninsured by 4 million in 2019 and 13 million in 2027.

There have also been efforts by government officials or legislators to implement measures to regulate prices or payment for pharmaceutical products, including legislation on drug importation. Recently, there has been considerable public and government scrutiny of pharmaceutical pricing and proposals to address the perceived high cost of pharmaceuticals. There have also been recent state legislative efforts to address drug costs, which generally have focused on increasing transparency around drug costs or limiting drug prices.

General legislative cost control measures may also affect reimbursement for our products. The Budget Control Act, as amended, resulted in the imposition of 2% reductions in Medicare (but not Medicaid) payments to providers in 2013 and will remain in effect through 2025 unless additional Congressional action is taken. Any significant spending reductions affecting Medicare, Medicaid or other publicly funded or subsidized health programs that may be implemented and/or any significant taxes or fees that may be imposed on us could have an adverse impact on our results of operations.

Third Party Reimbursement and Pricing outside the U.S.

In the EU and certain other territories, price controls and Health Technology Assessments for new, highly priced medicines are expected. Uncertainty exists about the pricing and reimbursement status of newly approved products in the EU. Criteria such as cost-effectiveness, cost per quality-adjusted life year, budget impact, or others, in addition to the clinical benefit, are often required to demonstrate added value or benefit of a drug and vary by country. Third party reimbursement limits may reduce the demand for our products. The pace of the application process in some countries could also delay commercial product launches. Gaining acceptance of our product pipeline and an

economically viable reimbursement terms in the EU and other markets will require strong education and awareness efforts around DMD.

Competition

The pharmaceutical and biotechnology industries are intensely competitive, and any product candidate developed by us would likely compete with existing drugs and therapies. There are many pharmaceutical companies, biotechnology companies, public and private universities, government agencies and research organizations that compete with us in developing various approaches to the treatment of rare, neuromuscular and other diseases. Many of these organizations have substantially greater financial, technical, manufacturing and marketing resources than we have. Several of them have developed or are developing therapies that could be used for treatment of the same diseases that we are targeting. In addition, some of these competitors have significantly greater commercial infrastructures than we have. Our ability to compete successfully will depend largely on:

- our ability to complete clinical development and obtain regulatory approvals for our product candidates;
- the efficacy, safety and reliability of our product candidates;
- the timing and scope of regulatory approvals;
- product acceptance by physicians and other health-care providers;
- protection of our proprietary rights and the level of generic competition;
- the ability to have freedom to operate to commercialize our product candidates;
- the speed at which we develop product candidates;
- our ability to supply commercial quantities of a product to the market;
- obtaining reimbursement for product use in approved indications;
- our ability to recruit and retain skilled employees; and
- the availability of substantial capital resources to fund development and commercialization activities, including the availability of funding from the U.S. government.

DMD Program Competition. Currently, other than EXONDYS 51, no disease-modifying product in the U.S. has been approved for the treatment of DMD and no such product is commercially available outside the European Economic Area ("EEA"). Other companies, however, have product candidates or other interests in development for the treatment of DMD.

PTC Therapeutics ("PTC") has a small molecule candidate, ataluren, which targets nonsense mutations in development. The European Commission granted conditional marketing authorization for ataluren for the treatment of a subset of DMD patients in August 2014. In January 2016, PTC announced the completion of its rolling submission of an NDA for ataluren to the FDA and submission of its Phase 3 Ataluren Confirmatory Trial ("ACT") DMD clinical trial result to the EMA. In October 2017, PTC announced its receipt of a complete response letter from FDA and PTC's intent to file a formal dispute resolution request. In February 2018, PTC announced that the FDA reiterated its prior position and denied PTC's appeal of the complete response letter. Ataluren uses a distinct scientific approach that addresses a different genotype of DMD patients compared to eteplirsen. Therefore, we do not believe ataluren is appropriate for the treatment of DMD patients that are amenable to exon-skipping therapy.

BioMarin has an exon 51-skipping product candidate, drisapersen. An NDA for drisapersen was filed by the FDA and an MAA was submitted to the EMA in June 2015. In January 2016, the FDA issued a complete response letter and declined the approval for drisapersen for the treatment of DMD. 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. BioMarin further announced its intent to continue to explore the development of next generation oligonucleotides for the treatment of DMD.

Wave Life Sciences ("Wave") announced the selection of its exon-skipping candidate, WVE-210201, to target deletions of Exon 51. In November 2017, Wave announced the initiation of a global Phase 1 clinical trial for WVE-210201 in DMD patients amenable to exon 51 skipping.

Nippon Shinyaku Co. Ltd. (“Nippon”) has reported early clinical development data for its exon 53 skipping candidate, NS-065, and the first patient dosed in a phase 2 study. An extension study of this Phase 2 study currently is enrolling by invitation. It has also reported a phase 1/2 study in Japan. NS-065 has been reported to have received an orphan drug designation in the U.S. and was granted Fast Track by FDA.

Daiichi Sankyo has reported a phase 1/2 clinical trial being underway in Japan for its exon 45 skipping candidate, DS-5141b.

Solid Biosciences, LLC (“Solid”) has reported that the safety and efficacy of its micro-dystrophin gene transfer product candidate for DMD, SGT-001, are being evaluated in a phase 1/2 clinical trial.

Pfizer Inc., following its acquisition of Bamboo Therapeutics, Inc., has initiated a phase 1 clinical trial to test the safety and tolerability of its micro-dystrophin gene transfer product candidate for DMD, PF-06939926.

Other companies continue to pursue approval of products for the treatment of DMD and their products may or may not prove to be safer and/or more efficacious than the products and product candidates in our DMD pipeline. Regarding any of these competitors, it is unknown if further clinical development of these or other exon-skipping compounds is planned.

Additionally, companies such as Santhera, Summit, Catabasis, Pfizer and Tivorsan have unique product candidates in different stages of development or approval in DMD which we believe could be seen as complementary to exon skipping and not a direct replacement of our clinical candidates at this time.

In addition, several companies and institutions have recently entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (CRISPR, AAV, etc.) or 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 Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire, Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, Capricor Therapeutics, Oxford University, Exonics Therapeutics, and Editas Medicine.

Platform Technology Competition. We believe that other biotechnology and pharmaceutical companies share a focus on RNA-targeted drug discovery and development. Competitors with respect to our RNA-targeted technologies include, but are not limited to, Alnylam, Tekmira Pharmaceuticals Corp., Ionis, BioMarin, Sanofi, Synthena AG, Santaris Pharma A/S (now Roche), Nippon, Daiichi Sankyo and Wave.

Employees

As of December 31, 2017, we had 255 employees, 113 of whom hold advanced degrees. Of these employees, 121 are engaged directly in research and development activities and 134 are in selling, general and administration including 29 in the sales force. None of our employees are covered by collective bargaining agreements and we consider relations with our employees to be good.

General Corporate Information

We were originally incorporated in the State of Oregon on July 22, 1980, and on June 6, 2013, we reincorporated in the State of Delaware. Our principal executive offices are located at 215 First Street, Suite 415, Cambridge, MA 02142 and our telephone number is (617) 274-4000. On July 12, 2012, our common stock began trading under the symbol “SRPT” on the NASDAQ Global Market on a split-adjusted basis following a one-for-six reverse stock split that was effective on July 11, 2012. Our common stock is quoted on the NASDAQ Global Select Market under the same symbol.

While we achieve revenue from EXONDYS 51 in the U.S. and through distribution of eteplirsen on a named-patient basis or through our MAP outside the U.S., we are likely to continue to incur operating losses in the near term associated with our ongoing operations, research and development activities and potential business development activities. For more information about our revenues and operating losses, see *Item 7, Management’s Discussion and Analysis of Financial Condition and Results of Operations*.

As of December 31, 2017, we had approximately $1,089.9 million of cash, cash equivalents and investments, consisting of $599.7 million of cash and cash equivalents, $479.4 million of short-term investments, $10.0 million of long-term investments and $0.8 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. In addition to pursuing additional cash resources through public or private financings, we may also seek to enter into contracts, including collaborations or licensing agreements with respect to our technologies, with third parties, including government entities.

Where You Can Find Additional Information

We make available free of charge through our corporate website, www.sarepta.com, our annual reports, quarterly reports, current reports, proxy statements and all amendments to those reports as soon as reasonably practicable after such material is electronically filed or furnished with the SEC. These reports may also be obtained without charge by submitting a written request via mail to Investor Relations, Sarepta Therapeutics, Inc., 215 First Street, Suite 415, Cambridge, MA 02142 or by e-mail to investorrelations@sarepta.com. Our internet website and the information contained therein or incorporated therein are not intended to be incorporated into this Annual Report on Form 10-K. In addition, the public may read and copy any materials we file or furnish with the SEC, at the SEC's Public Reference Room at 100 F Street, N.E., Washington, D.C. 20549 or may obtain information on the operation of the Public Reference Room by calling the U.S. Securities and Exchange Commission (the "SEC") at 1-800-SEC-0330. Moreover, the SEC maintains an Internet site that contains reports, proxy and information statements, and other information regarding reports that we file or furnish electronically with the SEC at www.sec.gov.

We have adopted a Code of Business Conduct and Ethics and written charters for our Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee. Each of the foregoing is available on our website at www.sarepta.com under "For Investors—Corporate Governance." In accordance with SEC rules, we intend to disclose any amendment (other than any technical, administrative, or other non-substantive amendment) to the above code, or any waiver of any provision thereof with respect to any of the executive officers, on our website within four business days following such amendment or waiver. In addition, we may use our website as a means of disclosing material non-public information and for complying with our disclosure obligations under Regulation Fair Disclosure promulgated by the SEC. These disclosures will be included on our website under the "For Investors" section.

Item 1A. Risk Factors.

Factors That Could Affect Future Results

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 EXONDYS 51 in the U.S.; we may not be able to meet expectations with respect to EXONDYS 51 sales or attain profitability and positive cash-flow from operations.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. EXONDYS 51 is currently commercially available in the U.S. only, although it is available in certain countries outside of the U.S. on a named patient basis and through our MAP. The commercial success of EXONDYS 51 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 EXONDYS 51;
- the consistency of any new data we collect and analyses we conduct with prior results, whether they support a favorable safety and efficacy profile of EXONDYS 51 and any potential impact on our FDA accelerated approval status and/or FDA package insert for EXONDYS 51;

- the effectiveness of our ongoing EXONDYS 51 commercialization activities, including negotiating and entering into any additional commercial, supply and distribution contracts, scaling up manufacturing 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 and safety of EXONDYS 51 and acceptance of the same by the FDA and medical community since continued approval for this indication 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 cost-effectiveness of EXONDYS 51 and whether we can consistently manufacture it in commercial quantities and at acceptable costs;
- the rate and consistency with which EXONDYS 51 is prescribed by physicians, which depends on physicians' views on the safety and efficacy of EXONDYS 51;
- our ability to secure and maintain adequate reimbursement for EXONDYS 51, including during 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;
- our ability to obtain and maintain patent protection for EXONDYS 51, 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 or commercialization 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 EXONDYS 51, which may be different than expected;
- the sufficiency of our drug supply to meet commercial and clinical demands which could be negatively impacted if our projections on the potential number of amenable patients and their average weight are inaccurate, we are subject to unanticipated regulatory requirements that increase our drug supply needs, our current drug supply is destroyed or negatively impacted at our manufacturing sites, storage sites or in transit, or it takes longer than we project for the number of patients we anticipate to get on EXONDYS 51 and any significant portion of our EXONDYS 51 supply expires before we are able to sell it;
- our ability to obtain regulatory approvals to commercialize EXONDYS 51 in markets outside of the U.S.; and
- the awareness of patients with DMD of their mutation and whether the mutation is amenable to EXONDYS 51.

In addition, the process leading to a patient's first infusion of EXONDYS 51 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. As the launch of EXONDYS 51 continues to progress, we expect the variation among patients to decline, leading to a faster time to infusion. However, delays in the process prior to first infusion could negatively impact the sales of EXONDYS 51.

We may experience significant fluctuations in sales of EXONDYS 51 from period to period and, ultimately, we may never generate sufficient revenues from EXONDYS 51 to reach or maintain profitability or sustain our anticipated levels of operations.

We may not be able to expand the global footprint of, or obtain any significant revenues, from sales of eteplirsen outside of the U.S.

Although we contracted with third party distributors to distribute eteplirsen in certain countries outside the U.S. on a named patient basis, and initiated a limited launch of an ex-U.S. eteplirsen MAP, which we plan to expand to other jurisdictions in the future, and although we continue to pursue regulatory approval of eteplirsen in certain targeted jurisdictions, such as the EU and Israel, we may not be successful in expanding access to eteplirsen nor produce any significant revenues from eteplirsen sales outside of the U.S. For example, healthcare providers in MAP jurisdictions may not be convinced that their patients can benefit from eteplirsen or may prefer to wait until such time as eteplirsen is approved by a regulatory authority in their country before prescribing eteplirsen. Even if a healthcare provider is interested in obtaining access to eteplirsen for its patient through the MAP, the patient will not be able to obtain access to eteplirsen if payment for the drug is not secured. Additionally, we may not be able to obtain regulatory approval in the jurisdictions we have targeted, such as the EU, if our product approval applications, data packages submitted to regulatory authorities, and any additional data and analyses we submit in response to requests and concerns from regulatory authorities, do not support or convince regulatory authorities of the safety and efficacy of eteplirsen. If we fail to obtain regulatory approvals, particularly for our eteplirsen MAA in the EU, our ability to make revenues from eteplirsen sales outside of the U.S. will be limited. Even if we are successful in obtaining regulatory approval of eteplirsen outside of the U.S., our revenue earning capacity will depend on commercial and medical infrastructure, pricing and reimbursement negotiations and decisions with third party payors, including government payors. See "*— Even though EXONDYS 51 has been approved for marketing in the U.S., we may not receive approval to commercialize EXONDYS 51 outside of the U.S."*

EXONDYS 51 may cause undesirable side effects or have other properties that could negatively impact its U.S. approval status and/or limit its commercial potential outside of the U.S.

If we or others identify previously unknown side effects, in particular if they are severe, or if known side effects are more frequent or severe than in the past, then:

- sales of EXONDYS 51 may decrease;
- regulatory approvals for EXONDYS 51 may be restricted, withdrawn or pending applications for approvals may be rejected;
- we may decide to, or be required to, send product warning letters or field alerts to physicians, pharmacists and hospitals;
- additional non-clinical or clinical trials, changes in labeling or changes to manufacturing processes, specifications and/or facilities may be required;
- our reputation in the marketplace may suffer; and
- government investigations or lawsuits, including class action suits, may be brought against us.

Any of the above occurrences would harm or prevent sales of EXONDYS 51, increase our expenses and impair our ability to successfully commercialize EXONDYS 51. Furthermore, as EXONDYS 51 is used in wider populations and in a less rigorously controlled environment than in clinical trials, regulatory authorities, healthcare practitioners, third party payors or patients may perceive or conclude that the use of EXONDYS 51 is associated with previously unknown serious adverse effects, undermining our commercialization efforts.

We currently rely on third parties to manufacture EXONDYS 51 and to produce our product candidates; our dependence on these parties, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet commercial, MAP, 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 EXONDYS 51 or our product candidates in the quantities needed to meet commercial, clinical or MAP demand for EXONDYS 51, or to conduct our research and development programs and conduct clinical trials for our product candidates, including PPMO. 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 EXONDYS 51 and our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of EXONDYS 51 and our product candidates, which 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 can be lengthy and could cause delays in our development efforts. Any interruption of the development or operation of those facilities due to, among other reasons, events such as order delays for equipment or materials, equipment malfunction, 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 earthquake or fire, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available EXONDYS 51, product candidates or materials.

If these third parties were to 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 EXONDYS 51 or our product candidates in sufficient quality and quantity required for our planned commercial, pre-clinical and clinical or MAP use of EXONDYS 51 would adversely affect our various product research, development and commercialization efforts.

We have, through our third party manufacturers, produced or are in the process of producing supply of our product candidates and EXONDYS 51, respectively, based on our current understanding of market demands and our anticipated needs for our research and development efforts, clinical trials, MAPs and commercial sales. In light of the limited number of third parties with the expertise to produce EXONDYS 51 and our 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 EXONDYS 51 and our other product candidates to meet demands that meet or exceed our projected needs. 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 EXONDYS 51 and our 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 could impact our ability to execute our business plans on expected or required timelines in connection with the commercialization of EXONDYS 51 and the continued development of our product candidates, including our follow-on exon-skipping product candidates and PPMO. We may also be required to enter into long-term manufacturing agreements that contain exclusivity provisions and /or substantial termination penalties, which could have a material adverse effect on our business prior to and after commercialization.

The third parties we use in the manufacturing process for EXONDYS 51 and our 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. While we work diligently with all contract manufacturers to maintain full compliance, we do not have direct control over a third party manufacturer’s compliance with these regulations and requirements. In addition, changes in cGMP could negatively impact the ability of our contract manufacturers to complete the manufacturing process of EXONDYS 51 and our product candidates in a compliant manner on the schedule we require for commercial and clinical trial use, respectively. The failure to achieve and maintain compliance with cGMP and other applicable government regulations, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in product recalls, clinical holds, delayed or withheld approvals, patient injury or death. This risk is particularly heightened as we optimize manufacturing for follow-on exon skipping products and next-generation technologies such as PPMO. If our contract manufacturers fail to adhere to applicable cGMP and other applicable government regulations, or experience manufacturing problems, we will suffer significant 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 results, the success of our commercialization of EXONDYS 51 and/or our development efforts for our product candidates, including golodirsen, casimersen and SRP-5051, could be significantly delayed, fail or otherwise be negatively impacted.

We may not be able to successfully scale up manufacturing of EXONDYS 51 or our product candidates in sufficient quality and quantity or within sufficient timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact the commercial success of EXONDYS 51 and/or the development of our product candidates and next generation chemistries like PPMO.

We are working to increase manufacturing capacity and scale up production of some of the components of our drug products. During the remainder of 2017, our focus remains on (i) achieving larger-scale manufacturing capacity for EXONDYS 51 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, including PPMO. 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. Compliance with cGMP requirements and other quality issues may arise during our efforts to increase manufacturing capacity and scale up production with our current or any new contract manufacturers. These issues may arise in connection with the underlying materials, the inherent properties of EXONDYS 51 or a product candidate, EXONDYS 51 or a product candidate in combination with other components added during the manufacturing and packaging process or during shipping and storage of the APIs or finished drug product. In addition, in order to release EXONDYS 51 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. We may not be able to successfully validate, or maintain validation of, our manufacturing processes and analytical methods or demonstrate adequate purity, stability or comparability of EXONDYS 51 or our product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our manufacturing processes and analytical methods or to demonstrate adequate purity, stability or comparability, the commercial availability of EXONDYS 51 and the continued development and/or regulatory approval of our product candidates, including PPMO, may be delayed or otherwise negatively impacted, which could significantly harm our business.

During work with our third party manufacturers to increase and optimize manufacturing capacity and scale up production, it is possible that they could make proprietary improvements in the manufacturing and scale-up processes for EXONDYS 51 or our product candidates, including PPMO. 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, it is possible that we will 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. Any failure to secure the intellectual rights required for the manufacturing process needed for large-scale clinical trials or commercialization of EXONDYS 51 or the continued development of our product candidates, including PPMO, could cause significant delays in our business plans or otherwise negatively impact the commercialization of EXONDYS 51 or the continued development of our product candidates, including PPMO.

If we are unable to maintain our agreements with third parties to distribute EXONDYS 51 to patients, our results of operations and business could be adversely affected.

We rely on third parties to commercially distribute EXONDYS 51 to patients in the U.S. We have contracted with a third party logistics company to warehouse EXONDYS 51 and with distributors and specialty pharmacies to sell and distribute it 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 EXONDYS 51. If we are unable to effectively manage the distribution process, the sales of EXONDYS 51, 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 EXONDYS 51 or serious adverse events and/or product complaints regarding EXONDYS 51;
- not effectively sell or support EXONDYS 51;
- reduce or discontinue their efforts to sell or support EXONDYS 51;
- not devote the resources necessary to sell EXONDYS 51 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 eteplirsen to patients outside of the U.S., we have contracted with third party distributors and service providers to distribute eteplirsen in certain countries on a named patient basis and through our ex-U.S. MAP. We will need to continue building out our network for commercial distribution in jurisdictions in which eteplirsen is 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 eteplirsen. Any such events may result in regulatory actions that may include suspension or termination of the distribution and sale of eteplirsen in a certain country, loss of revenue, and/or reputational damage, which could harm our results of operations and business.

If we are unable to successfully maintain and further develop internal commercialization capabilities, sales of EXONDYS 51 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 EXONDYS 51 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 EXONDYS 51, to deliver a consistent message regarding EXONDYS 51 and be effective in convincing physicians to prescribe EXONDYS 51;
- 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 EXONDYS 51 and its proper administration and educate payors on the safety and efficacy profile of EXONDYS 51 to support favorable coverage decisions; and
- unforeseen costs and expenses associated with maintaining and further developing an independent sales and marketing organization.

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 EXONDYS 51 in the U.S., which would adversely affect our business and financial condition.

We are subject to uncertainty relating to reimbursement policies which, if not favorable for EXONDYS 51, could hinder or prevent EXONDYS 51's commercial success.

Our ability to successfully maintain and/or increase EXONDYS 51 sales 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 EXONDYS 51, or we may be required to sell EXONDYS 51 at an unsatisfactory price.

We expect that private insurers will continue to consider the efficacy, cost-effectiveness and safety of EXONDYS 51, 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 EXONDYS 51 and at what levels. If any new data and information we collect is not favorable, third party insurers may make coverage decisions that negatively impact sales of EXONDYS 51. We continue to have discussions with payors, some of which may eventually deny coverage. We may not receive approval for reimbursement of EXONDYS 51 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 insurers, including managed care organizations, the Medicare or Medicaid programs or other reimbursing bodies or payors limit the indications for which EXONDYS 51 will be reimbursed or fail to recognize accelerated approval and surrogate endpoints as clinically meaningful.

Additionally, in the wake of government and public scrutiny of pharmaceutical pricing practices, there have been efforts at the federal and state levels to implement legislation or regulations to promote transparency in drug pricing or limit drug prices. Such initiatives are likely to continue the pressure on pharmaceutical pricing, may require us to modify our business practices with healthcare practitioners, and may also increase our regulatory burdens and operating costs.

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.

We expect to experience pricing pressures in connection with the sale of EXONDYS 51 and our 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.

Healthcare reform and other governmental and private payor initiatives may have an adverse effect upon, and could prevent commercial success of EXONDYS 51 and our other 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. See *"GOVERNMENT REGULATION- Pharmaceutical Pricing and Reimbursement- Third Party Reimbursement and Pricing in the U.S.-Healthcare and Other Reform."* 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. 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:

- 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 EXONDYS 51 and our other potential products, which would have an adverse effect on our net revenues and operating results.

The Food and Drug Administration Amendments Act of 2007 also provides the FDA enhanced post-marketing authority, including the authority to require post-marketing studies and clinical trials, labeling changes based on new safety information, and compliance with risk evaluations and mitigation strategies approved by the FDA. The FDA's exercise of this authority could result in increased development-related costs following the commercial launch of EXONDYS 51, and could result in potential restrictions on the sale and/or distribution of EXONDYS 51, even in its approved indications and patient populations.

Even though EXONDYS 51 received accelerated approval by the FDA as a treatment for DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping, it faces future post-approval development and regulatory requirements, which will present additional challenges we will need to successfully navigate.

On September 19, 2016, the FDA granted accelerated approval for EXONDYS 51 as a therapeutic treatment for patients with DMD who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. This indication is based on an increase in dystrophin in skeletal muscles observed in some patients treated with EXONDYS 51. EXONDYS 51 will be subject to ongoing FDA requirements governing the labeling, packaging, storage, advertising, promotion, recordkeeping, and we are required to submit additional safety, efficacy and other post-marketing information.

Continued approval for this indication 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 EXONDYS 51; 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 EXONDYS 51 studies, would lead to negative regulatory action from the FDA and/or withdrawal of regulatory approval of EXONDYS 51, and could also negatively impact a decision from EMA on our MAA. In addition, if additional data we collect on eteplirsen in connection with our MAA does not support the safety and efficacy or EXONDYS 51, our approval status in the U.S. could be negatively impacted.

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 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 EXONDYS 51 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.

Even though EXONDYS 51 has been approved for marketing in the U.S., we may not receive approval to commercialize EXONDYS 51 outside of the U.S.

We are not permitted to market or sell EXONDYS 51 in the EU or in any other foreign countries on a commercial basis until we receive the requisite approval from such country's regulatory authorities. In order to market any product in a foreign country, 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. 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. 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 EXONDYS 51.

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 eteplirsen and could adversely affect our business and financial condition. Any such complications may reduce our target market and delay or limit the full commercial potential of eteplirsen. Many foreign countries are undertaking cost-containment measures that could affect pricing or reimbursement of eteplirsen.

In November 2016, we submitted an MAA for eteplirsen to the EMA. The application was validated in December 2016 and is currently under review. We believe that we submitted a robust package of clinical, dystrophin and safety data to support the review of eteplirsen; however, EMA may or could take a different view. We also believe that, in contrast to the FDA approval, the clinical data will be central in evaluating the application, while dystrophin will be supportive of the drug's mechanism of action. Obtaining approval of an MAA or any other application for approval in a foreign country is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject an application or delay, limit or deny approval of eteplirsen for many reasons, including:

- we may not be able to demonstrate to the satisfaction of foreign regulatory authorities that eteplirsen is safe and effective for the treatment of patients with DMD who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping;
- the results of clinical trials may not meet the level of statistical or clinical significance required for approval by foreign regulatory authorities;
- foreign regulatory authorities 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 may conclude that data we submit to them, including data from clinical trials or any other additional data and analyses we submit in support of an approval or in response to requests from regulatory authorities, fail to demonstrate an appropriate level of safety or efficacy of eteplirsen or that eteplirsen's clinical benefits outweigh its safety risks; or such regulatory authorities may disagree with our interpretation of data from preclinical trials or clinical trials and require that we conduct one or more additional trials;
- 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 eteplirsen, 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;
- we may not be able to validate our manufacturing process to the satisfaction of regulatory authorities outside the U.S. or demonstrate adequate cGMP compliance; or
- regulatory authorities outside the U.S. may adopt new or revised approval policies and regulations.

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 is our first commercial product. As a result, our sales, marketing, managerial and other non-technical capabilities are relatively new in the U.S. and we are currently in the process of building a commercial sales force in Europe. We plan to continue to build commercial infrastructure in the EU and in other key countries in order to be ready to launch eteplirsen with a relatively small specialty sales force in the event eteplirsen is 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 fully 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 eteplirsen in such jurisdictions will be adversely affected. Even if we are able to effectively hire a sales force and develop a marketing and sales capabilities, our sales force may not be successful in commercializing eteplirsen or any other 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.

EXONDYS 51 may not be widely adopted by patients, payors or healthcare providers, which would adversely impact our potential profitability and future business prospects.

EXONDYS 51's commercial success, particularly in the near term in the U.S., depends upon its level of market adoption by patients, payors and healthcare providers. If EXONDYS 51 does not achieve an adequate level of market adoption for any reason, our potential profitability and our future business prospects will be severely adversely impacted. The degree of market acceptance of EXONDYS 51 depends on a number of factors, including:

- our ability to demonstrate to the medical community, including specialists who may purchase or prescribe EXONDYS 51, the clinical efficacy and safety of EXONDYS 51 as the prescription product of choice DMD amenable to exon-51 skipping in the U.S.;
- the effectiveness of our sales and marketing organizations and distribution networks;
- the ability of patients or providers to be adequately reimbursed for EXONDYS 51 in a timely manner from government and private payors;
- the actual and perceived efficacy and safety profile of EXONDYS 51, particularly if unanticipated adverse events related to EXONDYS 51 treatment arise and create safety concerns among potential patients or prescribers or if new data and analyses we obtain for eteplirsen do not support, or are interpreted by some parties to not support, the efficacy of EXONDYS 51; and
- the efficacy and safety of our other exon-skipping product candidates, including our exon 45 and exon 53 product candidates, and third parties' competitive therapies.

The patient population suffering from DMD, and in particular those with mutations amenable to exon-51 skipping, 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 is a fatal genetic neuromuscular disorder affecting 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. Our estimate of the size of the patient population is based on published studies as well as internal analyses. If the results of these studies or our analysis of them do not accurately reflect the number of patients with DMD, 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. The small population of DMD patients may also delay patients' recruitment for our clinical trials, especially in light of competing clinical trials.

Since EXONDYS 51 targets a small patient population, 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 been granted orphan drug exclusivity for EXONDYS 51 in the U.S. and an orphan drug designation for eteplirsen in the EU, however, there can be no guarantee that we will be able to maintain orphan exclusivity for such product and product candidates nor that we will receive orphan drug approval or exclusivity and prevent third parties from developing and commercializing products that are competitive to EXONDYS 51 or our other product candidates.

To date, we have been granted orphan drug exclusivity for EXONDYS 51 in the U.S and an orphan drug designation in the EU for eteplirsen. Product candidates granted orphan status in Europe can be provided with up to ten years of marketing exclusivity, meaning that another application for marketing authorization of a later, similar medicinal product for the same therapeutic indication will generally not be approved in Europe during that time period. Although we may have product candidates that obtain orphan drug exclusivity in Europe, the orphan status and associated exclusivity period 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.

As discussed above, we are not guaranteed to receive or maintain orphan status for our current or future product candidates, and if our product candidates that are granted orphan status were to lose their status as orphan drugs or the marketing exclusivity provided for them in the U.S. or the EU, our business and operations could be adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the U.S. and

the EU 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 active ingredient for the same indication beyond the exclusivity period applicable to our product on the basis of orphan drug status. In addition, we cannot guarantee that another company will not receive approval to market a product candidate that is granted orphan drug status in the U.S. or the EU for the same drug and orphan indication as any of our product candidates for which we plan to file an NDA or MAA. If that were to happen, any pending NDA 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.

If we are unable to maintain or obtain orphan drug exclusivity for EXONDYS 51 or other products in the U.S., we may face increased competition.

Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition affecting fewer than 200,000 people in the U.S. A company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition generally receives orphan drug marketing exclusivity for that drug for a period of seven years from the date of its approval. This orphan drug exclusivity prevents the approval of another drug containing the same active moiety used for the same orphan indication, except in circumstances where, based on the FDA's determination, a subsequent drug is safer, more effective or makes a major contribution to patient care, or if the orphan drug manufacturer is unable to assure that a sufficient quantity of the orphan drug is available to meet the needs of patients with the rare disease or condition. Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective. EXONDYS 51 was granted orphan drug exclusivity in the U.S. through September 19, 2023 for the treatment of DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping. However, such exclusivity may not effectively protect the product from competition if the FDA determines that a subsequent drug containing the same active moiety for the same indication is safer, more effective or makes a major contribution to patient care, or if we are unable to assure the FDA that sufficient quantities of EXONDYS 51 are available to meet patient demand. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active moiety or from approving a drug containing the same active moiety for a different indication. If a subsequent drug is approved for marketing for the same or similar indication, we may face increased competition, and our revenues from the sale of EXONDYS 51 will be adversely affected.

We could incur significant liability if it is determined that we are promoting any "off-label" use of EXONDYS 51.

Physicians are permitted to prescribe drug products for uses that are not described in the product's labeling and that differ from those approved by applicable regulatory agencies. Off-label uses are common across medical specialties. Although the FDA and other regulatory agencies do not regulate a physician's choice of treatments, the FDA and other regulatory agencies do generally prohibit advertising and promotion of off-label uses of approved drug products or promotion of an approved drug on information that is not in the final, FDA-approved label for a product and restrict communications on off-label use. Accordingly, we may not promote EXONDYS 51 in the U.S. for use in any indications other than for the treatment of DMD in patients who have a confirmed mutation in the DMD gene that is amenable to exon 51 skipping. Additionally, we face limitations on our ability to promote EXONDYS 51 based on any information that is not included in the final FDA-approved label, including previously published clinical data. The FDA and other regulatory authorities actively enforce laws and regulations prohibiting promotion of a product for off-label uses and the promotion of products for which marketing approval has not been obtained. A company that is found to have improperly promoted its drug product will be subject to significant liability, including civil and administrative remedies as well as criminal sanctions.

Notwithstanding the regulatory restrictions on off-label promotion, the FDA and other regulatory authorities allow companies to engage in truthful, non-misleading and non-promotional scientific exchange concerning their products and recent FDA guidance suggests that there are circumstances in which the FDA would not object to the promotion of certain information that is not included in the approved labeling but that is consistent with the approved labeling. We intend to engage in medical education activities and communicate with healthcare providers in compliance with all applicable laws, regulatory guidance and industry best practices. Although we have established a compliance program and continue to enhance it to ensure that all such activities are performed in a legal and compliant manner, EXONDYS 51 is our first commercial product which could increase risk of non-compliance with our internal compliance policies and applicable rules and regulations, which could negatively impact our business.

Most of our product candidates are at an early stage of development and may never receive regulatory approval.

Other than EXONDYS 51, which the FDA approved for use in the U.S. in September 2016 and for which we filed an MAA in November 2016 with the EMA, our most advanced product candidates are exon 45- and 53-skipping products (casimersen and golodirsen, respectively), PPMO DMD exon 51 skipping product (SRP-5051), and Nationwide Children's Hospital's micro-dystrophin gene therapy program and Galgt2 gene therapy program.

We are in the process of conducting, starting or planning various EXONDYS 51 clinical trials, including trials that are required to comply with regulatory NDA and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements/commitments to verify and describe clinical benefit. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic. The Part I dose-titration portion of this Phase 1/2a study has been completed and Part II open label portion of the study is ongoing. We have also completed the dose titration portion and are conducting the open-label portion of a study for our exon 45-skipping product candidate. Additionally, we are enrolling patients for a clinical trial using exon 45- and 53-skipping product candidates, which we refer to as the ESSENCE study. We have also initiated a first in human study for PPMO DMD exon 51 (SRP-5051). In addition, Nationwide Children's Hospital, with whom we are collaborating, initiated Phase 1/2a clinical trials for their micro-dystrophin gene therapy program and their Galgt2 gene therapy program.

The remainder of our product candidates are in discovery or early stages of development. These product candidates will require significant further development, financial resources and personnel to develop into commercially viable products and obtain regulatory approval, if at all. Currently, our exon 45-skipping product candidate, the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, and the PPMO exon 51 product candidate, each for DMD, are in active clinical development. In addition, both the micro-dystrophin gene therapy program and the Galgt2 gene therapy program of Nationwide Children's Hospital, with whom we are collaborating, have entered into the clinic. Our other product candidates are in discovery, pre-clinical development or inactive. Given the FDA approval of EXONDYS 51, we expect that much of our effort and many of our expenditures over the next several years will be devoted to clinical development and regulatory activities associated with EXONDYS 51 and other exon-skipping candidates as part of our larger follow-on exon strategy in DMD, our other disease candidates, our proprietary chemistry, and other potential therapeutic areas that provide long-term market opportunities. We may be delayed, restricted, or unable to further develop our active and other product candidates or successfully obtain approvals needed to market them. Although EXONDYS 51 was approved under accelerated approval by the FDA in the U.S., we may not be able to obtain an approval of EXONDYS 51 in the EU.

Our RNA-targeted antisense technologies have only been incorporated into one therapeutic commercial product and additional studies may not demonstrate safety or efficacy of our technologies in other product candidates.

Our RNA-targeted platform, utilizing proprietary PMO-based technology has only been incorporated into one therapeutic commercial product to date, EXONDYS 51, however, our confirmatory trials for EXONDYS 51 must verify and describe the clinical benefits in order for EXONDYS 51 to remain approved in the U.S. Although we have conducted and are in the process of conducting clinical trials with EXONDYS 51, an exon 45-skipping product candidate and an exon 53-skipping product candidate and pre-clinical trials with our other product candidates that use our PMO-based antisense technology, additional studies may be needed to determine the safety and efficacy of our PMO-based antisense technology, including our novel PPMO technology. In addition, nonclinical models used to evaluate the activity and toxicity of product candidate compounds are not necessarily predictive of toxicity or efficacy of these compounds in the treatment of human disease. As such, there may be substantially different results observed in clinical trials from those observed in pre-clinical trials. Any failures or setbacks in developing or utilizing our PMO-based technologies, including adverse effects in humans, could have a detrimental impact on our product candidate pipeline and our ability to maintain and/or enter into new corporate collaborations regarding these technologies, which would negatively affect our business and financial condition.

Our pre-clinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, including those based on our PMO-based technologies, 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 the pre-clinical data for PPMO

collected to date is promising, 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. For example, we cannot provide assurances that data from our EXONDYS 51 ongoing studies will be positive and consistent through the study periods or that the interpretation by regulators, such as the FDA or EMA, of the data we collect for our product candidates will be consistent with our interpretations. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, including for those that are based on our PMO-based technologies, then 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. The completion of pre-clinical and clinical trials and regulatory approvals may be delayed for other reasons, such as delays related to patients enrollment for reasons including small patient population, competing clinical trials and patients' concerns regarding trial design; manufacturing of product candidates; and clinical holds. For example, the U.K. Medicine and Healthcare products Regulatory Agency has recently required that dosing stop at all U.K. sites of our Study 4045-301 (ESSENCE) due to one serious adverse event that could possibly be related to the product candidate.

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. 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 or MAA submissions for one or more of our product candidates, and may delay, reject or refuse to accept for review, or approve any NDA or MAA 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 we submit to the FDA. Even if we meet FDA requirements and an advisory committee votes to recommend approval of an NDA 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, and 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. We cannot be sure that any of our product candidates will qualify for accelerated approval or any other expedited development, review and approval programs, or that, if a drug does qualify, that the product candidates will be approved, will be accepted as part of any such program or that the review time will be shorter than a standard review. 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 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 and dystrophin analyses), 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 result in a decision by the Company not to proceed with an NDA 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 the exon 53-, exon 45- and PPMO exon 51-skipping 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 or MAA submissions.

Due to the above factors, among others, our product candidates could take a significantly longer time to gain regulatory approval than we expect, or may never gain regulatory approval, which would delay or eliminate any potential commercialization or product revenue for us and result in a material adverse effect on the Company that could involve changes, delays in or terminations of programs in our pipeline, delays or terminations of pre-clinical and clinical trials, and termination of contracts related to the development of our product candidates which can include significant termination costs, workforce reductions and limited ability to raise additional funds to execute company plans.

Even if we are able to comply with all regulatory requests and requirements, the delays resulting from satisfying such requests and requirements, the cost of compliance, or the effect of regulatory decisions (e.g., decisions limiting labeling and indications requested by us for a product candidate) may no longer make commercialization of a product candidate desirable for us from a business perspective, which could lead us to decide not to commercialize a product candidate.

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 are not able to maintain regulatory compliance, we may be subject to civil and criminal penalties or we may not be permitted to continue marketing our products, which could have a material adverse effect on our financial condition and harm our competitive position in the market place.

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, certain federal and state healthcare laws and regulations will apply to or affect our business. The 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.

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.

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. In particular, the Healthcare Reform Act includes a number of provisions aimed at strengthening the government’s ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, and amendments to the False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations. While it is too early to predict what effect these changes will have on our business, 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. For example, federal enforcement agencies recently have shown interest in pharmaceutical companies’ product and patient assistance programs for private patients, including manufacturer reimbursement support services and relationships with specialty pharmacies. Some of these investigations have resulted in significant civil and criminal settlements. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business and financial condition and growth prospects.

In connection with the commercial launch of EXONDYS 51, we have initiated our compliance program and are in the process of expanding our experienced compliance team that will continue to work towards developing a program based on industry best practices that is designed to ensure that our commercialization of EXONDYS 51 complies with all applicable laws, regulations and industry standards. As this program has not yet been tested and 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. Any action against us for violation of these laws, even if we successfully defend against such action, could 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 rely on third parties to provide services in connection with our pre-clinical and clinical development programs. The inadequate performance by or loss of any of these service providers could affect our product candidate development.

Several third parties provide services in connection with our pre-clinical and clinical development programs, including in vitro and in vivo studies, assay and reagent development, immunohistochemistry, toxicology, pharmacokinetics, clinical assessments, data monitoring and management, statistical analysis and other outsourced activities. If these service providers do not adequately perform the services for which we have contracted or cease to continue operations and we are not able to quickly find a replacement provider or we lose information or items associated with our product candidates, our development programs may be delayed.

We are winding down our expired U.S. government contract, and thus further development of our Ebola and Marburg product candidates may be limited by our ability to obtain additional funding for these programs and 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 development programs. The July 2010 U.S. DoD contract providing funds for our Marburg program expired in July 2014, and the Ebola portion of the contract was previously terminated by the DoD in 2012 for convenience of the DoD. We are currently involved in contract wind-down activities and may be subject to additional government audits prior to collecting final cost reimbursements and fees owed by the government. If we are not able to complete such audits or other government requirements successfully, then the government may withhold 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.

We may not be able to successfully conduct clinical trials due to various process-related factors which could negatively impact our business plans.

The successful start and completion of any of our clinical trials within time frames consistent with our business plans is dependent on regulatory authorities and various factors, which include, but are not limited to, our ability to:

- recruit and retain employees, consultants or contractors with the required level of expertise;
- recruit and retain sufficient patients needed to conduct a clinical trial;
- enroll and retain participants, which is a function of many factors, including the size of the relevant population, the proximity of participants to clinical sites, activities of patient advocacy groups, the eligibility criteria for the trial, the existence of competing clinical trials, the availability of alternative or new treatments, side effects from the therapy, lack of efficacy, personal issues and ease of participation;
- timely and effectively contract with (under reasonable terms), manage and work with investigators, institutions, hospitals and the contract research organizations (“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;
- ensure adherence to trial designs and protocols agreed upon and approved by regulatory authorities and applicable legal and regulatory guidelines;
- manage or resolve unforeseen adverse side effects during a clinical trial;
- conduct the clinical trials in a cost-effective manner, including managing foreign currency risk in clinical trials conducted in foreign jurisdictions and cost increases due to unforeseen or unexpected complications such as enrollment delays, or needing to outsource certain Company functions during the clinical trial; and
- execute clinical trial designs and protocols approved by regulatory authorities without deficiencies.

If we are not able to manage the clinical trial process successfully, our business plans could be delayed or be rendered unfeasible for us to execute within our planned or required time frames, or at all.

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

We incurred an operating loss of $23.1 million for the three months ended December 31, 2017. Our accumulated deficit was $1.2 billion as of December 31, 2017. Although we launched EXONDYS 51 in the U.S. in September 2016, we believe that it will take us some time to attain profitability and positive cash flow from operations. We have generally incurred expenses related to research and development of our technologies and product candidates, 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 our launch and commercialization of EXONDYS 51 in the U.S.;
- expand the global footprint of EXONDYS 51 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 2018. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when, or if, we will become profitable.

We will need additional funds to conduct our planned research, development, manufacturing and business development efforts. If we fail to attract and manage significant capital on acceptable terms or fail to enter into strategic relationships, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

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 EXONDYS 51 as well as continue the development of product candidates in our pipeline, 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. The Company and our board of directors continue to assess optimization in the size and structure of the Company as well as in its strategic plans. For example, in March 2016, we announced a long-term plan to consolidate facilities within Massachusetts and closing our Corvallis, Oregon offices by end of that year. In June 2017, we announced the opening of our research and manufacturing center in Andover, Massachusetts. In addition, we recently established our European headquarters in Zug, Switzerland. Any failure on our part to strategically and successfully manage the funds we raise, with respect to factors within our control, could impact our ability to successfully commercialize EXONDYS 51 and continue developing our product candidates. Some of the factors partially or entirely outside of our control that could impact our ability to raise funds, as well as the sufficiency of funds the Company has to execute its business plans successfully, include the success of our research and development efforts, the status of our pre-clinical and clinical testing, costs and timing relating to securing regulatory approvals and obtaining patent rights, regulatory changes, competitive and technological developments in the market, regulatory decisions, and any commercialization expenses related to any product sales, marketing, manufacturing and distribution. An unforeseen change in these factors, or others, might increase our need for additional capital.

While we are currently well capitalized, we could seek additional financing from the sale and issuance of equity or equity-linked or debt securities in the future, and we cannot predict that financing will be available when and as we need financing or that, if available, the financing terms will be commercially reasonable. If we are unable to obtain additional financing when and if we require it, or on commercially reasonable terms, this would have a material adverse effect on our business and results of operations.

If we are able to consummate such financings, the trading price of our common stock could be adversely affected and/or the terms of such financings may adversely affect the interests of our existing stockholders. To the extent we issue additional equity securities or convertible securities, our existing stockholders could experience substantial dilution in their economic and voting rights. Additional financing, if available, 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. We could also be required to seek funds through arrangements with collaborators 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.

Further, we may also enter into relationships with pharmaceutical or biotechnology companies to perform research and development with respect to our technologies, research programs, conduct clinical trials or market our product candidates. Other than pre-clinical collaborations with academic or research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD, we currently do not have a strategic relationship with a third party to perform research or development using our technologies or assist us in funding the continued development and commercialization of any of our programs or product candidates. If we were to have such a strategic relationship, such third party may require us to issue equity to such third party, relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates, or to grant licenses on terms that may not be favorable to us.

Our indebtedness resulting from our Amended and Restated Credit and Security Agreement and new Revolving Credit Agreement and security agreement with MidCap could adversely affect our financial condition or restrict our future operations.

On July 18, 2017, we entered into (i) an the Amended and Restated Credit and Security Agreement with MidCap that provides a term loan of $60.0 million, (ii) the Revolving Credit Agreement that provides a revolving loan commitment of $40.0 million (which may be increased by an additional tranche of $20.0 million), (iii) an amendment to the pledge agreement related to the Amended and Restated Credit and Security Agreement and (iv) a pledge agreement related to the Revolving Credit Agreement. Our agreements with MidCap create limitations on us, including:

- requiring us to maintain pledge cash and certain other assets in favor of MidCap during the term of the agreements;
- limiting our flexibility in planning for, or reacting to, changes in our business and our industry;
- placing us at a competitive disadvantage compared to our competitors who have less debt or competitors with comparable debt at more favorable interest rates;
- limiting our ability to borrow additional amounts for working capital, capital expenditures, research and development efforts, acquisitions, debt service requirements, execution of our business strategy and other purposes; and
- resulting in an acceleration of the maturity of such term loans upon the occurrence of a material adverse change or another default under the agreements with MidCap.

Any of these factors could materially and adversely affect our business, financial condition and results of operations.

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.

Comprehensive tax reform in the United States could adversely affect our business and financial condition.

The Tax Cuts and Jobs Act (the "TJCA") was enacted on December 22, 2017 in the United States. The TJCA 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.

Notwithstanding the reduction in the corporate income tax rate, the overall impact of the TJCA is uncertain, and our business and financial condition could be adversely affected. We are still in the process of evaluating the TJCA and do not know the full effect it will have on our business, including our consolidated financial statements. The TJCA is complex and far-reaching and we cannot predict with certainty the impact its enactment will have on us. Moreover, that effect, whether adverse or favorable, may not become evident for some period of time. Further, we urge stockholders to consult with their legal and tax advisors with respect to the Tax Reform Act and the potential tax consequences of investing in our common stock.

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.

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 related 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.

If we are unable to effectively manage our growth, execute our business strategy and implement compliance controls and systems, the trading price of our common stock could decline. Any failure to establish and maintain effective internal control over financial reporting could adversely affect investor confidence in our reported financial information.

We anticipate continued growth in our business operations due, in part, to the commercialization of EXONDYS 51. This future growth could create a strain on our organizational, administrative and operational infrastructure. 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 build or maintain the management and human resources and infrastructure necessary

to support the growth of our business. The time and resources required to implement systems and infrastructure that may be needed to support our growth is uncertain, and failure to complete implementation in a timely and efficient manner could adversely affect our operations.

We may engage in future acquisitions or collaborations with other entities that increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We actively evaluate various strategic transactions on an ongoing basis, including licensing or acquiring complementary products, technologies or businesses. Potential acquisitions or collaborations with other entities may entail numerous risks, including increased operating expenses and cash requirements, assimilation of operations and products, retention of key employees, diversion of our management's attention and uncertainties in our ability to maintain key business relationships of the acquired entities. In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense.

Our success, competitive position and future revenue depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies, product and product candidates, 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.

We currently hold various issued patents and exclusive rights to issued patents and own and have licenses to various patent applications, in each case in the U.S. as well as other countries. We anticipate filing additional patent applications both in the U.S. and in other countries. The patent process, however, is subject to numerous risks and uncertainties, and we can provide no assurance that we will be successful in obtaining and defending patents or in avoiding infringement of the rights of others. Even when our patent claims are allowed, the claims may not issue, or in the event of issuance, may not be sufficient to protect the technology owned by or licensed to us or our collaborators. Even if our patents and patent applications do provide our product, product candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such product and product candidates or platform technology due to patent positions held by one or more third parties.

We may not be able to obtain and maintain patent protection for our product or product candidates necessary to prevent competitors from commercializing competing product candidates. Our patent rights might be challenged, invalidated, circumvented or otherwise not provide any competitive advantage, and we might not be successful in challenging the patent rights of our competitors through litigation or administrative proceedings. Additionally, 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. For example, in July 2017, we and The University of Western Australia on the one hand, and the BioMarin Parties and AZL on the other hand, executed a Settlement Agreement pursuant to which all existing efforts pursuing ongoing litigation, opposition and other administrative proceedings would be stopped as between the Settlement Parties and the Settlement Parties would cooperate to withdraw the Actions before the European Patent Office (except for actions involving third parties), the United States Patent and Trademark Office ("USPTO"), the U.S. Court of Appeals for the Federal Circuit and the High Court of Justice of England and Wales, except for the cross-appeal of the Interlocutory Decision of the Opposition Division dated April 15, 2013 of the European Patent Office of EP 1619249B1 in which we withdrew our appeal and the BioMarin Parties and AZL will continue with its appeal, with us having the right to provide input on the appeal. Any adverse rulings on the appeal, or any of the Actions that continue irrespective of the settlement, could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. Defending our patent positions may continue to require significant financial resources and could negatively impact other Company objectives. In addition, the expected benefits and opportunities related to the Settlement Agreement and the License Agreement may not be realized or may take longer to realize than expected due to challenges and uncertainties regarding the sales of EXONDYS 51, the research and development of future exon-skipping products, BioMarin's retained rights to convert the exclusive patent license under the Settlement Agreement to a co-exclusive license, BioMarin continuing certain oppositions and appeals, and patent oppositions that have been filed by other third parties, and patent oppositions and other patent challenges that may be filed by third parties in the future.

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. 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. In addition, 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.

The DMD patent landscape is continually evolving, and we may be able to assert that certain activities engaged in by third parties infringe on 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 or license and rely on for exclusivity for our product candidates may be challenged. In the U.S., our patents may be challenged in an Inter Partes Review proceeding or other related proceeding. In other countries, other procedures are available for a third party to challenge the validity of our patent rights. For instance, we have rights to European Patent No. 2206781, which protects golodirsen. This patent was opposed at the European Patent Office. On December 19, 2017, the Opposition Division issued a Decision ordering the revocation of this patent. We have appealed this Decision. Other patents we have rights to from BioMarin are involved in third party opposition proceedings in Europe, as described above in the section *"Patents and Proprietary Rights."* In addition, a patent in Japan that we have rights to from BioMarin and protects golodirsen is in the preliminary stage of an opposition proceeding. These patents that we are defending in third party opposition proceedings, however, are not expected to be the sole basis for exclusivity for our product candidates, if at all, in view of their standard expiration dates.

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. Additionally, jurisdictions other than the U.S. might have less restrictive patent laws than the U.S., giving foreign competitors the ability to exploit these laws to create, develop and market competing products. 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.

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, but many of the substantive changes to patent law associated with the Leahy-Smith Act have only recently become effective. Accordingly, 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 PTAB seeking to challenge the validity of some or all of the claims in any of our patents through an *Inter Partes Review* ("IPR") or other post-grant proceeding. Should the PTAB institute an IPR (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.

The full impact of several recent U.S. Supreme Court decisions relating to patent law is not yet known. For example, on March 20, 2012, in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc* ., the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. The decision appears to impact diagnostics patents that merely apply a law of nature via a series of routine steps and it has created uncertainty around the ability to patent certain biomarker-related method claims. Additionally, on June 13, 2013, in *Association for Molecular Pathology v. Myriad Genetics, Inc.*, the Court held that claims to isolated genomic DNA are not patentable, but claims to complementary DNA molecules were held to be valid. The effect of the decision on patents for other isolated natural products is uncertain and, as with the Leahy-Smith Act, these decisions 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.

Our business prospects will be impaired if third parties successfully assert that EXONDYS 51 or our product candidates or technologies infringe proprietary rights of such third parties.

Our competitors may make significant investments in competing technologies, and might have or obtain patents that limit, interfere with or eliminate our ability to make, use and sell EXONDYS 51 or our product candidates in important commercial markets.

If EXONDYS 51 or our product candidates or technologies 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;
- redesign EXONDYS 51, 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 substantially harm our potential earnings, financial condition and operations. The DMD patent landscape 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 EXONDYS 51 or our product candidates infringe on the intellectual property rights of such parties. Similarly, we may be able to assert that certain activities engaged in by these parties infringe on 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. We also cannot be certain that other third parties will not assert patent infringement in the future with respect to any of our development programs.

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 related to antisense technology and other technologies, or that are developing alternative approaches to or therapeutics for the disease indications on which we are focused. Some of these competitors are developing or testing product candidates that now, or may in the future, compete directly with EXONDYS 51 or our product candidates. For example, we believe that companies including Alnylam Pharmaceuticals, Inc., Ionis Pharmaceuticals, Inc. (formerly Isis Pharmaceuticals, Inc.), Roche Innovation Center Copenhagen (formerly Santaris Pharma A/S), Wave Life Sciences, Daiichi Sankyo and Nippon share a focus on RNA-targeted drug discovery and development. Competitors with respect to EXONDYS 51 or our product candidates include Nippon Shinyaku, Daiichi Sankyo, Wave, Solid Shire plc; and other companies such as PTC have also been working on DMD programs. Additionally, several companies and institutions have entered into collaborations or other agreements for the development of product candidates, including mRNA, gene (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, Biogen Inc., Ionis, Alexion Pharmaceuticals, Inc., Sanofi, Shire, Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit, Akashi, Catabasis, Capricor Therapeutics, Oxford University, Exonics Therapeutics, and Editas Medicine. 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.

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

It is possible that our competitors will succeed in developing technologies that limit the market size for EXONDYS 51 or our product candidates, impact the regulatory approval process for our product candidates that are more effective than our product candidates or that 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 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 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 EXONDYS 51 and future 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 limited general commercial liability insurance coverage for our clinical trials. We intend to expand our insurance coverage to include the sale of commercial products in connection with the FDA's approval of EXONDYS 51. 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.

Our operations involve the use of hazardous materials, and we must comply with environmental laws, which can be expensive, and may affect our business and operating results.

Our research and development activities involve the use of hazardous materials, including organic and inorganic solvents and reagents. Accordingly, we are subject to federal, state and local laws and regulations governing the use, storage, handling, manufacturing, exposure to and disposal of these hazardous materials. In addition, we are subject to environmental, health and workplace safety laws and regulations, including those governing laboratory procedures, exposure to blood-borne pathogens and the handling of bio-hazardous materials. 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.

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 EXONDYS 51 patients, 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.

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 twenty four months, our stock has increased as much as 74% in a single day or decreased as much as 55% 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 EXONDYS 51 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 EXONDYS 51 or our product candidates or entry into strategic relationships on terms that are not deemed to be favorable to our Company;
- 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 such as the stockholder lawsuits 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. 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.

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.

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 Centers for Medicare & Medicaid Services (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;
- introduction of new products by others that render our product obsolete or noncompetitive;
- the ability to maintain selling prices and gross margin on our product;
- increases in the cost of raw materials contained within our product;
- 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; 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.

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 in the future. 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.

As of December 31, 2017, there were approximately 64.8 million shares of common stock outstanding and outstanding awards to purchase 9.4 million shares of common stock under various incentive stock plans. Additionally, as of December 31, 2017, there were approximately 2.3 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, approximately 0.2 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and approximately 0.6 million shares of common stock available for issuance under our 2014 Employment Commencement Incentive Plan. We may issue additional common stock and warrants from time to time to finance our operations. We may also issue additional shares to fund potential acquisitions or in connection with additional stock options or other equity awards granted to our employees, officers, directors and consultants under our Amended and Restated 2011 Equity Incentive Plan, our 2013 Employee Stock Purchase Plan or our 2014 Employment Commencement Incentive Plan. 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 options may have a dilutive impact on other stockholders and could have a material negative effect on the market price of our common stock.

Risks Related to Our Convertible Senior Notes

Servicing our 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 million aggregate principal amount of Notes. Our ability to make scheduled payments of the principal of, to pay interest on, or to refinance our indebtedness, including 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 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 our 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 our 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 1B. Unresolved Staff Comments.

None.

Item 2. Properties.

A description of the facilities we own and/or occupy is included in the following table. We believe that our current facilities in Cambridge, Massachusetts and Andover, Massachusetts are suitable and will provide sufficient capacity to meet the projected needs of our business for the next 12 months. Except as noted below, all of our properties are currently being used in the operation of our business.

Location of Property	Square Footage	Lease Expiration Date	Purpose	Other Information
215 First Street, Cambridge, MA	88,459	January – February 2021	Laboratory and office space	Corporate headquarters
100 Federal Street, Andover, MA	60,000	N/A – facility is owned	Manufacturing and office space	Primarily manufacturing space
4575 SW Research Way, Suite 200, Corvallis, OR	53,468	December 2020	Laboratory and office space	Primarily lab space*
1749 SW Airport Avenue, Corvallis, OR	N/A	N/A	N/A	**
33930C Eastgate Circle, Corvallis, OR	1,500	April 2019	Laboratory and office space	Primarily lab space

* As a result of the plan to close the Corvallis site, this property has been vacated as of December 31, 2017.

** As of the date of this report, this property has been sold to an unrelated third party.

Item 3. Legal Proceedings.

For material legal proceedings, please read *Note 19, Commitments and Contingencies - Litigation* to our consolidated financial statements included in this report.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

The following table sets forth the high and low intraday sales prices as reported by The NASDAQ Global Select Market for each quarterly period in the two most recent years:

	High	Low
Year Ended December 31, 2017		
First Quarter	$ 39.36	$ 26.26
Second Quarter	$ 39.34	$ 28.14
Third Quarter	$ 47.96	$ 32.80
Fourth Quarter	$ 57.57	$ 46.07
Year Ended December 31, 2016		
First Quarter	$ 38.80	$ 10.20
Second Quarter	$ 24.60	$ 8.00
Third Quarter	$ 63.73	$ 19.06
Fourth Quarter	$ 63.03	$ 26.66

Holders

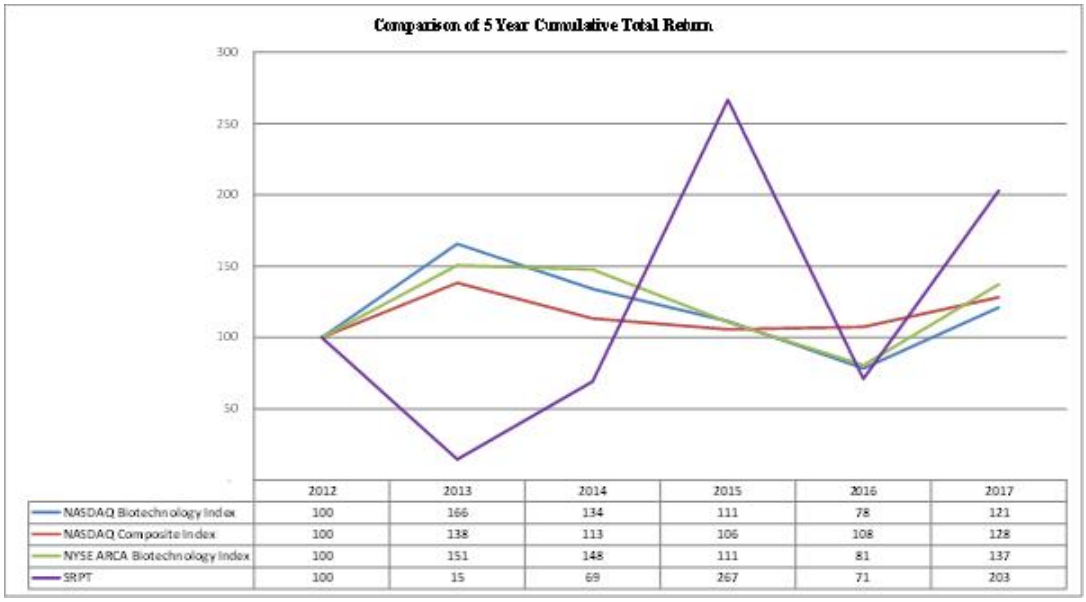
As of February 26, 2018, we had 222 stockholders of record of our common stock.

Dividends

We did not declare or pay cash dividends on our common stock in 2017, 2016 or 2015. We currently expect to retain future earnings, if any, to finance the operation and expansion of our business, and we do not anticipate paying any cash dividends in the foreseeable future. Any future determination related to our dividend policy will be made at the discretion of our board of directors.

Performance Graph

The following graph compares the performance of our Common Stock for the periods indicated with the performance of the NASDAQ Composite Index, NASDAQ Biotechnology Index and the NYSE ARCA Biotechnology Index. This graph assumes an investment of $100 on December 31, 2011 in each of our common stock, the NASDAQ Composite Index, NASDAQ Biotechnology Index and the NYSE ARCA Biotechnology Index, and assumes reinvestment of dividends, if any. The stock price performance shown on the graph below is not necessarily indicative of future stock price performance. This graph is not "soliciting material," is not deemed "filed" with the SEC and is not to be incorporated by reference into any of our filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof and irrespective of any general incorporation language in any such filing.



	2012	2013	2014	2015	2016	2017
NASDAQ Biotechnology Index	100	166	134	111	78	121
NASDAQ Composite Index	100	138	113	106	108	128
NYSE ARCA Biotechnology Index	100	151	148	111	81	137
SRPT	100	15	69	267	71	203

Recent Sales of Unregistered Securities.

None.

Purchases of Equity Securities by the Issuer and Affiliated Purchasers.

None.

Item 6. Selected Financial Data.

The following selected financial data are derived from our consolidated financial statements and should be read in conjunction with, and is qualified in its entirety by, *Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations*, and *Item 8, Financial Statements and Supplementary Data.*

	For the Year Ended December 31,				
	2017	2016	2015	2014	2013
	(in thousands, except per share amounts)				
Operations data:					
Revenues	$ 154,584	$ 5,421	$ 1,253	$ 9,757	$ 14,219
Cost of sales (excluding amortization of in-licensed rights)	7,353	101	—	—	—
Research and development	166,707	188,272	146,394	94,231	72,909
Selling, general and administrative	122,682	83,749	75,043	49,315	31,594
Settlement and license charges	28,427	—	—	—	—
Amortization of in-licensed rights	1,053	29	—	—	—
Operating loss	(171,638)	(266,730)	(220,184)	(133,789)	(90,284)
Interest (expense) income and other, net	(1,990)	(535)	154	779	326
Gain from sale of Priority Review Voucher	125,000	—	—	—	—
Loss on change in warrant valuation	—	—	—	(2,779)	(22,027)
Loss before income tax expense	(48,628)	(267,265)	(220,030)	(135,789)	(111,985)
Income tax expense	2,060	—	—	—	—
Net loss	$ (50,688)	$ (267,265)	$ (220,030)	$ (135,789)	$ (111,985)
Net loss per share—basic and diluted	$ (0.86)	$ (5.49)	$ (5.20)	$ (3.39)	$ (3.31)
Balance sheet data:					
Cash and cash equivalents	$ 599,691	$ 122,420	$ 80,304	$ 73,551	$ 256,965
Marketable securities	489,349	195,425	112,189	136,793	—
Working capital	1,140,312	298,054	162,249	210,929	234,840
Total assets	1,307,964	424,104	273,782	295,033	291,569
Stockholders' equity	789,217	336,691	190,347	247,653	247,192

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

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K. This discussion contains forward-looking statements that involve risks and uncertainties. Please review our legend titled "Forward-Looking Information" at the beginning of this Annual Report on Form 10-K which is incorporated herein by reference. Our actual results could differ materially from those discussed below. Factors that could cause or contribute to such differences include, but are not limited to, those identified below, and those discussed in the section titled "Risk Factors" included elsewhere in this Annual Report on Form 10-K. Throughout this discussion, unless the context specifies or implies otherwise, the terms "Sarepta", "we", "us" and "our" refer to Sarepta Therapeutics, Inc. and its subsidiaries.

Overview

We are a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic medicine approaches for the treatment of rare neuromuscular diseases.

Applying our proprietary, highly-differentiated and innovative RNA-targeted platform technologies, we are able to develop candidate therapies for a broad range of diseases and disorders.

Our first commercial product in the U.S., EXONDYS 51, was granted accelerated approval by the 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. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

A summary description of our product and main product candidates is as follows:

- *EXONDYS 51*, our first product, uses our PMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS51 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.

  We are in the process of conducting, starting or planning various EXONDYS 51 clinical trials, including studies that are required to comply with regulatory NDA and/or MAA filing requirements as well as studies we need to conduct to comply with our post-marketing FDA requirements/commitments to verify and describe clinical benefit.

- *Golodirsen*, one of our main product candidates, uses our PMO chemistry and exon-skipping technology to skip exon 53 of the DMD gene. Golodirsen is designed to bind to exon 53 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 53 skipping. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

  Golodirsen, an exon 53-skipping product candidate, is currently in the clinic as part of a Phase 1/2 study. Part I has been completed and Part II, an open label portion of this study, is ongoing (Study 4053-101). In September 2017, we announced positive results of an analysis that included biopsies of the bicep muscle at baseline and on-treatment at the Part II, Week 48 time point. The study results demonstrated statistical significance on all primary and secondary biological endpoints.

- *Casimersen*, one of our main product candidates, 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. Exon skipping is intended to promote the production of an internally truncated but functional dystrophin protein.

  We are enrolling and dosing patients in ESSENCE (Study 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.

In addition, pursuant to an ongoing Sarepta-sponsored Phase 1/2 clinical trial studying casimersen (Study 4045-101), we have completed a dose titration portion (Phase 1) and are currently conducting the open-label portion of the study (Phase 2).

- *SRP-5051*, one of our main product candidates, 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 received clearance from the FDA and commenced a first in human, single ascending dose, study for our PPMO for the treatment of DMD in patients who are amenable to exon 51 skipping (SRP-5051). We expect to have data regarding safety and future dosing for SRP-5051 in the second half of 2018. In addition to SRP-5051, our 2018 plans currently include IND-enabling pre-clinical work on 5 additional PPMOs.

In addition to advancing our exon-skipping product candidates for DMD, we are working with several strategic partners under various agreements to research and develop multiple treatment approaches to DMD. These strategic partners include:

- Nationwide Children's Hospital, with whom we are collaborating on the advancement of (1) their micro-dystrophin gene therapy program under a research and exclusive license option agreement and (2) their Galgt2 gene therapy program under an exclusive license agreement;
- Genethon, with whom we are collaborating on the advancement of their micro-dystrophin gene therapy program under a sponsored research and exclusive license option agreement;
- Duke University, with whom we are collaborating on the advancement of gene editing CRISPR/Cas9 technology for muscular dystrophy under a sponsored research and exclusive license option agreement that grants us rights to certain of Duke University's intellectual property for CRISPR/Cas9; and
- Summit, with whom we are collaborating under an exclusive License and Collaboration Agreement that grants us exclusive rights to Summit's utrophin modulator pipeline, including ezutromid, in Europe, Turkey and the Commonwealth of Independent States and an option to acquire rights in Latin America.

Our RNA-targeted technologies work at the most fundamental level of biology and potentially could have a meaningful impact across a broad range of human diseases and disorders. Our lead program focuses on the development of disease-modifying therapeutic candidates for DMD, a rare genetic muscle-wasting disease caused by the absence of dystrophin, a protein necessary for muscle function. The basis of our novel RNA-targeted therapeutics is the PMO.

PMO-based compounds are highly resistant to degradation by enzymes, potentially enabling robust and sustained biological activity. In contrast to other RNA-targeted therapeutics, which are usually designed to down-regulate protein expression, our technologies are designed to selectively up-regulate or down-regulate protein expression, and more importantly, create novel proteins. PMO-based compounds have demonstrated inhibition of mRNA translation and alteration of pre-mRNA splicing. PMO-based compounds have the potential to reduce off-target effects, such as the immune stimulation often observed with ribose-based RNA technologies. We believe that our highly differentiated, novel, proprietary and innovative RNA-targeted PMO-based platforms may represent a significant improvement over other RNA-targeted technologies. In addition, PMO-based compounds are highly adaptable molecules: with minor structural modifications, they can potentially be rapidly designed to target specific tissues, genetic sequences, or pathogens, and therefore, we believe they could potentially be applied to treat a broad spectrum of diseases.

Our next generation PMO-based chemistries include PPMO, PMO-X® and PMO*plus*®. PPMO features covalent attachment of a cell-penetrating peptide to a PMO with the goal of enhanced delivery into the cell. In pre-clinical research, our proprietary class of PPMO compounds demonstrated an increase in dystrophin production and a more durable response compared to PMO. In addition, PPMO treatment in non-human primates is well tolerated and results in high levels of exon-skipping in skeletal, cardiac and smooth muscle tissues. Preclinical trials also indicate that PPMOs may require less frequent dosing than PMO, and that PPMOs could potentially be tailored to reach other organs beyond muscle.

We have developed proprietary state-of-the-art manufacturing and techniques that allow synthesis and purification of our 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 products and optimizing manufacturing for PPMO. We have entered into certain manufacturing and supply arrangements with third-party suppliers which will in part utilize these techniques to support production of certain of our product candidates and their components. In 2017, we opened a facility in Andover, Massachusetts, which significantly enhances our research and development manufacturing capabilities. However, we currently do not have own internal manufacturing capabilities to produce our product and product candidates for commercial and/or clinical use.

As of December 31, 2017, we had approximately $1,089.9 million of cash, cash equivalents and investments, consisting of $599.7 million of cash and cash equivalents, $479.4 million of short-term investments, $10.0 million of long-term investments and $0.8 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. We may never achieve significant revenue or profitable operations.

Critical Accounting Policies and Estimates

The discussion and analysis of our financial condition and results of operations are based upon our consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The preparation of our consolidated financial statements in accordance with accounting principles generally accepted in the U.S. 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 consolidated financial statements will be affected. Although we believe that our judgments and estimates are appropriate, actual results may differ from these estimates. We believe the following accounting policies to be most critical to the judgments and estimates used in the preparation of our consolidated financial statements:

- revenue recognition;
- Inventory;
- research and development expense;
- stock-based compensation; and
- income tax.

Revenue Recognition

Revenues are recognized when persuasive evidence of an arrangement exists, delivery has occurred and title of the product and associated risk of loss has passed to the customer, the price is fixed or determinable and collection from the customer has been reasonably assured.

Product revenue, net

We are currently approved to sell EXONDYS 51 in the U.S. We distribute our product principally through a limited number of specialty distributor and specialty pharmacies (collectively, “U.S. Customers”). Title and risk of loss transfers upon delivery of EXONDYS 51 to our U.S. Customers’ facilities. Our U.S. Customers subsequently resell our products to patients and health care providers. During 2017, we commenced shipment of eteplirsen to a distributor in the E.U. under our MAP, and title and risk of loss transfers to the distributor upon shipment of eteplirsen to their customers. Additionally, we started shipment of eteplirsen to the distributors in Israel and Middle East on a named patient basis, and title and risk of loss transfers to the distributors when their first carriers pick up the drug products. Product revenue generated from these programs were immaterial in 2017. We provide no right of return to any customers except in cases of shipping error or product defect.

Product revenues are recorded net of estimated rebates and discounts, Public Health Service ("PHS") chargebacks, prompt payment discounts, co-pay assistance and distribution fees. We establish reserves for Medicaid rebates, PHS chargebacks, prompt payment discounts, co-pay assistance and distribution fees. These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Reserves established for these discounts and allowances are classified as reductions of accounts receivable (if the amount is payable to our customer) or a liability (if the amount is payable to a party other than our customer). Our estimates take into consideration current contractual and statutory requirements. If actual results vary, we may need to adjust these estimates, which could have an effect on earnings in the period of the adjustment. In addition to the discounts and rebates described above and classified as a reduction of revenue, we also maintain certain customer service contracts with customers in the distribution channel that provide us with inventory management, data and distribution services, which are generally accounted for as a reduction of revenue. To the extent we can demonstrate a separable benefit and fair value for these services, we classify these payments within selling, general and administrative expenses.

Revenue from research contracts and other grants

Our contracts with the U.S. government are generally cost plus contracts providing for reimbursed costs which include overhead, general and administrative costs and a target fee. We recognize revenue from U.S. government research contracts during the period in which the related expenses are incurred and present such revenues and related expenses on a gross basis in the consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments. As of December 31, 2014, we had completed all development activities of our contracts with the U.S. government. The majority of the revenue under our U.S. government contracts was recognized as of December 31, 2015 and only revenue for contract finalization, if any, is expected in the future.

Deferred revenue

We defer recognition of non-refundable up-front fees if we have continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if we have continuing involvement through research and development services that are required because of our know-how or because the services can only be performed by us, such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2017, we had deferred revenue of $3.3 million, which primarily represents up-front fees we may recognize as revenue upon settlement of certain obligations.

Please read *Note 7, Accounts Receivable and Reserves for Product Sales* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of revenue recognition.

Inventory

Inventories are stated at the lower of cost or estimated net realizable value, on a first-in, first-out basis. We capitalize inventory costs associated with our products upon regulatory approval. Prior to regulatory approval, we expense costs incurred to manufacture our drug products that could potentially be available to support the commercial launch of our products. Beginning in the third quarter of 2016 following the FDA approval of EXONDYS 51, we began to capitalize inventory costs associated with EXONDYS 51 when it was determined that the inventory had a probable future economic benefit. Additionally, we periodically analyze our inventory levels, and write down inventory that has become obsolete, inventory that has a cost basis in excess of its estimated net realizable value and inventory in excess of expected sales requirements as cost of sales. The determination of whether inventory costs will be realizable requires estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required.

Research and Development Expenses

All research and development expenses are expensed as incurred and are comprised of:

- employee-related expenses, including salaries, bonuses, benefits, travel and stock-based compensation expense;
- fees and expenses incurred under agreements with contract research organizations, investigative sites and other entities in connection with the conduct of clinical trials and preclinical trials and related services, such as data management, laboratory and biostatistics services;

- the cost of acquiring, developing and manufacturing APIs for product candidates that have not received regulatory approval;
- fees and costs related to regulatory filings and activities;
- up-front fees and milestones paid to third parties in connection with technologies which have not reached technological feasibility and do not have an alternative future use are expensed when incurred;
- facilities, depreciation and other expenses, including rent, utilities, maintenance of facilities, insurance and other supplies.

When third-party service providers' billing terms do not coincide with our period-end, we are required to make estimates of our obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of our drug candidates, incurred in a given accounting period and record accruals at the end of the period. We base our estimates on our knowledge of the research and development programs, services performed for the period and past history, where applicable.

Stock Compensation Expense

We use the fair value method to determine stock-based compensation expense. To determine the fair value of stock-based awards on the date of grant, we use the Black-Scholes-Merton option-pricing model. The Black-Scholes-Merton option-pricing model requires the use of subjective assumptions which include the award's expected term and the price volatility of the underlying stock. We recognize the fair value of the portion of the awards expected to vest as expense over the requisite vesting periods on a straight-line basis for the entire award. Stock awards granted to employees vest over a four-year period and have a ten-year term. Forfeitures are estimated at the time of grant and revised, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

The assumptions used in calculating the fair value of stock-based compensation expense represent management's best estimates, but these estimates involve inherent uncertainties and the application of management judgment. As a result, if factors change and we use different assumptions, our stock-based compensation expense could be materially different in the future. Please read *Note 14, Stock-Based Compensation* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of stock-based compensation.

Income Tax

The Company follows the asset and liability method of accounting for income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. It is the intention of the Company to reinvest the earnings of its non-U.S. subsidiaries in those operations and not to repatriate the earnings to the U.S. Accordingly, the Company does not provide for deferred taxes on the excess of the financial reporting over the tax basis in its investments in foreign subsidiaries as they are considered permanent in duration. To date, the Company has not had any earnings in its non-U.S. subsidiaries.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered and settled. A valuation allowance is recorded to reduce the net deferred tax asset to zero because it is more likely than not that the net deferred tax asset will not be realized. The Company recognizes the effect of income tax positions only if those positions are more likely than not of being sustained upon an examination.

The TJCA was enacted on December 22, 2017. The TJCA reduces the US federal corporate tax rate from 35% to 21%, requires companies to pay a one-time transition tax on earnings of certain foreign subsidiaries that were previously tax deferred and creates new taxes on certain foreign sourced earnings. On December 22, 2017, the Securities and Exchange Commission issued guidance under Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act ("SAB 118"). SAB118 directs taxpayers to consider the impact of the U.S. legislation as "provisional" when it does not have the necessary information available to prepare or analyze (including computations) in reasonable detail to complete its accounting for the change in tax law.

At December 31, 2017, we made reasonable estimates of the effects on its existing U.S. deferred tax balances and the corresponding valuation allowance. For the year ended December 31, 2017, we recognized no transition tax, have remeasured deferred taxes, and our reassessment of permanently reinvested earnings, uncertain tax positions and valuation allowances. These estimates may be impacted by the need for further analysis and future clarification and guidance regarding available tax accounting methods and elections, earnings and profits computations, and state tax conformity to federal tax changes.

Please read *Note 2, Summary Of Significant Accounting Policies and Recent Accounting Pronouncements* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K for a further discussion of our critical accounting policies and estimates.

Results of Operations for the years ended December 31, 2017, 2016 and 2015

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

	For the Year Ended December 31			
	2017	2016	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 154,584	$ 5,421	$ 149,163	NM*
Total revenues	154,584	5,421	149,163	NM*
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	7,353	101	7,252	NM*
Research and development	166,707	188,272	(21,565)	(11)%
Selling, general and administrative	122,682	83,749	38,933	46%
Settlement and license charges	28,427	—	28,427	NA
Amortization of in-licensed rights	1,053	29	1,024	NM*
Total cost and expenses	326,222	272,151	54,071	20%
Operating loss	(171,638)	(266,730)	95,092	(36)%
Other (loss) income:				
Interest expense and other, net	(1,990)	(535)	(1,455)	272%
Gain from sale of Priority Review Voucher	125,000	—	125,000	NA
Loss before income tax expense	(48,628)	(267,265)	218,637	(82)%
Income tax expense	2,060	—	2,060	NA
Net loss	$ (50,688)	$ (267,265)	$ 216,577	(81)%
Net loss per share — basic and diluted	$ (0.86)	$ (5.49)	$ 4.63	(84)%

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands, except per share amounts)		$	%
Revenues:				
Product, net	$ 5,421	$ —	$ 5,421	NA
Revenue from research contracts and other grants	—	1,253	(1,253)	(100)%
Total revenues	5,421	1,253	4,168	333%
Cost and expenses:				
Cost of sales (excluding amortization of in-licensed rights)	101	—	101	NA
Amortization of in-licensed rights	29	—	29	NA
Research and development	188,272	146,394	41,878	29%
Selling, general and administrative	83,749	75,043	8,706	12%
Total cost and expenses	272,151	221,437	50,714	23%
Operating loss	(266,730)	(220,184)	(46,546)	21%
Other (loss) income:				
Interest (expense) income and other, net	(535)	154	(689)	(447)%
Net loss	$ (267,265)	$ (220,030)	$ (47,235)	21%
Net loss per share — basic and diluted	$ (5.49)	$ (5.20)	$ (0.29)	6%

*NM: not meaningful

Revenues

We record product revenue net of applicable discounts and allowances which include Medicaid rebates, PHS chargebacks, prompt pay discount, co-pay and distribution fees. These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Our estimates take into consideration current contractual and statutory requirements. Actual amounts may ultimately differ from our estimates. If actual results are different from our estimates, we adjust these estimates, which will have an effect on earnings in the period of adjustment.

We recognize revenue from U.S. government research contracts and grants during the period in which the related expenses are incurred and present such revenues and related expenses on a gross basis in the consolidated financial statements. Our government contracts are subject to government audits, which may result in catch-up adjustments. As of December 31, 2014, we had completed all development activities of our contracts with the U.S. government. The majority of the revenue under our U.S. government contracts was recognized as of December 31, 2015 and only revenue for contract finalization, if any, is expected in the future.

Net product revenues for EXONDYS 51 for 2017 increased by $149.2 million compared with 2016. The increase primarily reflects increasing demand for EXONDYS 51 in the U.S.

Net product revenues for EXONDYS 51 was $5.4 million in 2016. These revenues related to sales of EXONDYS 51 following the September 2016 commercial launch of EXONDYS 51 in the U.S. Revenue from research contracts and other grants decreased by $1.3 million in 2016, or 100% compared with 2015, as all of the work related to government grants was completed prior to 2016. Only revenue from contract finalization, if any, is expected to be recognized in future periods.

Cost of Sales

Our cost of sales relates to sales of EXONDYS 51 following its commercial launch in the U.S. Prior to receiving regulatory approval for EXONDYS 51 from the FDA in September 2016, we expensed such manufacturing and material costs as research and development expenses. For EXONDYS 51 sold in 2017 and 2016, 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 EXONDYS 51. The following table summarizes the components of our cost of sales for the periods indicated:

	For the Year Ended December 31			
	2017	2016	Change	Change
	(in thousands)		$	%
Royalty payments to BioMarin	$ 4,719	$ —	$ 4,719	NA
Inventory costs related to release testing	1,387	29	1,358	NM*
Inventory costs related to EXONDYS 51 sold	414	26	388	NM*
Other inventory costs	833	46	787	NM*
Total cost of sales	$ 7,353	$ 101	$ 7,252	NM*

*NM: not meaningful

The cost of sales for 2017 increased $7.3 million compared with 2016. The increase primarily reflects royalty payments to BioMarin as a result of the execution of the settlement and license agreements with BioMarin in July 2017 as well as increasing demand for EXONDYS 51 during 2017. If product related costs had not previously been expensed as research and development expenses prior to receiving FDA approval, the inventory costs related EXONDYS 51 sold in 2017 and 2016 would have been approximately $8.6 million and $0.5 million, respectively.

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 preclinical 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 which 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 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 Year Ended December 31			
	2017	2016	Change	Change
	(in thousands)		$	%
Eteplirsen	$ 40,161	$ 65,454	$ (25,293)	(39)%
Casimersen	19,867	9,562	10,305	108%
Golodirsen	19,626	11,847	7,779	66%
SRP-5051	8,145	570	7,575	NM*
Other projects	1,200	678	522	77%
Up-front and milestone payments	22,000	48,035	(26,035)	(54)%
Internal research and development expenses	55,708	52,126	3,582	7%
Total research and development expenses	$ 166,707	$ 188,272	$ (21,565)	(11)%

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands)		$	%
Eteplirsen	$ 65,454	$ 72,147	$ (6,693)	(9)%
Golodirsen	11,847	5,583	6,264	112%
Casimersen	9,562	6,649	2,913	44%
SRP-5051	570	—	570	NA
Other projects	678	2,178	(930)	(43)%
Up-front and milestone payments	48,035	165	47,870	NM*
Internal research and development expenses	52,126	59,672	(7,546)	(13)%
Total research and development expenses	$ 188,272	$ 146,394	$ 41,878	29%

*NM: not meaningful

We have revised the presentation as well as the caption of certain items in research and development by project table to conform to the current period presentation. “SRP-5051” of $0.6 million was reclassified from “other projects” and presented separately in the table. This reclassification had no impact on total research and development expenses.

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

	For the Year Ended December 31			
	2017	2016	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 75,728	$ 82,077	$ (6,349)	(8)%
Compensation and other personnel expenses	25,607	21,322	4,285	20%
Up-front and milestone payments	22,000	48,035	(26,035)	(54)%
Professional services	10,132	7,537	2,595	34%
Preclinical expenses	9,407	3,415	5,992	175%
Facility-related expenses	8,940	8,095	845	10%
Stock-based compensation	8,542	9,499	(957)	(10)%
Restructuring expenses	188	2,013	(1,825)	(91)%
Other	6,163	6,279	(116)	(2)%
Total research and development expenses	$ 166,707	$ 188,272	$ (21,565)	(11)%

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands)		$	%
Clinical and manufacturing expenses	$ 82,077	$ 80,977	$ 1,100	1%
Up-front and milestone payments	48,035	165	47,870	NM
Compensation and other personnel expenses	21,322	25,746	(4,424)	(17)%
Stock-based compensation	9,499	10,403	(904)	(9)%
Facility-related expenses	8,095	9,919	(1,824)	(18)%
Professional services	7,537	8,329	(792)	(10)%
Preclinical expenses	3,415	3,948	(533)	(14)%
Restructuring expenses	2,013	—	2,013	NA
Research and other	6,279	6,907	(628)	(9)%
Total research and development expenses	$ 188,272	$ 146,394	$ 41,878	29%

*NM: not meaningful

Research and development expenses for 2017 decreased by $21.6 million, or 11%, compared with 2016. The up-front and milestone payments decreased by $26.0 million. In 2017, we made a $22.0 million payment to Summit as a result of achieving the milestone of the last patient being dosed in the safety arm cohort to the PhaseOut DMD study. In 2016, we made $40.0 million and $7.0 million up-front payments to Summit and UWA, respectively, as a result of the execution of their respective license agreements in 2016. Additionally, clinical and manufacturing expenses decreased by $6.3 million because of the capitalization of inventory following the approval of EXONDYS 51 by the FDA partially offset by increased patient enrollment in our on-going late stage clinical trials. Further, the year over year reduction in research and development expenses was also driven by decreases of $1.8 million in restructuring expenses as a majority of activities of the restructuring plans implemented in 2016 were completed prior to 2017 and $1.0 million in stock-based compensation. The decreases were partially offset by increases of $6.0 million in preclinical expenses as a result of a ramp-up of preclinical trials in our PPMO platform and other follow-on exons, $4.3 million in compensation and other personnel expenses as a result of a net increase in headcount and $2.6 million in professional services.

Research and development expenses for 2016 increased by $41.9 million, or 29%, compared with 2015. The increase was primarily driven by increases of $47.5 million in up-front and milestone payments related to the Collaboration Agreement with Summit and the Amended and Restated UWA License Agreement and its First Amendment with UWA, $2.0 million in restructuring expenses, and $1.1 million in clinical and manufacturing expenses due to increased patient enrollment in our ongoing clinical trials, partially offset by lower manufacturing expenses because of the capitalization of inventory upon the approval of EXONDYS 51 by the FDA. The increases were partially offset by decreases of $4.4 million in compensation and other personnel expenses, $1.8 million in facility-related expenses and $0.9 million in stock-based compensation primarily driven by decreases in headcount.

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 costs and professional fees for legal, consulting and accounting services.

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

	For the Year Ended December 31			
	2017	2016	Change	Change
	(in thousands)		$	%
Professional services	$ 44,652	$ 19,372	$ 25,280	130%
Compensation and other personnel expenses	36,956	29,807	7,149	24%
Stock-based compensation	19,848	20,463	(615)	(3)%
Facility-related expenses	5,828	4,669	1,159	25%
Former CEO severance expense	3,537	—	3,537	NA
Restructuring expenses	2,832	2,548	284	11%
Other	9,029	6,890	2,139	31%
Total selling, general and administrative expenses	$ 122,682	$ 83,749	$ 38,933	46%

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands)		$	%
Compensation and other personnel expenses	$ 29,807	$ 17,513	$ 12,294	70%
Stock-based compensation	20,463	12,329	8,134	66%
Professional services	19,372	25,884	(6,512)	(25)%
Facility-related expenses	4,669	2,838	1,831	65%
Restructuring expenses	2,548	—	2,548	NA
Former CEO severance expense	—	9,182	(9,182)	(100)%
Other	6,890	7,297	(411)	(6)%
Total selling, general and administrative expenses	$ 83,749	$ 75,043	$ 8,702	12%

Selling, general and administrative expenses for 2017 increased by $38.9 million, or 46%, compared with 2016. The increase was primarily due to $25.3 million in professional services driven by increased legal fees because of on-going litigations and global commercial expansion, $7.1 million in compensation and other personnel expenses due to net increase in headcount, $3.5 million in estimated severance due to the resignation of our former CEO and $1.2 million in facility-related expenses.

Selling, general and administrative expenses for 2016 increased by $8.7 million, or 12%, compared with 2015. The increase was primarily due to increases of $12.3 million in compensation and other personnel expenses, $8.1 million in stock-based compensation and $1.8 million in facility-related expenses. These increases were primarily driven by increases in commercial headcount. Additionally, we also incurred $2.5 million in restructuring expenses in 2016. The increases were partially offset by decreases of $9.2 million in severance expense related to the resignation of our former CEO in March 2015 and $6.4 million in professional services primarily due to lower litigation activities.

Settlement and License Charges

In July 2017, we and BioMarin executed a license agreement, pursuant to which the BioMarin Parties granted us a royalty-bearing, worldwide license under patent rights and know-how controlled by the BioMarin Parties with respect to the BioMarin Parties' DMD program, which are potentially necessary or useful for the treatment of DMD, to practice and exploit the Licensed Patents and Licensed Know-How in all fields of use and for all purposes, including to develop and commercialize antisense oligonucleotide products that target one or more exons of the dystrophin gene to induce exon skipping, including eteplirsen. In addition, in July 2017, we and UWA on the one hand, and the BioMarin Parties AZL on the other hand, executed a settlement agreement pursuant to which all legal actions in the U.S. and certain legal actions in Europe would be stopped or withdrawn as between the Settlement Parties. Under the terms of the License Agreement and the Settlement Agreement, we agreed to make total up-front payments of $35.0 million upon execution of these agreements, consisting of $20.0 million under the Settlement Agreement and $15.0 million under the License Agreement. Additionally, we may be liable for up to approximately $65.0 million in regulatory and sales milestones for eteplirsen as well as exon 45 and exon 53 skipping product candidates. The BioMarin Parties will also be eligible to receive royalty payments, ranging from 4% - 8%, which will expire in December 2023 in the U.S. and September 2024 in the EU. For the year ended December 31, 2017, we recorded settlement and license charges of $28.4 million.

Amortization of In-licensed Rights

Amortization of in-license rights relate to the two 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 as a result of the settlement and license agreements with BioMarin. Additionally, following the first sale of EXONDYS 51 in September 2016, we recorded an in-licensed right asset of $1.0 million related to a license agreement with UWA. Both in-licensed rights are being amortized on a straight-line basis over the life of the patent from the first commercial sale of EXONDYS 51. For the years ended December 31, 2017 and 2016, we recorded amortization of in-licensed rights of approximately $1.1 million and less than $0.1 million, respectively.

Interest (expense) income and other, net

Interest (expense) income and other, net, primarily consists of interest income on our cash, cash equivalents and investments, interest expense and rental income and loss. Our cash equivalents and investments consist of money market funds, commercial paper, government and government agency debt securities, money market investments and certificates of deposit. Interest expense includes interest accrued on our convertible notes, term loan, revolving line of credit and mortgage loan related to our Corvallis, Oregon property. Rental income and loss is from leasing excess space in some of our facilities.

Interest expense and other, net for 2017 increased by $1.6 million compared with 2016. The increase was primarily driven by an increase in interest expense incurred in connection with the $570.0 million convertible debt offering.

Interest expense and other, net for 2016 was $0.5 million compared to interest income and other, net of $0.2 million for 2015. The unfavorable change was primarily due to interest expense incurred in connection with the $20.0 million senior secured term loan.

Gain from Sale of Priority Review Voucher

In February 2017, we entered into an agreement with Gilead Sciences, Inc. ("Gilead") to sell our Rare Pediatric Disease Priority Review Voucher ("PRV"). We received the PRV when EXONDYS 51 was approved by the FDA for the treatment of patients with DMD amenable to exon 51 skipping. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2017, we completed our sale of the PRV to a subsidiary of Gilead. Pursuant to the agreement, the subsidiary of Gilead paid us $125.0 million, 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.

Liquidity and Capital Resources

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

	As of December 31, 2017	As of December 31, 2016	Change	Change
	(in thousands)		$	%
Financial assets:				
Cash and cash equivalents	$ 599,691	$ 122,420	$ 477,271	390%
Short-term investments	479,369	195,425	283,944	145%
Long-term investments	9,980	—	9,980	NA
Restricted cash and investments	784	11,479	(10,695)	(93)%
Total cash, cash equivalents and investments	$ 1,089,824	$ 329,324	$ 760,500	231%
Borrowings:				
Long-term debt	$ 30,410	$ 16,150	$ 14,260	88%
Convertible debt	400,641	—	400,641	NA
Total borrowings	$ 431,051	$ 16,150	$ 414,901	NM*
Working capital				
Current assets	$ 1,228,644	$ 373,476	$ 855,168	229%
Current liabilities	88,332	75,422	12,910	17%
Total working capital	$ 1,140,312	$ 298,054	$ 842,258	283%

*NM: not meaningful

For the year ended December 31, 2017, our principal source of liquidity was from debt and equity financings, sale of the PRV and product sales from EXONDYS 51. For the year ended December 31, 2016, our principal source of liquidity was from equity financings and product sales. 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.

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 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;
- the timing and costs associated with our clinical trials and preclinical trials;
- the attainment of milestones and our obligations to make milestone payments to BioMarin, Summit, UWA and other institutions;
- repayment of outstanding debts 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 or the licensing or sale of our technologies. 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

The following table summarizes our cash flow activity for each of the periods indicated:

	For the Year Ended December 31			
	2017	2016	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (231,996)	$ (245,820)	$ 13,824	(6)%
Investing activities	(178,815)	(90,193)	(88,622)	98%
Financing activities	888,082	378,129	509,953	135%
Increase in cash and cash equivalents	$ 477,271	$ 42,116	$ 435,155	NM*

	For the Year Ended December 31			
	2016	2015	Change	Change
	(in thousands)		$	%
Cash provided by (used in)				
Operating activities	$ (245,820)	$ (149,465)	$ (96,355)	64%
Investing activities	(90,193)	8,410	(98,603)	NM*
Financing activities	378,129	147,808	230,321	156%
Increase in cash and cash equivalents	$ 42,116	$ 6,753	$ 35,363	524%

*NM: not meaningful

Operating Activities.

Cash used in operating activities decreased by $13.8 million for 2017 compared with 2016. This was primarily due to a decrease of $91.6 million in net loss (excluding the gain from sale of PRV) which was driven by net product revenues for EXONDYS 51 and decreased research and development expenses primarily due to capitalization of inventory following the approval of EXONDYS 51 partially offset by increased selling, general and administrative expenses. Additionally, non-cash adjustments increased by $3.9 million. These were partially offset by unfavorable changes of $81.7 million in operating assets and liabilities primarily related to increases in accounts receivables and inventory as we launched EXONDYS 51.

Cash used in operating activities for 2016 increased by $96.4 million compared with 2015. The increase was primarily due to an increase of $47.2 million in net loss driven by increases in research and development and selling, general and administrative expenses slightly offset by product revenue, an unfavorable change of $47.8 million in operating assets and liabilities due to the timing of certain activities and a decrease in non-cash adjustments of $1.4 million.

Investing Activities.

Cash used in investing activities increased by $88.6 million for 2017 compared with 2016. The increase was primarily due to increases of purchases of $394.1 million of available-for-sale securities, $6.7 million of property and equipment and $7.7 million of intangible assets. The increase was partially offset by increases of proceeds of $184.1 million from maturity of available-for-sale securities, $125.0 million from sales of the PRV and $10.7 million from maturity of a restricted investment.

Cash used in investing activities for 2016 was $90.2 million compared with $8.4 million provided by investing activities in 2015. The change was primarily due to a decrease of $73.8 million in proceeds from the maturities and sales of available-for-sale securities, increases of $33.4 million in purchases of available-for-sale securities and $1.9 million in

purchases of property and equipment. These were offset by a decrease of $10.7 million due to the purchase of a restricted investment in 2015.

Financing Activities.

Cash provided by financing activities increased by $510.0 million for 2017 compared with 2016. The increase was primarily driven by increases of $624.6 million from debt financings partially offset by $50.9 million in purchase of capped call options, an increase of $47.2 million in repayment of outstanding debts and decreases of $5.7 million in proceeds from exercise of options and employee stock purchase program and $10.8 million in proceeds from sales of common stock.

Cash provided by financing activities in 2016 increased by $230.3 million compared with 2015. In June and September 2016, we sold approximately 5.8 million and 2.1 million shares of common stock, generating net proceeds of $327.4 million and $37.3 million, respectively, $244.9 million higher than the prior year's equity offering. Additionally, net proceeds from stock option exercises and the employee stock purchase program was $10.0 million higher than 2015. The increases were partially offset by $5.0 million of repayments of long-term debt and notes payable and a decrease of $19.6 million in proceeds from our senior long-term secured loan.

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 December 31, 2017:

	Payment Due by Period				
	Total	Less Than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
	(in thousands)				
Term Loan (1)	$ 34,646	$ 7,197	$ 22,340	$ 5,109	$ —
Convertible debt (1)	628,781	8,550	17,100	17,100	586,031
Mortgage loan (1)	1,265	1,265	—	—	—
Lease obligations	15,353	4,946	10,075	332	—
Purchase obligations (2)	95,616	81,677	13,939	—	—
Total contractual obligations and contingencies	$ 775,661	$ 103,635	$ 63,454	$ 22,541	$ 586,031

(1) Interest is included.

(2) Purchase obligations include agreements to purchase goods or services that are enforceable and legally binding or subject to cancellation fees and that specify all significant terms. Purchase obligations relate primarily to our commercialization of EXONDYS 51 and clinical programs for DMD.

Milestone Obligations

For product candidates that are currently in various research and development stages, we may be obligated to make up to $808.5 million of future development, up-front royalty and sales milestone payments associated with our collaboration and license agreements. Payments under these agreements generally become due and payable upon achievement of certain development, regulatory or sales milestones. Because the achievement of these milestones is not probable and payment is not required as of December 31, 2017, such contingencies have not been recorded in our 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.

Other Funding Commitments

We have several on-going clinical trials in various clinical trial stages. Our most significant clinical trial expenditures are to CROs. The CRO contracts are generally cancellable at our option. As of December 31, 2017, we have approximately $52.9 million in cancellable future commitments based on existing CRO contracts.

Recent Accounting Pronouncements

Please read *Note 2, Summary of Significant Accounting Policies and Recent Accounting Pronouncements* to the consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

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

Our current investment policy is to maintain a diversified investment portfolio consisting of money market investments, commercial paper, government and government agency bonds and high-grade corporate bonds with maturities of 36 months or less. Our cash is deposited in and invested through highly rated financial institutions in North America. As of December 31, 2017, we had $1,089.9 million of cash, cash equivalents and investments, comprised of $599.7 million of cash and cash equivalents, $479.4 million short-term investments, $10.0 million long-term investments and $0.8 million of 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. For each of the years ended December 31, 2017 and 2016, we estimate that such hypothetical adverse 10 basis point movement would result in a hypothetical loss in fair value of approximately $0.3 million and less than $0.1 million, respectively, to our interest rate sensitive instruments.

Item 8. Financial Statements and Supplementary Data.

The information required by this Item 8 begins on page F-1 in Item 15 of Part IV of this Annual Report on Form 10-K and is incorporated into this item by reference.

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

None.

Item 9A. Controls and Procedures.

Disclosure Controls and Procedures

We carried out an evaluation as of the end of the period covered by this Annual Report on Form 10-K, under the supervision and with the participation of our management, including our principal executive officer and principal financial officer, of the effectiveness of our disclosure controls and procedures pursuant to paragraph (b) of Rule 13a-15 and 15d-15 under the Exchange Act. Based on that review, the principal executive officer and principal financial officer have concluded that our disclosure controls and procedures are effective to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act (1) is recorded, processed, summarized, and reported within the time periods specified in the SEC rules and forms, and (2) is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure.

We do not expect that our disclosure controls and procedures will prevent all errors and all fraud. A control procedure, no matter how well conceived and operated, can provide only reasonable, not absolute, assurance that the objectives of the control procedure are met. Because of the inherent limitations in all control procedures, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, within our Company have been detected. These inherent limitations include the realities that judgments in decision making can be faulty, and that breakdowns can occur because of simple error or mistake. Additionally, controls can be circumvented by the individual acts of some persons, by collusion of two or more people, or by management override of the control. We considered these limitations during the development of our disclosure controls and procedures, and will continually reevaluate them to ensure they provide reasonable assurance that such controls and procedures are effective.

Internal Control over Financial Reporting

Management's Annual Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over financial reporting for our Company, as such term is defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act.

Our internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles, and includes those policies and procedures that:

- pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of our assets;
- provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that our receipts and expenditures are being made only in accordance with authorizations of our management and directors; and
- provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use or disposition of our assets that could have a material effect on our financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate. Management assessed the effectiveness of our internal control over financial reporting as of December 31, 2017. In making this assessment, management used the criteria set forth by the *Committee of Sponsoring Organizations of the Treadway Commission* ("COSO") in its 2013 Internal Control Integrated Framework.

Based on this assessment, management has concluded that, as of December 31, 2017, our internal control over financial reporting was effective based on those criteria.

The effectiveness of our internal control over financial reporting as of December 31, 2017 has been audited by KPMG LLP, an independent registered public accounting firm, as stated in their report which appears in this Annual Report on Form 10-K.

Changes in Internal Control over Financial Reporting

There have not been material changes in our internal control over financial reporting as defined in Rules 13a–15(f) and 15d–15(f) under the Exchange Act for the quarter ended December 31, 2017 that our certifying officers concluded materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders
Sarepta Therapeutics, Inc.:

Opinion on Internal Control Over Financial Reporting

We have audited Sarepta Therapeutics, Inc. and subsidiaries' (the "Company") internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission. In our opinion, the Company maintained, in all material respects, effective internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the consolidated balance sheets of the Company as of December 31, 2017 and December 31, 2016, the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2017, and the related notes (collectively, the consolidated financial statements), and our report dated March 1, 2018 expressed an unqualified opinion on those consolidated financial statements.

Basis for Opinion

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting, included in the accompanying Management's Report on Internal Control Over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit of internal control over financial reporting included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, and testing and evaluating the design and operating effectiveness of internal control based on the assessed risk. Our audit also included performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

Definition and Limitations of Internal Control Over Financial Reporting

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

(signed) KPMG LLP

Cambridge, Massachusetts
March 1, 2018

Item 9B. Other Information.

None.

PART III

Item 10. Directors, Executive Officers and Corporate Governance.

The information regarding our directors and executive officers required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2018 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 11. Executive Compensation.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2018 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

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

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2018 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

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

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2018 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

Item 14. Principal Accounting Fees and Services.

The information required by this item will be included in either an amendment to this Annual Report on Form 10-K or in our definitive proxy statement for our 2018 annual meeting of stockholders to be filed with the Commission not later than 120 days after the end of the fiscal year covered by this Annual Report on Form 10-K and is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

(a) The following documents are filed as part of this Annual Report on Form 10-K:

(1) Financial Statements

The following consolidated financial statements of the Company and the Report of KPMG LLP, Independent Registered Public Accounting Firm, are included in Part IV of this Annual Report on Form 10-K on the pages indicated:

Report of Independent Registered Public Accounting Firm	F-2
Consolidated Balance Sheets	F-3
Consolidated Statements of Operations and Comprehensive Loss	F-4
Consolidated Statements of Stockholders' Equity	F-5
Consolidated Statements of Cash Flows	F-6
Notes to Consolidated Financial Statements	F-7

(2) Financial Statement Schedules

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

(3) Exhibits

The exhibits required by Item 601 of Regulation S-K are listed in paragraph (b) below.

(b) Exhibits.

The following exhibits are filed herewith or are incorporated by reference to exhibits filed with the SEC:

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
2.1	Agreement and Plan of Merger dated June 6, 2013 between Sarepta Therapeutics, Inc., a Delaware corporation, and Sarepta Therapeutics, Inc., an Oregon corporation.	8-K12B	001-14895	2.1	6/6/13	
3.1	Amended and Restated Certificate of Incorporation.	8-K12B	001-14895	3.1	6/6/13	
3.2	Amendment to the Amended and Restated Certificate of Incorporation.	8-K	001-14895	3.1	6/30/15	
3.3	Amended and Restated Bylaws.	8-K	001-14895	3.1	9/25/14	
4.1	Form of Specimen Certificate for Common Stock.	10-Q	001-14895	4.1	8/8/13	
4.2	Form of Common Stock Purchase Warrant, issued on January 30, 2009.	8-K	001-14895	4.4	1/30/09	
4.3	Form of Common Stock Purchase Warrant, issued on August 25, 2009.	8-K	001-14895	4.1	8/24/09	
4.4	Indenture, dated as of November 14, 2017, by and between Sarepta Therapeutics, Inc. and U. S. Bank National Association (including the form of the 1.50% Convertible Senior Note due 2024).	8-K	001-14895	4.1	11/14/17	
4.5	Form of Note (included in Exhibit 4.1)	8-K	001-14895	4.1	11/14/17	
10.1†	Employment Agreement with Patrick Iversen, Ph.D., dated July 14, 1997.	10KSB	000-22613	10.12	3/30/98	
10.2†	Amendment to Employment Agreement with Patrick Iversen, Ph.D., dated December 28, 2008.	10-K	001-14895	10.5	3/15/11	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.3†	Amendment No. 2 to Employment Agreement with Patrick Iversen, Ph.D., dated January 18, 2010.	10-K	001-14895	10.6	3/15/11	
10.4†	Amended and Restated Executive Employment Agreement dated April 19, 2013 by and between Sarepta Therapeutics, Inc. and Christopher Garabedian.	10-Q	001-14895	10.2	5/9/13	
10.5†	Executive Employment Agreement dated January 10, 2011 by and between AVI BioPharma, Inc. and Effie Toshav.	10-Q	001-14895	10.1	5/10/11	
10.6†	Executive Employment Agreement dated March 29, 2011 by and between AVI BioPharma, Inc. and Peter S. Linsley, Ph.D.	10-Q	001-14895	10.4	5/10/11	
10.7†	Executive Employment Agreement dated June 13, 2011 by and between AVI BioPharma, Inc. and Edward Kaye, M.D.	10-Q	001-14895	10.4	8/8/11	
10.8†	Stand Alone Stock Option Grant between AVI BioPharma, Inc. and Effie Toshav dated January 10, 2011.	10-Q	001-14895	10.2	5/10/11	
10.9†	Stand Alone Stock Option Grant between the Registrant and Peter Linsley dated May 16, 2011.	S-8	333-175031	4.8	6/20/11	
10.10†	Stand Alone Stock Option Grant between the Registrant and Edward Kaye dated June 20, 2011.	S-8	333-175031	4.9	6/20/11	
10.11†	AVI BioPharma, Inc. 2002 Equity Incentive Plan.	Schedule 14A	001-14895	Appendix A	4/11/02	
10.12†	Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan.	8-K	001-14895	10.1	7/1/16	
10.13†	Form of Stock Option Award Agreement under the Amended and Restated 2011 Equity Incentive Plan.	10-K	001-14895	10.13	2/28/17	
10.14†	Form of Restricted Stock Agreement under the Amended and Restated 2011 Equity Incentive Plan.	10-K	001-14895	10.14	2/28/17	
10.15†	AVI BioPharma, Inc. Non-Employee Director Compensation Policy.	8-K	001-14895	10.85	10/1/10	
10.16†	Form of Indemnification Agreement.	8-K	001-14895	10.86	10/8/10	
10.17†	Form of Restricted Stock Unit Award Agreement under 2011 Equity Incentive Plan.	10-K	001-14895	10.17	2/28/17	
10.18†	Form of Stock Appreciate Right Award Agreement under the 2011 Equity Incentive Plan.	10-K	001-14895	10.18	2/28/17	
10.19†	Form of Senior Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.19	3/15/13	
10.20†	Form of Vice President Change in Control and Severance Agreement.	10-K	001-14895	10.20	3/15/13	
10.21†	Sarepta Therapeutics, Inc. Amended and Restated 2013 Employee Stock Purchase Plan.	8-K	001-14895	10.2	7/1/16	
10.22†	Executive Employment Agreement with Jayant Aphale, Ph.D.	10-Q	001-14895	10.1	8/8/13	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.23†	Retention and Severance Benefits Letter Agreement dated May 9, 2013 by and between the Company and Michael A. Jacobsen.	10-Q	001-14895	10.3	5/9/13	
10.24†	Offer Letter dated October 23, 2013 by and between Sarepta Therapeutics, Inc. and Sandesh Mahatme.	10-K	001-14895	10.24	3/3/14	
10.25†	Offer Letter dated October 23, 2012 by and between Sarepta Therapeutics, Inc. and David Tyronne Howton.	10-K	001-14895	10.25	3/3/14	
10.26†	Executive Inducement Stock Option Agreement between Arthur Krieg and Sarepta Therapeutics, Inc.	10-K	001-14895	10.26	3/3/14	
10.27†	Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan, as amended.	S-8	001-14895	4.4	2/25/16	
10.28	Form of Stock Option Award Agreement under 2014 Employment Commencement Incentive Plan	10-K	001-14895	10.28	3/3/14	
10.29*	Amended and Restated Exclusive License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc., and Sarepta International CV dated April 10, 2013.	10-Q	001-14895	10.1	5/9/13	
10.30*	First Amendment to License Agreement by and among The University of Western Australia, Sarepta Therapeutics, Inc., and Sarepta International CV dated June 19, 2016.	10-Q	001-14895	10.1	8/9/16	
10.31^	Sponsored Research Agreement between AVI BioPharma, Inc. and Charley's Fund, Inc., effective October 12, 2007.	10-K	001-14895	10.58	3/17/08	
10.32^	First Amendment to Sponsored Research Agreement between AVI BioPharma, Inc. and Charley's Fund, Inc. dated June 2, 2009.	10-Q	001-14895	10.75	8/10/09	
10.33	Commercial Lease between Research Way Investments, Landlord, and Antivirals, Inc., Tenant, effective June 15, 1992.	SB-2	333-20513	10.9	1/28/97	
10.34	Lease Extension and Modification Agreement dated September 1, 1996, by and between Research Way Investments and Antivirals, Inc.	10-K	001-14895	10.53	3/15/11	
10.35	Second Lease Extension and Modification Agreement dated January 24, 2006 by and between Research Way Investments and AVI BioPharma, Inc.	10-Q	001-14895	10.55	8/9/06	
10.36	Real Property Purchase Agreement by and between WKL Investments Airport, LLC and AVI BioPharma, Inc., dated March 1, 2007, as amended.	10-Q	001-14895	10.61	8/9/07	
10.37	Lease Agreement between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc., dated November 23, 2011.	10-K	001-14895	10.42	3/13/12	
10.38	First Amendment to Lease Agreement dated December 22, 2011 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.43	3/13/12	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.39	Second Amendment to Lease Agreement dated January 20, 2012 between AVI BioPharma, Inc. and Perpetua Power Source Technologies, Inc.	10-K	001-14895	10.44	3/13/12	
10.40	Lease dated July 27, 2009 by and between BMR-3450 Monte Villa Parkway, LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.76	11/9/09	
10.41	First Amendment to Lease dated August 30, 2011 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.4	11/8/11	
10.42	Second Amendment to Lease dated January 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-K	001-14895	10.47	3/13/12	
10.43	Third Amendment to Lease dated May 31, 2012 by and between BMR-3450 Monte Villa Parkway LLC and AVI BioPharma, Inc.	10-Q	001-14895	10.2	8/7/12	
10.44	Lease dated October 20, 2010, by and between S/I North Creek VII LLC and AVI BioPharma, Inc.	10-K	001-14895	10.57	3/15/11	
10.45	Lease Agreement dated June 25, 2013 by and between Sarepta Therapeutics, Inc. and ARE-MA Region No. 38, LLC.	8-K	001-14895	10.1	7/1/13	
10.46	Purchase and Sale Agreement dated May 22, 2014 between Sarepta Therapeutics, Inc. and Eisai Inc.	10-Q	001-14895	10.1	8/7/14	
10.47	Offer Letter dated January 6, 2014 by and between Sarepta Therapeutics, Inc. and Arthur Krieg, M.D.	10-Q	001-14895	10.1	5/8/14	
10.48†	Employment Agreement dated September 20, 2016 between Sarepta Therapeutics, Inc. and Edward M. Kaye, M.D.	10-Q	001-14895	10.1	11/7/16	
10.49	Credit and Security Agreement between Sarepta Therapeutics, Inc. and MidCap Financial dated June 26, 2015	10-Q	001-14895	10.1	8/6/15	
10.50	Pledge Agreement between Sarepta Therapeutics, Inc. and MidCap Financial dated June 26, 2015	10-Q	001-14895	10.2	8/6/15	
10.51†	Separation and Consulting Agreement and General Release between Sarepta Therapeutics, Inc. and Christopher Garabedian entered into on June 30, 2015	10-Q	001-14895	10.3	8/6/15	
10.52†	Amendment No. 1 to the Sarepta Therapeutics, Inc. Amended and Restated 2011 Equity Incentive Plan	8-K	001-14895	10.1	6/30/15	
10.53*	License and Collaboration Agreement between Summit (Oxford) Ltd and Sarepta Therapeutics, Inc. dated October 3, 2016	10-Q	001-14895	10.2	11/7/16	
10.54	Asset Purchase Agreement dated February 20, 2017 by and between Sarepta Therapeutics Inc. and Gilead Sciences, Inc.	10-Q	001-14895	10.1	5/4/17	
10.55†	Offer Letter dated December 5, 2012 by and between Sarepta Therapeutics, Inc. and Shamim Ruff	10-Q	001-14895	10.2	5/4/17	
10.56†	Offer Letter dated December 3, 2012 by and between Sarepta Therapeutics, Inc. and Alexander “Bo” Cumbo	10-Q	001-14895	10.3	5/4/17	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.57†	Offer Letter dated February 2, 2017 by and between Sarepta Therapeutics, Inc. and Dr. Catherine Stehman-Breen	10-Q	001-14895	10.4	5/4/17	
10.58† **	Form of Severance Letter Agreement entered between Sarepta Therapeutics, Inc. and each of Sandesh Mahatme, Alexander "Bo" Cumbo, David Tyronne Howton, Jr. and Shamim Ruff					X
10.59†	Employment Agreement, dated as of June 26, 2017, between Sarepta Therapeutics, Inc. and Douglas S. Ingram	8-K	001-14895	10.1	6/28/17	
10.60†	Change in Control and Severance Agreement by and between Douglas S. Ingram and Sarepta Therapeutics, Inc., effective June 26, 2017	8-K	001-14895	10.2	6/28/17	
10.61†	Amendment No. 1 to the Sarepta Therapeutics, Inc. 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.3	6/28/17	
10.62†	Restricted Stock Agreement under the 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.4	6/28/17	
10.63†	Performance Stock Option Award Agreement under the 2014 Employment Commencement Incentive Plan	8-K	001-14895	10.5	6/28/17	
10.64	Amendment No. 1 to the License and Collaboration Agreement between Summit (Oxford) Ltd. and Sarepta Therapeutics Inc. dated June 13, 2017	10-Q	001-14895	10.1	8/3/17	
10.65*	Settlement Agreement between Sarepta Therapeutics, Inc., Sarepta International C.V. and The University of Western Australia on the one hand, and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV on the other hand dated July 17, 2017	10-Q	001-14895	10.7	8/3/17	
10.66*	License Agreement between Sarepta Therapeutics, Inc. and Sarepta International C.V. on the one hand and BioMarin Leiden Holding BV, BioMarin Nederlands BV and BioMarin Technologies BV on the other hand dated July 17, 2017	10-Q	001-14895	10.8	8/3/17	
10.67	Amended and Restated Credit and Security Agreement between Sarepta Therapeutics, Inc. and MidCap Financial Trust dated July 18, 2017	10-Q	001-14895	10.9	8/3/17	
10.68	Revolving Credit and Security Agreement between Sarepta Therapeutics, Inc. and MidCap Financial Trust dated July 18, 2017	10-Q	001-14895	10.10	8/3/17	
10.69	Amendment to the Pledge Agreement related to the Amended and Restated Credit and Security Agreement between Sarepta Therapeutics, Inc. and MidCap Financial Trust dated July 18, 2017	10-Q	001-14895	10.11	8/3/17	
10.70	Pledge Agreement related to the Revolving Credit Agreement between Sarepta Therapeutics, Inc. and MidCap Financial Trust dated July 18, 2017	10-Q	001-14895	10.12	8/3/17	
10.71	Consulting Agreement dated August 17, 2017 by and between Sarepta Therapeutics, Inc. and Dr. Edward M. Kaye	10-Q	001-14895	10.1	11/1/17	

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
10.72	Offer Letter dated August 28, 2017 by and between Sarepta Therapeutics, Inc. and Guriqbal S. Basi	10-Q	001-14895	10.2	11/1/17	
10.73	Letter Agreement by and between Sarepta Therapeutics, Inc. and Guriqbal S. Basi dated September 25, 2017	10-Q	001-14895	10.3	11/1/17	
10.74	Letter Agreement by and between Sarepta Therapeutics, Inc. and Catherine Stehman-Breen dated September 26, 2017	10-Q	001-14895	10.4	11/1/17	
10.75	First Amendment to the Amended and Restated Credit and Security Agreement, dated November 7, 2017, between the Company and MidCap Financial Trust, as administrative agent.	8-K	001-14895	4.3	11/14/17	
10.76	First Amendment to the Credit and Security Agreement, dated November 7, 2017, between the Company and MidCap Financial Trust, as administrative agent	8-K	001-14895	4.4	11/14/17	
10.77	Base Call Option Transaction Confirmation, dated as of November 8, 2017, between Sarepta Therapeutics, Inc. and JPMorgan Chase Bank, National Association, London Branch.	8-K	001-14895	10.1	11/14/17	
10.78	Base Call Option Transaction Confirmation, dated as of November 8, 2017, between Sarepta Therapeutics, Inc. and Goldman Sachs & Co. LLC.	8-K	001-14895	10.2	11/14/17	
10.79	Additional Call Option Transaction Confirmation, dated as of November 9, 2017, between Sarepta Therapeutics, Inc. and JPMorgan Chase Bank, National Association, London Branch	8-K	001-14895	10.3	11/14/17	
10.80	Additional Call Option Transaction Confirmation, dated as of November 9, 2017, between Sarepta Therapeutics, Inc. and Goldman Sachs & Co. LLC	8-K	001-14895	10.4	11/14/17	
21.1**	Subsidiaries of the Registrant.					X
23.1**	Consent of Independent Registered Public Accounting Firm.					X
24.1**	Power of Attorney (contained on signature page).					X
31.1**	Certification of the Company's President and 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 President and 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

Exhibit Number	Description	Incorporated by Reference to Filings Indicated				Provided Herewith
		Form	File No.	Exhibit	Filing Date	
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

† Indicates management contract or compensatory plan, contract or arrangement.
^ Confidential treatment has been requested for portions of this exhibit.
* Confidential treatment has been granted for portions of this exhibit.
** Field herewith
*** Furnished herewith.

Item 16. Form 10-K Summary.

Not applicable.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) 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.

Dated: March 1, 2018

SAREPTA THERAPEUTICS, INC.

By: /s/ Douglas S. Ingram
Douglas S. Ingram
President and Chief Executive Officer

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below constitutes and appoints Douglas S. Ingram and Sandesh Mahatme, and each of them, with full power of substitution and resubstitution and full power to act without the other, as his or her true and lawful attorney-in-fact and agent to act in his or her name, place and stead and to execute in the name and on behalf of each person, individually and in each capacity stated below, and to file, any and all documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing, ratifying and confirming all that said attorneys-in-fact and agents or any of them or their and his or her substitute or substitutes, may lawfully do or cause to be done by virtue thereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities indicated on March 1, 2018:

Signature	Title
/s/ Douglas S. Ingram Douglas S. Ingram	President, Chief Executive Officer and Director (Principal Executive Officer)
/s/ Sandesh Mahatme Sandesh Mahatme	Executive Vice President, Chief Financial Officer and Chief Business Officer (Principal Financial and Accounting Officer)
/s/ M. Kathleen Behrens M. Kathleen Behrens, Ph.D.	Chairwoman of the Board
/s/ Richard Barry Richard Barry	Director
/s/ Michael W. Bonney Michael W. Bonney	Director
/s/ Claude Nicaise, MD Claude Nicaise, MD	Director
/s/ Hans Wigzell Hans Wigzell, M.D., Ph.D.	Director

SAREPTA THERAPEUTICS, INC.
CONSOLIDATED FINANCIAL STATEMENTS

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Report of Independent Registered Public Accounting Firm

The Board of Directors and Stockholders
Sarepta Therapeutics, Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries (the "Company") as of December 31, 2017 and December 31, 2016, the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2017, and the related notes (collectively, the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2017 and December 31, 2016, and the results of its operations and its cash flows for each of the years in the three-year period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) ("PCAOB"), the Company's internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control – Integrated Framework (2013) issued by the Committee of Sponsoring Organizations of the Treadway Commission, and our report dated March 1, 2018 expressed an unqualified opinion on the effectiveness of the Company's internal control over financial reporting.

Basis for Opinion

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

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

(signed) KPMG LLP

We have served as the Company's auditor since 2002.

Cambridge, Massachusetts
March 1, 2018

Sarepta Therapeutics, Inc.

Consolidated Balance Sheets

(in thousands, except share and per share amounts)

	As of December 31, 2017	As of December 31, 2016
Assets		
Current Assets:		
Cash and cash equivalents	$ 599,691	$ 122,420
Short-term investments	479,369	195,425
Accounts receivable	29,468	5,228
Inventory	83,605	12,813
Restricted investment	—	10,695
Other current assets	36,511	26,895
Total Current Assets	1,228,644	373,476
Property and equipment, net of accumulated depreciation of $18,022 and $30,346 as of December 31, 2017 and 2016, respectively	43,156	37,801
Intangible assets, net of accumulated amortization of $4,145 and $3,134 as of December 31, 2017 and 2016, respectively	14,355	8,076
Investments and other assets	21,809	4,751
Total Assets	$ 1,307,964	$ 424,104
Liabilities and Stockholders' Equity		
Current Liabilities:		
Accounts payable	$ 8,467	$ 29,690
Accrued expenses	68,982	31,016
Current portion of long-term debt	6,175	10,108
Deferred revenue	3,316	3,303
Other current liabilities	1,392	1,305
Total Current Liabilities	88,332	75,422
Long-term debt	424,876	6,042
Deferred rent and other	5,539	5,949
Total Liabilities	518,747	87,413
Commitments and contingencies (Note 19)		
Stockholders' Equity:		
Preferred stock, $.0001 par value, 3,333,333 shares authorized; none issued and outstanding	—	—
Common stock, $.0001 par value, 99,000,000 shares authorized; 64,791,670 and 54,759,234 issued and outstanding at December 31, 2017 and 2016, respectively	6	5
Additional paid-in capital	2,006,598	1,503,126
Accumulated other comprehensive loss	(379)	(120)
Accumulated deficit	(1,217,008)	(1,166,320)
Total Stockholders' Equity	789,217	336,691
Total Liabilities and Stockholders' Equity	$ 1,307,964	$ 424,104

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except per share data)

	For the Year Ended December 31,		
	2017	2016	2015
Revenues:			
Product, net	$ 154,584	$ 5,421	$ —
Revenue from research contracts and other grants	—	—	1,253
Total revenues	154,584	5,421	1,253
Cost and expenses:			
Cost of sales (excluding amortization of in-licensed rights)	7,353	101	—
Research and development	166,707	188,272	146,394
Selling, general and administrative	122,682	83,749	75,043
Settlement and license charges	28,427	—	—
Amortization of in-licensed rights	1,053	29	—
Total cost and expenses	326,222	272,151	221,437
Operating loss	(171,638)	(266,730)	(220,184)
Other income (loss) :			
Interest (expense) income and other, net	(1,990)	(535)	154
Gain from sale of Priority Review Voucher	125,000	—	—
Total other income (loss)	123,010	(535)	154
Loss before income tax expense	(48,628)	(267,265)	(220,030)
Income tax expense	2,060	—	—
Net loss	(50,688)	(267,265)	(220,030)
Other comprehensive loss:			
Unrealized loss on short-term securities - available-for-sale	(259)	(9)	(16)
Total other comprehensive loss	(259)	(9)	(16)
Comprehensive loss	$ (50,947)	$ (267,274)	$ (220,046)
Net loss per share — basic and diluted	$ (0.86)	$ (5.49)	$ (5.20)
Weighted average number of shares of common stock outstanding for computing basic and diluted net loss per share	58,818	48,697	42,290

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.

Consolidated Statements of Stockholders' Equity

(in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
BALANCE AT DECEMBER 31, 2014	**41,312**	**4**	**926,769**	**(95)**	**(679,025)**	**247,653**
Exercise of options for common stock	817	—	10,010	—	—	10,010
Grant of restricted stock awards	181	—	—	—	—	—
Shares withheld for taxes	(6)	—	(182)	—	—	(182)
Issuance of common stock for cash, net of offering costs	3,250	1	119,915	—	—	119,916
Issuance of common stock under employee stock purchase plan	76	—	879	—	—	879
Stock-based compensation	—	—	32,117	—	—	32,117
Unrealized loss from available-for-sale securities	—	—	—	(16)	—	(16)
Net loss	—	—	—	—	(220,030)	(220,030)
BALANCE AT DECEMBER 31, 2015	**45,630**	**5**	**1,089,508**	**(111)**	**(899,055)**	**190,347**
Exercise of options for common stock	1,113	—	19,353	—	—	19,353
Grant of restricted stock awards, net of cancellations	50	—	—	—	—	—
Shares withheld for taxes	(47)	—	(2,168)	—	—	(2,168)
Issuance of common stock for cash, net of offering costs	7,876	—	364,749	—	—	364,749
Issuance of common stock under employee stock purchase plan	137	—	1,577	—	—	1,577
Stock-based compensation	—	—	30,107	—	—	30,107
Unrealized loss from available-for-sale securities	—	—	—	(9)	—	(9)
Net loss	—	—	—	—	(267,265)	(267,265)
BALANCE AT DECEMBER 31, 2016	**54,759**	**5**	**1,503,126**	**(120)**	**(1,166,320)**	**336,691**
Exercise of options for common stock	793	—	13,799	—	—	13,799
Grant of restricted stock awards and vest of restricted stock units, net of cancellations	400	—	—	—	—	—
Shares withheld for taxes	(60)	—	(2,227)	—	—	(2,227)
Issuance of common stock for cash, net of offering costs	8,798	1	353,958	—	—	353,959
Issuance of common stock under employee stock purchase plan	102	—	1,425	—	—	1,425
Equity component of convertible notes	—	—	156,953	—	—	156,953
Purchase of capped call share options	—	—	(50,901)	—	—	(50,901)
Stock-based compensation	—	—	30,465	—	—	30,465
Unrealized loss from available-for-sale securities	—	—	—	(259)	—	(259)
Net loss	—	—	—	—	(50,688)	(50,688)
BALANCE AT DECEMBER 31, 2017	**64,792**	**$ 6**	**$ 2,006,598**	**$ (379)**	**$ (1,217,008)**	**$ 789,217**

See accompanying notes to consolidated financial statements.

Sarepta Therapeutics, Inc.
Consolidated Statements of Cash Flows
(in thousands)

	For the Year Ended December 31,		
	2017	2016	2015
Cash flows from operating activities:			
Net loss	$ (50,688)	$ (267,265)	$ (220,030)
Adjustments to reconcile net loss to cash flows in operating activities:			
Gain from sale of Priority Review Voucher	(125,000)	—	—
Depreciation and amortization	8,092	5,611	5,247
(Accretion of discount) amortization of premium on available-for-sale securities	(888)	80	652
Non-cash interest expense	2,679	355	367
Loss on disposal of assets	805	293	197
Stock-based compensation	30,465	29,962	32,117
Non-cash restructuring expense	—	911	—
Changes in operating assets and liabilities, net:			
Increase in accounts receivable	(24,240)	(1,251)	(1,561)
Increase in inventory	(70,792)	(12,813)	—
(Increase) decrease in other assets	(15,354)	(9,012)	15,249
Increase in accounts payable, accrued expenses, deferred revenue and other liabilities	12,925	7,309	18,297
Net cash used in operations	(231,996)	(245,820)	(149,465)
Cash flows from investing activities:			
Purchase of property and equipment	(12,000)	(5,341)	(3,401)
Purchase of intangible assets	(9,215)	(1,525)	(1,432)
Purchase of available-for-sale securities	(589,520)	(195,427)	(162,001)
Proceeds from sale of Priority Review Voucher	125,000	—	—
Purchase of restricted investment	—	—	(10,695)
Maturity of restricted investment	10,695	—	—
Maturity and sales of available-for-sale securities	296,225	112,100	185,939
Net cash (used in) provided by investing activities	(178,815)	(90,193)	8,410
Cash flows from financing activities:			
Proceeds from term loan	30,000	—	20,000
Proceeds from revolving line of credit	39,708	—	—
Payments of term loan and notes payable	(15,109)	(7,603)	(2,598)
Payment of revolving line of credit	(39,645)	—	—
Proceeds from sales of common stock, net of offering costs	353,959	364,802	119,916
Proceeds from convertible debt offering	570,000	—	—
Debt issuance costs	(15,154)	—	(399)
Purchase of capped call options	(50,901)	—	—
Proceeds from exercise of options and warrants and employee stock purchase program	15,224	20,930	10,889
Net cash provided by financing activities	888,082	378,129	147,808
Increase in cash, cash equivalents and restricted cash	477,271	42,116	6,753
Cash, cash equivalents and restricted cash:			
Beginning of period	122,556	80,440	73,687
End of period	$ 599,827	$ 122,556	$ 80,440
Supplemental disclosure of cash flow information:			
Cash paid during the period for interest	$ 1,912	$ 1,562	$ 769
Cash paid during the period for income taxes	$ 5,336	$ —	$ —
Supplemental schedule of non-cash investing activities and financing activities:			
Accrued exit and legal fees for debts	$ 625	$ 400	$ 400
Reclassification of software licenses	$ 204	$ —	$ —
Property and equipment reclassified to asset held for sale	$ 1,529	$ —	$ —
Property and equipment included in accrued expenses	$ 2,525	$ 1,186	$ 318
Intangible assets included in accrued expenses	$ 343	$ 1,163	$ 335
Shares withheld for taxes	$ 2,227	$ 2,168	$ 182
Accrual for offering costs related to the equity offerings	$ —	$ 53	$ —

See accompanying notes to 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 the discovery and development of unique RNA-targeted therapeutics, gene therapy and other genetic medicine approaches for the treatment of rare neuromuscular diseases. Applying its proprietary, highly-differentiated and innovative platform technologies, the Company is able to target a broad range of diseases and disorders. Its first commercial product in the U.S., 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 Duchenne muscular dystrophy ("DMD") gene that is amenable to exon 51 skipping. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

In addition to advancing its exon-skipping product candidates for DMD, including eteplirsen, golodirsen, casimersen and SRP-5051, the Company is working with several strategic partners under various agreements to research and develop multiple treatment approaches to DMD, which include Nationwide Children's Hospital, Genethon, Duke University and Summit (Oxford) Ltd. ("Summit").

As of December 31, 2017, the Company had approximately $1,089.9 million of cash, cash equivalents and investments, consisting of $599.7 million of cash and cash equivalents, $479.4 million of short-term investments, $10.0 million of long-term investments and $0.8 million of long-term restricted cash and investments. The Company believes that its balance of cash, cash equivalents and investments as of December 31, 2017 is sufficient to fund its current operational plan for at least the next twelve months, though it may pursue 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 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 DMD. The Company's CEO, as the chief operating decision-maker, manages and allocates resources to the operations of the Company on a total company basis. The Company's research and development organization is responsible for the research and discovery of new product candidates and supports development and registration efforts for potential future products. The Company's supply chain organization manages the development of the manufacturing processes, clinical trial supply and commercial product supply. The Company's commercial organization is responsible for commercialization of EXONDYS 51 in the U.S. and internationally. The Company is supported by other back-office general and administration functions. Consistent with this decision-making process, the Company's CEO uses consolidated, single-segment financial information for purposes of evaluating performance, forecasting future period financial results, allocating resources and setting incentive targets.

Estimates and Uncertainties

The preparation of the 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. Significant items subject to such estimates and assumptions include revenue recognition, inventory, convertible debt, valuation of stock-based awards, research and development expenses and income tax.

Reclassification

The Company has revised the presentation as well as the caption of certain items within the consolidated balance sheets to conform to the current period presentation. "Restricted cash and investments" of $0.8 million as December 31, 2016 is grouped into "investments and other assets". This revision had no impact on total assets.

Additionally, the Company has revised the presentation as well as caption of certain items within the consolidated statements of operations and comprehensive loss to conform to current period presentation. “Amortization of in-licensed rights” of less than $0.1 million was reclassified from “cost of sales” and presented separately in the consolidated statements of operations and comprehensive loss. The reclassification had no impact on operating loss or net loss.

The Company also has revised the presentation as well as the caption of certain items within the consolidated statements of cash flows for 2015 to conform to current period presentation “Debt issuance costs” of $0.4 million has been reclassified from “proceeds from term loan” and presented separately in the statements of cash flows. This reclassification had no impact on net cash provided by financing activities or cash, cash equivalents and restricted cash at end of period.

Further, the Company has revised the presentation as well as the caption of certain other current assets in *Note 8, Other Assets* to the consolidated financial statements to conform to the current period presentation. “Prepaid maintenance services” of approximately $0.1 million, “prepaid clinical and preclinical expenses” of $1.2 million and “prepaid commercial expenses” of less than $0.1 million as of December 31, 2016 have been reclassified from “other prepaids” and presented separately in the other current assets table. These reclassifications had no impact on total current assets or total assets.

The Company also has revised the presentation as well as the caption of certain accrued expenses in *Note 10, Accrued Expenses* to the consolidated financial statements to conform to the current period presentation. “Accrued interest expenses” of $0.1 million, “Product revenue related reserves of $0.3 million and “accrued property and equipment” of $1.2 million as of December 31, 2016 have been reclassified from “Other” and presented separately in the accrued expenses table. The reclassification had no impact on total current liabilities or total liabilities.

Fair Value Measurements

The Company has certain financial assets 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 fair value of some of the Company’s financial assets is categorized as Level 2 within the fair value hierarchy. These financial assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing observable market data. For additional information related to fair value measurements, please read *Note 5, Fair Value Measurements* to the consolidated financial statements.

Cash and Cash Equivalents

Only investments that are highly liquid and readily convertible to cash and have original maturities of three months or less are considered cash equivalents.

Available-For-Sale Debt Securities

Available-for-sale debt securities are recorded at fair market value and unrealized gains and losses are included in accumulated other comprehensive loss in stockholder’s equity. Realized gains and losses are reported in interest (expense) income and other, net, on a specific identification basis.

Accounts Receivable

The Company’s accounts receivable arise from product sales, government research contracts and other grants. They are generally stated at the invoiced amount and do not bear interest.

The accounts receivable from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies in the U.S. as well as distributors outside of the U.S. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of December 31, 2017, the credit profile for the Company's customers is deemed to be in good standing and reserves against accounts receivable are not considered necessary. Historically, no accounts receivable amounts related to government research contracts and other grants have been written off and, thus, an allowance for doubtful accounts receivable related to government research contracts and other grants is not considered necessary.

Concentration of Credit Risk

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

For the year ended December 31, 2017, 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 30 to 60 days. Outside of the U.S., the payment terms range between 60 and 120 days. Three individual customers accounted for 47%, 34% and 19% of net product revenues and 56%, 27% and 16% of accounts receivable from product sales, respectively. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profile. As of December 31, 2017, the Company believes that such customers are of high credit quality.

As of December 31, 2017, the Company's cash equivalents and investments were concentrated at a single financial institution, 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 institution.

Inventory

Inventories are stated at the lower of cost and net realizable value with cost determined on a first-in, first-out basis. The Company capitalizes inventory costs associated with products following regulatory approval when future commercialization is considered probable and the future economic benefit is expected to be realized. EXONDYS 51 which may be used in clinical development programs are included in inventory and charged to research and development expense when the product enters the research and development process and no longer can be used for commercial purposes.

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

	As of December 31, 2017	As of December 31, 2016
	(in thousands)	
Raw materials	$ 53,875	$ 9,531
Work in progress	27,442	3,175
Finished goods	2,288	107
Total inventory	$ 83,605	$ 12,813

The Company periodically reviews its inventories for excess amounts or obsolescence and writes down obsolete or otherwise unmarketable inventory to its estimated net realizable value. Additionally, though the Company's product is subject to strict quality control and monitoring which it performs throughout the manufacturing processes, certain batches or units of product may not meet quality specifications resulting in a charge to cost of sales.

Property and Equipment

Property and equipment are initially recorded at cost, including the acquisition cost and all costs necessarily incurred to bring the asset to the location and working condition necessary for its intended use. The cost of normal, recurring or periodic repairs and maintenance activities related to property and equipment are expensed as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if the repair will result in future economic benefits. Interest costs incurred during the construction period of major capital projects are capitalized until the asset is ready for its intended use, at which point the interest costs are amortized as depreciation expense over the life of the underlying asset.

The Company generally depreciates the cost of its property and equipment using the straight-line method over the estimated useful lives of the respective assets, which are summarized as follows:

Asset Category	Useful lives
Lab equipment	5 years
Office equipment	5 years
Software and computer equipment	3 - 5 years
Leasehold improvements	Lesser of the useful life or the term of the respective lease
Land	Not depreciated
Building	30 years
Construction in Progress	Not depreciated until put into service

Intangible assets

The Company's intangible assets consist of in-licensed rights and patent costs, which are stated in the Company's consolidated balance sheets net of accumulated amortization and impairments, if applicable.

The in-licensed rights relates to agreements with BioMarin (*defined in Note 3)* and the University of Western Australia ("UWA"). Following the execution of the settlement and license agreements with BioMarin in July 2017, The Company recorded a $6.6 million intangible asset related to EXONDYS 51 in the U.S. Additionally, as a result of the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement (*also defined in Note 3*), the Company was obligated to pay a $1.0 million sales milestone to UWA and, accordingly, recorded an in-licensed right. The in-licensed rights are being amortized on a straight-line basis over the remaining life of the related patent because the life of the related patent reflects the expected time period that the Company will benefit from the in-licensed right. For the years ended December 31, 2017 and 2016, the Company recorded $1.1 million and less than $0.1 million, respectively, of amortization related to the in-license rights.

Patent costs consist primarily of external legal costs, filing fees incurred to file patent applications and renewal fees on proprietary technology developed or licensed by the Company. Patent costs associated with applying for a patent, being issued a patent and annual renewal fees are capitalized. Costs to defend a patent and costs to invalidate a competitor's patent or patent application are expensed as incurred. Patent costs are amortized on a straight-line basis over the shorter of the estimated economic lives or the initial term of the patents, which is generally 20 years. Patent amortization expense was $0.6 million, $0.6 million and $0.5 million for the years ended December 31, 2017, 2016 and 2015, respectively. The Company also expensed the remaining net book value of previously capitalized patents that were later abandoned of $0.6 million, $0.3 million and $0.2 million for the years ended December 31, 2017, 2016 and 2015, respectively, which were included in research and development expenses on the consolidated statements of operations and comprehensive loss.

The following table summarizes the estimated future amortization for intangible assets for the next five years:

	As of December 31, 2017 (in thousands)
2018	1,681
2019	1,507
2020	1,465
2021	1,453
2022	1,446
Total	$ 7,552

Impairment of Long-Lived Assets

Long-lived assets held and used by the Company and intangible assets with definite lives are reviewed for impairment whenever events or circumstances indicate that the carrying amount of assets may not be recoverable. The Company evaluates recoverability of assets to be held and used by comparing the carrying amount of an asset to future net undiscounted cash flows to be generated by the asset. If such assets are considered to be impaired, the impairment to be recognized is measured as the amount by which the carrying amount of the assets exceeds the fair value of the assets. Such reviews assess the fair value of the assets based upon estimates of future cash flows that the assets are expected to generate.

Convertible Debt Transactions

The Company separately accounts for the liability and equity components of convertible debt instruments that can be settled in cash by allocating the proceeds from issuance between the liability component and the embedded conversion option in accordance with accounting for convertible debt instruments that may be settled in cash (including partial cash settlement) upon conversion. The value of the equity component is calculated by first measuring the fair value of the liability component, using the interest rate of a similar liability that does not have a conversion feature, as of the issuance date. The difference between the proceeds from the convertible debt issuance and the amount measured as the liability component is recorded as the equity component with a corresponding discount recorded on the debt. The Company recognizes the amortization of the resulting discount using the effective interest method as interest expense in its consolidated statements of operations and comprehensive loss. Simultaneously, the Company bought capped call options from certain counterparties to minimize the impact of potential dilution upon conversion. The premium for the capped call options was recorded as additional paid-in capital in its consolidated balance sheets. For additional information related to the convertible debt transactions, please read *Note 12, Indebtedness* to the consolidated financial statements.

Revenue Recognition

The Company recognizes revenue when all of the following criteria are met:

1) persuasive evidence of an arrangement exists;
2) delivery has occurred or services have been rendered;
3) price to the customer is fixed or determinable; and
4) collectability is reasonably assured.

Product revenues

Revenue from product sales is recognized when title and risk of loss have passed to the customer and is recorded net of applicable reserves for rebates, discounts and allowances.

Reserves for rebates, discounts and allowances

The Company establishes reserves for various government rebate and chargeback programs, prompt payment discounts and co-pay assistance. Reserves established for these discounts and allowances are classified as either reductions of accounts receivable (if the amount is payable to the Company's customers) or a liability (if the amount is payable to a party other than the Company's customers). These reserves are based on estimates of the amounts earned or to be claimed on the related sales. Product revenue reserves represent the Company's estimates of outstanding claims for end-user rebate-eligible sales that have occurred, but for which related claim submissions have not been received. They are categorized as follows:

- Medicaid rebates relate to the Company's estimated obligations to states under established reimbursement arrangements. Rebate accruals are recorded in the same period the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability which is included in accrued expenses.
- Governmental chargebacks, including Public Health Service ("PHS") chargebacks, represent the Company's estimated obligations resulting from contractual commitments to sell products to qualified healthcare providers at prices lower than the list prices that the Company charges to wholesalers. The wholesaler charges the Company for the difference between what the wholesaler pays for the products and the ultimate selling price to the qualified healthcare providers. Chargeback reserves are established in the same period as the related revenue is recognized, resulting in a reduction in product revenue and accounts receivable. Chargeback amounts are generally determined at the time of resale to the qualified healthcare provider from the wholesaler, and the Company generally issues credits for such amounts within a few weeks of receiving notification of resale from the wholesaler.
- Prompt payment discounts relate to estimated obligations for credits to be granted to a specialty pharmacy for remitting payment on their purchases within established incentive periods. The reserve for the prompt payment discounts are recorded in the same period as the related revenue is recognized, resulting in a reduction in product revenue and accounts receivable.
- Co-pay represents financial assistance to qualified patients, assisting them with prescription drug co-payments required by insurance. The copay reserves are recorded in the same period as the related revenue is recognized, resulting in a reduction of product revenue and the establishment of a liability which is included in accrued expenses.

The Company also maintains certain customer service contracts with distributors and other customers in the distribution channel that will provide inventory management, data and distribution services, which generally will be reflected as a reduction of revenue. To the extent the Company can demonstrate a separable benefit and fair value for these services, the Company will classify these payments as selling, general and administrative expenses.

Revenue from research contracts and other grants

The Company's contracts with the U.S. government are cost plus contracts providing for reimbursed costs which include overhead and general and administrative costs and a target fee. The Company recognizes revenue from government research contracts during the period in which the related expenses are incurred and presents such revenues and related expenses on a gross basis in the consolidated financial statements. The Company's government contracts are subject to government audits, which may result in catch-up adjustments. As of December 31, 2014, the Company had completed all development activities under its contracts with the U.S. government. The majority of the revenue under government contracts was recognized as of December 31, 2017 and only revenue for contract finalization, if any, is expected in the future.

Deferred revenue

If a technology, right, product or service is separate and independent of our performance under other elements of an arrangement, the Company defers recognition of non-refundable up-front fees if it has continuing performance obligations when the technology, right, product or service conveyed in conjunction with the non-refundable fee has no utility to the licensee. In addition, if the Company has continuing involvement through research and development services that are required because of its know-how or because the services can only be performed by the Company, such up-front fees are deferred and recognized over the period of continuing involvement. As of December 31, 2017, the Company had deferred revenue of $3.3 million, which primarily represents up-front fees which it may recognize as revenue upon settlement of certain obligations.

Research and Development

Research and development expenses consist of costs associated with research activities as well as those with the Company's product development efforts, conducting preclinical trials, clinical trials and manufacturing activities. Research and development expenses are expensed as incurred. Up-front fees and milestones paid to third parties in connection with technologies which have not reached technological feasibility and do not have an alternative future use are expensed when incurred. Nonrefundable advance payments for goods or services that will be used or rendered for future research and development activities pursuant to an executory contractual arrangement will be capitalized, and recognized as an expense as the related goods are delivered or the related services are performed. If the Company does not expect the goods to be delivered or services to be rendered, the advance payment capitalized will be charged to expense.

Direct research and development expenses associated with the Company's programs include clinical trial site costs, clinical manufacturing costs, costs incurred for consultants 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 the Company's clinical programs include salaries, stock-based compensation and an allocation of its facility costs.

When third-party service providers' billing terms do not coincide with the Company's period-end, the Company is required to make estimates of its obligations to those third parties, including clinical trial and pharmaceutical development costs, contractual services costs and costs for supply of its drug candidates, incurred in a given accounting period and record accruals at the end of the period. The Company bases its estimates on its knowledge of the research and development programs, services performed for the period, past history for related activities and the expected duration of the third party service contract, where applicable.

Stock-Based Compensation

The Company's stock-based compensation programs include stock options, restricted stock awards ("RSAs"), restricted stock units ("RSUs"), stock appreciation rights ("SARs") and employee stock purchase program ("ESPP"). The Company accounts for stock-based compensation using the fair value method.

The fair values of stock options and SARs are estimated on the date of grant using the Black-Scholes-Merton option-pricing model. The fair values of RSAs and RSUs are based on the fair market value of the Company's common stock on the date of the grant. The fair value of stock awards, with consideration given to estimated forfeitures, is recognized as stock-based compensation expense on a straight-line basis over the vesting period of the grants. For stock awards with performance-vesting conditions, the Company does not recognize compensation expense until it is probable that the performance-vesting condition will be achieved.

Under the Company's ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company's common stock on the first business day and the last business day of the relevant purchase period. The fair values of stock purchase rights are estimated using the Black-Scholes-Merton option-pricing model. The fair value of the look-back provision plus the 15% discount is recognized on a graded-vesting basis as stock-based compensation expense over the purchase period.

In addition to stock options with service and performance conditions, the Company also granted its new CEO options with service and market conditions. A market condition relates to the achievement of a specified price of the Company's common stock, a specified amount of intrinsic value indexed to the Company's common stock or a specified price of the Company's common stock in terms of other similar equity shares. The grant date fair value for the options with service and market conditions is determined by a lattice model with Monte Carlo simulations and, with consideration given to estimated forfeitures, is recognized as stock-based compensation expense on a straight-line basis over the service period.

Income Taxes

The Company follows the asset and liability method of accounting for income taxes. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. It is the intention of the Company to reinvest the earnings of its non-U.S. subsidiaries in those operations and not to repatriate the earnings to the U.S. Accordingly, the Company does not provide for deferred taxes on the excess of the financial reporting over the tax basis in its investments in foreign subsidiaries as they are considered permanent in duration. To date, the Company has not had any earnings in its non-U.S. subsidiaries.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered and settled. A valuation allowance is recorded to reduce the net deferred tax asset to zero when it is more likely than not that the net deferred tax asset will not be realized. The Company recognizes the effect of income tax positions only if those positions are more likely than not of being sustained upon an examination.

The Tax Cuts and Jobs Act ("the Act") was enacted on December 22, 2017. The Act reduces the U.S. federal corporate tax rate from 35% to 21%, requires companies to pay a one-time transition tax on earnings of certain foreign subsidiaries that were previously tax deferred and creates new taxes on certain foreign sourced earnings. On December 22, 2017, the Securities and Exchange Commission ("SEC") issued guidance under Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act ("SAB 118"). SAB 118 directs taxpayers to consider the impact of the U.S. legislation as "provisional" when it does not have the necessary information available to prepare or analyze (including computations) in reasonable detail to complete its accounting for the change in tax law.

At December 31, 2017, the Company made reasonable estimates of the effects on its existing deferred tax balances and corresponding valuation allowance. For the year ended December 31, 2017, the Company recognized no transition tax, and has remeasured deferred taxes, its reassessment of permanently reinvested earnings, uncertain tax positions and valuation allowances.

Rent Expense

The Company's operating leases for its Cambridge, Massachusetts and Corvallis, Oregon facilities provide for scheduled annual rent increases throughout each lease's term. The Company recognizes the effects of the scheduled rent increases on a straight-line basis over the full term of the leases.

For the years ended December 31, 2017, 2016 and 2015, the Company recognized rent expense and occupancy costs of $4.4 million, $5.6 million and $5.2 million, respectively.

Commitments and Contingencies

The Company records liabilities for legal and other contingencies when information available to the Company indicates that it is probable that a liability has been incurred and the amount of loss can be reasonably estimated. Legal costs in connection with legal and other contingencies are expensed as costs are incurred.

Subsequent Events

The Company has evaluated events from December 31, 2017 through the date of issuance of this report and concluded that no subsequent events have occurred that would require recognition or disclosure in the consolidated financial statements.

Recent Accounting Pronouncements

In May 2017, the FASB issued ASU No. 2017-09, "*Compensation – Stock Compensation (Topic 718): Scope of Modification Accounting*". The amendments in this update provide guidance about which changes to the terms or conditions of a stock-based payment award requires an entity to apply modification accounting in Topic 718. ASU No. 2017-09 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. The Company elected to early adopt this guidance as of June 30, 2017 and determined that the adoption of this guidance does not have any impact on its consolidated financial statements.

In November 2016, the FASB issued ASU No. 2016-18, "Statement of Cash Flows: Restricted Cash". The amendments in this update requires amounts generally described as restricted cash and restricted cash equivalents be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. ASU No. 2016-18 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. The Company elected to early adopt this guidance as of January 1, 2017. This guidance was applied using a retrospective transition method for each period and, accordingly, the Company included approximately $0.1 million of restricted cash in cash and cash equivalents as of the beginning and ending periods in the consolidated statements of cash flows.

In August 2016, the FASB issued ASU No. 2016-15, "*Statement of Cash Flows: Classification of Certain Cash Receipts and Cash Payments*". The amendments in this update clarify how certain cash receipts and cash payments are presented and classified in the statement of cash flows. ASU No. 2016-15 will be effective for fiscal years beginning after December 15, 2017, with early adoption permitted. As of December 31, 2017, the Company has elected to early adopt this guidance and determined that adoption of this guidance does not have any impact on its consolidated financial statements.

In February 2016, the FASB issued ASU No. 2016-02, "*Leases (Topic 842)*", which supersedes Topic 840, "*Leases*". Under the new guidance, a lessee should recognize assets and liabilities that arise from its leases and disclose qualitative and quantitative information about its leasing arrangements. In transition, lessees and lessors are required to recognize and measure leases at the beginning of the earliest period presented using a modified retrospective approach. ASU No. 2016-02 will be effective for fiscal years beginning after December 15, 2018, with early adoption permitted. The adoption of this standard is expected to have an impact on the amount of the Company's assets and liabilities. As of December 31, 2017, the Company has not elected to early adopt this guidance or determined the effect that the adoption of this guidance will have on its consolidated financial statements.

In May 2014, the FASB issued ASU No. 2014-09, "*Revenue from Contracts with Customers (Topic 606)*". This ASU supersedes the revenue recognition requirements in Accounting Standards Codification Topic 605, "*Revenue Recognition*". Under the new guidance, a company is required to recognize revenue when it transfers goods or renders services to customers at an amount that it expects to be entitled to in exchange for these goods or services. The new standard allows for either a full retrospective with or without practical expedients or a retrospective with a cumulative catch upon adoption transition method. This guidance was originally intended to be effective for the fiscal years beginning after December 15, 2016, with early adoption not permitted. In August 2015, the FASB issued ASU No. 2015-14, "*Deferral of the Effective Date*", which states that the mandatory effective date of this new revenue standard will be delayed by one year, with early adoption only permitted in fiscal year 2017. During the second quarter of 2016, the FASB issued three amendments to the new revenue standard to address some application questions: ASU No. 2016-10, "*Identifying Performance Obligations and Licensing*", ASU No. 2016-11, "*Rescission of SEC Guidance Because of Accounting Standards Updates 2014-09*", and ASU No. 2016-12, "*Narrow-Scope Improvements and Practical Expedients*". In December 2016, the FASB issued ASU No. 2016-20, "*Technical Corrections and Improvements to Topic 606, Revenue from Contracts with Customers"*, which amends certain narrow aspects of the guidance issued in ASU 2014-09 including guidance related to the disclosure of remaining performance obligations and prior-period performance obligations, as well as other amendments to the guidance on loan guarantee fees, contract costs, refund liabilities, advertising costs and the clarification of certain examples. These three amendments will be effective upon adoption of Topic 606. The Company has assessed the impact of the new standards as compared to its current accounting policies with respect to its contracts with its customers. As of December 31, 2017, the Company has completed its assessment and determined that it will utilize the full retrospective adoption method and that adoption of this guidance will have an immaterial financial impact on its consolidated financial statements.

3. LICENSE AND COLLABORATION AGREEMENTS

BioMarin Pharmaceutical, Inc.

In July 2017, the Company and UWA entered into a settlement agreement with BioMarin Leiden Holding BV, its subsidiaries BioMarin Nederlands BV and BioMarin Technologies BV (collectively, "BioMarin"). On the same day, the Company entered into a license agreement with BioMarin and Academisch Ziekenhuis Leiden ("AZL") (collectively with the Company, UWA and BioMarin, the "Settlement Parties"). Under these agreements, BioMarin agreed to provide the Company with an exclusive license to certain intellectual property with an option to convert the exclusive license into a co-exclusive license and the Settlement Parties agreed to stop most existing efforts to continue with ongoing litigation and opposition and other administrative proceedings concerning BioMarin's intellectual property. Under terms of the agreements, the Company agreed to make total up-front payments of $35.0 million upon execution of the agreements, consisting of $20.0 million under the settlement agreement and $15.0 million under the license agreement. Additionally, the Company may be liable for up to approximately $65.0 million in regulatory and sales milestones for eteplirsen as well as exon 45 and exon 53 skipping product candidates. BioMarin will also be eligible to receive royalty payments, ranging from 4% - 8%, for exon 51 skipping products, exon 45 skipping products and exon 53 skipping products. The royalty terms under the license agreement will expire in December 2023 in the U.S. and September 2024 in the EU.

In connection with the above agreements, in July 2017, the Company made a cash payment of $35.0 million to BioMarin. Accordingly, the Company has recorded an intangible asset of $6.6 million on its consolidated balance sheets. In addition, for the year ended December 31, 2017, the Company recorded $28.4 million settlement and license charges, in its consolidated statements of operations and comprehensive loss.

The intangible asset represents the fair value of the U.S. license to BioMarin's intellectual property related to EXONDYS 51, which was determined by an income-based approach, and is being amortized on a straight-line basis over the remaining life of the patent. For the year ended December 31, 2017, the Company recognized intangible asset amortization expense and royalties of approximately $1.0 million and $4.7 million, respectively. The royalties are included in cost of sales in the Company's consolidated statements of operations and comprehensive loss.

Summit (Oxford) Ltd.

In October 2016, the Company entered into an exclusive Collaboration and License Agreement (the "Collaboration Agreement") with Summit which grants the Company the exclusive right to commercialize products in Summit's utrophin modulator pipeline in the EU, Switzerland, Norway, Iceland, Turkey and the Commonwealth of Independent States (the "Licensed Territory").

Under the terms of the Collaboration Agreement, the Company made an up-front payment of $40.0 million to Summit, with additional payments of up to $192.0 million based on achievement of certain development and regulatory milestones for ezutromid, a Summit product candidate in its utrophin modulator pipeline. For each of Summit's future generation small molecule utrophin modulators, the Company may be required to make up to $290.0 million in development and regulatory milestone payments. Additionally, on a product-by-product basis, the Company may be required to make up to $330.0 million in sales milestone payments. The Collaboration Agreement also grants the Company an option to expand the Licensed Territory ("Option Territory"). If the Company exercises this option, it will be liable for a one-time $10.0 million option fee as well as up to $7.0 million in regulatory milestone payments. For each licensed product in the Option Territory, the Company may be liable for up to $82.5 million in sales milestone payments. Additionally, the Company may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory.

Under the Collaboration Agreement, a joint steering committee has been established to plan, monitor and coordinate future development activities for ezutromid and future generation small molecule utrophin modulators. Summit will be solely responsible for all research and development costs for the licensed products until December 31, 2017. Thereafter, Summit will be responsible for 55.0% of the budgeted research and development costs related to the licensed products in the Licensed Territory, and the Company will be responsible for 45.0% of such costs. Any costs in excess of 110.0% of the budgeted amount are borne by the party that incurred such costs. Summit is also obligated to spend a specified minimum amount on the research and development of certain licensed products prior to the end of 2019.

For the years ended December 31, 2017 and 2016, the Company recorded a $22.0 million milestone and a $40.0 million up-front payment to Summit, respectively, as research and development expense in its consolidated statement of operations and comprehensive loss.

University of Western Australia

In April 2013, the Company and UWA entered into an agreement under which an existing exclusive license agreement between the Company and UWA was amended and restated (the "Amended and Restated UWA License Agreement"). The Amended and Restated UWA License Agreement grants the Company specific rights to the treatment of DMD by inducing the skipping of certain exons. EXONDYS 51 falls under the scope of the license agreement. Under the Amended and Restated UWA License Agreement, the Company may be required to make payments of up to $6.0 million in aggregate to UWA based on the successful achievement of certain development and regulatory milestones relating to EXONDYS 51 and up to five additional product candidates. The Company may also be obligated to make payments to UWA of up to $20.0 million upon the achievement of certain sales milestones. Additionally, the Company may be required to pay a low-single-digit percentage royalty on net sales of products covered by issued patents licensed from UWA during the term of the Amended and Restated UWA License Agreement. However, the Company has the option to purchase future royalties up-front. Under this option, prior to the First Amendment (defined below), the Company could elect to make a one-time royalty payment of $30.0 million to UWA.

In June 2016, the Company and UWA entered into the first amendment to the Amended and Restated UWA License Agreement (the "First Amendment"). Under the First Amendment, the Company was obligated to make an up-front payment of $7.0 million to UWA upon execution of the amendment. Under the terms of the First Amendment, UWA has waived certain rights and amended the timing of certain payments under the Amended and Restated UWA License Agreement, including lowering the up-front payment that is due by the Company upon exercise of the option to purchase future royalties up-front. Upon exercise of the option to purchase future royalties up-front, the Company will be obligated to make a $23.0 million payment to UWA. Additionally, the Company would still be obligated to make up to $20.0 million in payments to UWA upon achievement of certain sales milestones.

For the years ended December 31, 2016 and 2015, the Company recorded $7.6 million and $0.2 million, respectively, relating to the development milestone and up-front payments to UWA as research and development expense in the consolidated statement of operations and comprehensive loss as the Amended and Restated UWA License Agreement and its First Amendment were entered into before the FDA approval of EXONDYS 51. The Company did not incur any milestone expense for the year ended December 31, 2017. Additionally, corresponding to the FDA approval and the subsequent commercial sale of EXONDYS 51, as defined in the Amended and Restated UWA License Agreement, the Company recorded a $1.0 million milestone payment as an in-license right in its consolidated balance sheet. As of December 31, 2017, the in-license right is recorded net of $0.1 million accumulated amortization on its consolidated balance sheets. The amortization of the in-licensed right is recorded as cost of sales in the Company's consolidated statements of operations and comprehensive loss. As of December 31, 2017, the Company did not make any royalty payments but may be obligated to make these payments in the future.

4. GAIN FROM SALE OF PRIORITY REVIEW VOUCHER

In February 2017, the Company entered into an agreement with Gilead Sciences, Inc. ("Gilead") to sell the Company's Rare Pediatric Disease Priority Review Voucher ("PRV"). The Company received the PRV when EXONDYS 51 was approved by the FDA for the treatment of patients with DMD amenable to exon 51 skipping. Following the early termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, in March 2017, the Company completed its sale of the PRV to a subsidiary of Gilead. Pursuant to the Agreement, the subsidiary of Gilead paid the Company $125.0 million, 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 tables below present information about the Company's financial assets that are measured and carried at fair value and indicate the level within the fair value hierarchy of the valuation techniques it utilizes to determine such fair value:

	Fair Value Measurement as of December 31, 2017			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Assets				
Cash equivalents	$ 352,370	$ 352,370	$ —	$ —
Commercial paper	133,368	—	133,368	—
Government and government agency bonds	294,717	284,745	9,972	—
Corporate bonds	127,956	127,956	—	—
Certificates of deposit	648	648	—	—
Total assets	$ 909,059	$ 765,719	$ 143,340	$ —

	Fair Value Measurement as of December 31, 2016			
	Total	Level 1	Level 2	Level 3
	(in thousands)			
Cash equivalents	$ 1,147	$ 1,147	$ —	$ —
Commercial paper	69,304	—	69,304	—
Government and government agency bonds	105,287	—	105,287	—
Corporate bonds	20,834	—	20,834	—
Certificates of deposit	11,343	11,343	—	—
Total assets	$ 207,915	$ 12,490	$ 195,425	$ —

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, corporate bonds and certificates of deposit. Certain of the government and government agency bonds and corporate bonds are publicly traded fixed income securities and are presented as cash equivalents on the consolidated balance sheets as of December 31, 2017

The Company's assets with fair value categorized as Level 2 within the fair value hierarchy consist of commercial paper, government and government agency bonds and corporate bonds. These assets have been initially valued at the transaction price and subsequently valued, at the end of each reporting period, through income-based approaches utilizing market observable data.

The carrying amounts reported in the consolidated balance sheets for cash and cash equivalents, accounts receivable, accounts payable and revolving line of credit approximate fair value because of the immediate or short-term maturity of these financial instruments. The carrying amounts for the term loan approximate fair value based on market activity for other debt instruments with similar characteristics and comparable risk.

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 consolidated balance sheets for each of the periods indicated:

	As of December 31	
	2017	2016
	(in thousands)	
Money market funds	352,370	1,147
Corporate bonds	16,720	—
Government and government agency bonds	49,972	—
Total	419,062	1,147

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 December 31, 2017 and 2016 was approximately seven and four months, respectively. The following tables summarize the Company's cash, cash equivalents and investments for each of the periods indicated:

	As of December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 532,999	$ —	$ —	$ 532,999
Commercial paper - current	133,368	—	—	133,368
Government and government agency bonds - current	294,915	2	(200)	294,717
Corporate bonds				
Current	118,121	—	(145)	117,976
Non-current	10,016	—	(36)	9,980
Total assets	$ 1,089,419	$ 2	$ (381)	$ 1,089,040
As reported:				
Cash and cash equivalents	$ 599,698	$ 2	$ (9)	$ 599,691
Short-term investments	479,705	—	(336)	479,369
Long-term investments	10,016	—	(36)	9,980
Total assets	$ 1,089,419	$ 2	$ (381)	$ 1,089,040

	As of December 31, 2016			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Market Value
	(in thousands)			
Cash and money market funds	$ 122,420	$ —	$ —	$ 122,420
Commercial paper - current	69,355	—	(51)	69,304
Government and government agency bonds - current	105,340	—	(53)	105,287
Corporate bonds - current	20,850	—	(16)	20,834
Total assets	$ 317,965	$ —	$ (120)	$ 317,845
As reported:				
Cash and cash equivalents	$ 122,420	$ —	$ —	$ 122,420
Short-term investments	195,545	—	(120)	195,425
Total assets	$ 317,965	$ —	$ (120)	$ 317,845

7. ACCOUNTS RECEIVABLE AND RESERVES FOR PRODUCT SALES

The Company's accounts receivable arise from product sales, government research contracts and other grants. They are generally stated at the invoiced amount and do not bear interest.

The accounts receivable from product sales represents receivables due from the Company's specialty distributor and specialty pharmacies in the U.S. as well as certain distributors in Israel and Middle East. The Company monitors the financial performance and creditworthiness of its customers so that it can properly assess and respond to changes in the customers' credit profiles. The Company provides reserves against trade receivables for estimated losses that may result from a customer's inability to pay. Amounts determined to be uncollectible are written-off against the established reserve. As of December 31, 2017, the credit profiles for the Company's customers are deemed to be in good standing and write-offs of accounts receivable are not considered necessary. Historically, no accounts receivable amounts related to government research contracts and other grants have been written off and, thus, an allowance for doubtful accounts receivable related to government research contracts and other grants is not considered necessary.

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

	As of December 31,	
	2017	2016
	(in thousands)	
Product sales, net of discounts and allowances	$ 28,539	$ 4,002
Government contract receivables	929	1,226
Total accounts receivable	$ 29,468	$ 5,228

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 decrease in government contract receivables is related to contract finalization and subsequent collection of the European Union SKIP-NMD Agreement related to the Company's exon 53 product candidate.

The following table summarizes an analysis of the change in reserves for discounts and allowances for the periods indicated:

	Chargebacks	Rebates	Prompt Pay	Other Accruals	Total
	(in thousands)				
Balance, as of December 31, 2016	$ 1	$ 238	$ —	$ 67	$ 306
Provision	6,455	7,659	469	1,293	15,876
Payments/credits	(5,461)	(938)	(300)	(896)	(7,595)
Balance, as of December 31, 2017	$ 995	$ 6,959	$ 169	$ 464	$ 8,587

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

	As of December 31,	
	2017	2016
	(in thousands)	
Reduction to accounts receivable	$ 1,285	$ 1
Component of accrued expenses	7,302	305
Total reserves	$ 8,587	$ 306

8. OTHER ASSETS

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

	As of December 31, 2017	As of December 31, 2016
	(in thousands)	
Manufacturing-related deposits and prepaids	$ 18,650	$ 23,604
Prepaid clinical and preclinical expenses	5,175	1,225
Prepaid research expenses	2,896	—
Prepaid maintenance services	1,711	109
Prepaid commercial expenses	1,589	54
Asset held for sale	1,501	—
Other prepaids	2,726	989
Other	2,263	914
Total other current assets	$ 36,511	$ 26,895

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

	As of December 31, 2017	As of December 31, 2016
	(in thousands)	
Long-term available-for-sale securities	$ 9,980	$ —
Prepaid clinical expenses	7,488	3,725
Alternative minimum tax credit	3,315	—
Restricted cash and investments	784	784
Other	242	242
Total investments and other assets	$ 21,809	$ 4,751

9. PROPERTY AND EQUIPMENT

Property and equipment are recorded at historical cost, net of accumulated depreciation. The following table summarizes components of property and equipment, net for each of the periods indicated:

	As of December 31,	
	2017	2016
	(in thousands)	
Land	$ 4,158	$ 4,158
Building and improvements	14,543	12,729
Software and computer equipment	8,965	6,775
Lab equipment	9,860	14,149
Office equipment	2,211	3,028
Leasehold improvements	13,633	22,872
Construction in progress	7,808	4,436
Property and equipment, gross	61,178	68,147
Less: accumulated depreciation	(18,022)	(30,346)
Property and equipment, net	$ 43,156	$ 37,801

For the years ended December 31, 2017, 2016 and 2015, depreciation expense totaled $6.4 million, $5.0 million and $4.7 million, respectively. The decrease in accumulated depreciation related to write-offs of fully deprecated property and equipment that the Company no longer uses as of December 31, 2017. The cost basis of these assets is approximately $17.1 million. No loss was recognized. Included in the $30.3 million accumulated depreciation as of December 31, 2016 was $0.8 million of accelerated depreciation for certain assets whose expected useful lives were shortened due to the Corvallis plan (*defined in Note 11*).

10. ACCRUED EXPENSES

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

	As of December 31, 2017	As of December 31, 2016
	(in thousands)	
Accrued clinical and preclinical costs	$ 15,975	$ 10,033
Accrued employee compensation costs	14,402	8,748
Accrued contract manufacturing costs	14,019	4,673
Product revenue related reserves	7,302	305
Accrued professional fees	6,794	2,799
Accrued BioMarin royalties	2,846	—
Accrued property and equipment	2,525	1,186
Accrued interest expenses	1,291	100
Accrued income taxes	943	—
Accrued research costs	401	1,186
Other	2,484	1,986
Total accrued expenses	$ 68,982	$ 31,016

11. RESTRUCTURING

In March 2016, the Company announced a long-term plan ("Corvallis plan") to consolidate all of the Company's operations to Massachusetts and reduce its workforce by approximately 19% as part of a strategic plan to increase operational efficiency. As part of the consolidation, research activities and some employees transitioned to the Company's facilities in Andover and Cambridge, Massachusetts. As of September 30, 2017, all relocations and terminations under the Corvallis plan were completed.

The second floor and the first floor of the Corvallis facility were vacated and closed and made available for sub-leasing in December 2016 and April 2017, respectively. Using a discounted cash flow methodology and based on monthly rent payments as well as estimated sublease income, the Company recognized a total of approximately $1.6 million and $2.4 million, in restructuring expenses for the second and the first floor, respectively. As of December 31, 2017, the Company continues to be obligated to make $4.9 million of minimum lease payments and certain other contractual maintenance costs for the whole facility.

In August 2016, the Company implemented a restructuring plan in Cambridge, Massachusetts ("Cambridge plan") and reduced its workforce by approximately 6%. The restructuring costs associated with the Cambridge plan consist of costs associated with workforce reduction totaling $0.6 million. The Cambridge plan was completed as of October 31, 2016.

For the years ended December 31, 2017 and 2016, the Company recognized $3.0 million and $4.6 million as restructuring expenses, respectively, less than $0.1 million and $2.3 of which related to workforce reduction.

The following table summarizes the restructuring costs by function for the period indicated:

	For the Year Ended December 31					
	2017			2016		
	(in thousands)					
	Cash	Non-cash	Total	Cash	Non-cash	Total
Research and development	$ 188	$ —	$ 188	$ 1,631	$ 382	$ 2,013
Selling, general and administrative	2,832	—	2,832	2,020	529	2,549
Total restructuring expenses	$ 3,020	$ —	$ 3,020	$ 3,651	$ 911	$ 4,562

The following table summarizes the restructuring reserve for each of the periods indicated:

	For the Year Ended December 31	
	2017	2016
	(in thousands)	
Restructuring reserve beginning balance	$ 1,588	$ —
Restructuring expenses incurred during the period	3,020	3,651
Amounts paid during the period	(1,675)	(2,063)
Restructuring reserve ending balance	$ 2,933	$ 1,588

12. INDEBTEDNESS

2024 Convertible Notes

On November 14, 2017, the Company issued $570.0 million senior notes due on November 15, 2024 (the "2024 Notes"). The 2024 Notes were issued at face value and bear interest at the rate of 1.50% per annum, payable semi-annually in cash on each May 15 and November 15, commencing on May 15, 2018.

Upon conversion, the Company may pay cash, shares of its common stock or a combination of cash and stock, as determined by the Company in its discretion. The 2024 Notes may be convertible into 7,763,552 shares of the Company's common stock under certain circumstances prior to maturity at a conversion rate of 13.621 shares per $1,000 principal amount of the 2024 Notes, which represents a conversion price of $73.42 per share, subject to adjustment under certain conditions which include:

- as the result of a spin-off or other distribution of property;
- if the Company exclusively issues shares of common stock as a dividend or distribution on common stock, or if the Company effects a share split or share combination;
- cash dividend or distribution is made to all or substantially all holders of the common stock;
- tender offer or exchange offer
- if the Company issues to all or substantially all holders of common stock any rights, options or warrants entitling them to subscribe for or purchase shares of the common stock at a price per share that is less than the average of the last reported sales prices of the common stock for the 10 consecutive trading day period ending on, and include, the trading day immediately preceding the date of announcement of such issuance; and
- at the discretion of the Company's board of directors if deemed in the best interest of the Company.

In connection with the issuance of the 2024 Notes, the Company incurred $14.8 million of debt issuance costs. These costs are being amortized under an effective interest method and are recorded as additional interest expense over the life of the 2024 Notes. The Company has separately accounted for the liability and equity components of the 2024 Notes by allocating the proceeds from issuance of the Notes between the liability component and the embedded conversion option, or equity component. This allocation was done by first estimating an interest rate at the time of issuance for similar notes that do not include the embedded conversion option. In order to estimate the interest rate, the Company first selected an appropriate implied credit rating for the Company based on its financial metrics and then selected the market yield of the debt issuances within that rating category. The Company allocated $161.1 million to the equity component, net of offering costs of $4.2 million, which was recorded as additional paid-in capital. Additionally, the Company recorded a debt discount which equals the portion allocated to the equity component and debt issuance costs of $10.6 million on the notes, both of which will be amortized under an effective interest method and recorded as additional interest expense over the life of the 2024 Notes. The effective interest rate on the liability component of the Notes for the year ended December 31, 2017 was 6.9%. The Company may incur between 0.25% and 0.5% per annum additional interest expenses should it fail to register the 2024 Notes after 180 days and 380 days after the issuance date of the 2024 Notes, respectively.

Upon the occurrence of a "fundamental change", which includes (1) change in beneficial ownership of the Company where any person/group possesses more than 50% of the voting power of the Company, (2) consolidation or merger of the Company, (3) shareholder approval of a liquidation plan or (4) the Company is delisted from NYSE or NASDAQ, the holders may require the Company to repurchase all or a portion of the 2024 Notes for cash at 100% of the principal amount of the 2024 Notes being purchased, plus any accrued and unpaid interest. Additionally, upon the occurrence of a "make-whole fundamental change" prior to the maturity date, the Company shall adjust the conversion rate on a sliding scale basis detailed in the agreement

To minimize the impact of potential dilution upon conversion of the 2024 Notes, the Company separately entered into capped call transactions with certain counterparties. The capped calls have a strike price of $73.42 and a cap price of $104.88 and are exercisable when and if the 2024 Notes are converted. If, upon conversion of the 2024 Notes, the price of the Company's common stock is between the strike price and the cap price of the capped calls, the counterparties will deliver shares of the Company's common stock and/or cash with an aggregate value equal to the difference between the price of the Company's common stock at the conversion date and the strike price, multiplied by the number of shares of the Company's common stock related to the capped calls being exercised. The Company paid $50.9 million for these capped calls transactions, which was recorded as additional paid-in capital.

The following table summarizes the 2024 Notes for the period indicated:

	As of December 31, 2017
	(in thousand)
Par value of the 2024 Notes	$ 570,000
Unamortized discounts	$ (158,890)
Debt issuance expenses	(10,449)
Net carrying value of convertible debt	$ 400,661
Fair value of convertible debt	$ 619,641

The fair value of the Company's 2024 Notes is based on open market trades and is classified as level 1 in the fair value hierarchy.

Term Loans

In July 2017, the Company entered into an amended and restated credit agreement (the "Amended and Restated Credit and Security Agreement") which provides a term loan ("July 2017 Term Loan") of $60.0 million with MidCap Financial Trust ("MidCap"). Borrowings under the Amended and Restated Credit and Security Agreement bear interest at a rate per annum equal to 6.25%, plus the one-month London Interbank Offered Rate ("LIBOR"). In addition to paying interest on the outstanding principal under the Amended and Restated Credit and Security Agreement, the Company paid an origination fee equal to 0.50% of the amount of the term loan when advanced under the Amended and Restated Credit and Security Agreement and will be liable for a final payment fee equal to 2.00% of the amount borrowed under the Amended and Restated Credit and Security Agreement when the July 2017 Term Loan is fully repaid. Commencing on July 1, 2018, and continuing for the remaining thirty six months of the facility, the Company will be required to make monthly principal payments of approximately $0.8 million, set forth in the Amended and Restated Credit and Security Agreement, subject to certain adjustments as described therein. The facility matures in July 2021.

The Company may voluntarily prepay outstanding loans under the Amended and Restated Credit and Security Agreement at any time, provided that the Company may not prepay an amount that is less than the total of all of the credit extensions and other related obligations under the Amended and Restated Credit and Security Agreement then outstanding. In the event of a permitted prepayment, the Company is obligated to pay a prepayment fee equal to the following:

- 3.00% of the outstanding principal of such advance, if the prepayment is made within twelve months of the closing date;
- 2.00% of the outstanding principal of such advance, if the prepayment is made on or after the date which is twelve months after the closing date of such advance through the date which is twenty-four months after the closing date of such advance; and
- 1.00% of the outstanding principal of such advance, if the prepayment is made on or after the date which is twenty-four months after the closing date of such advance through the date immediately preceding the maturity date.

The Amended and Restated Credit and Security Agreement contains both affirmative and negative covenants. Affirmative covenants include government compliance, reporting requirements, maintaining property, making tax payments, maintaining insurance, cooperating during litigation, etc. Additionally, the Company is required to maintain an amount of cash and/or cash equivalents equal to not less than 75% of the sum of the outstanding principal amounts under both the Amended and Restated Credit and Security Agreement and the Revolving Credit Agreement (defined below). Negative covenants include restrictions on asset dispositions, mergers or acquisitions, indebtedness, liens, distributions, transactions with affiliates and other restrictions. The Amended and Restated Credit and Security Agreement includes customary events of default, including cross defaults and material adverse changes. Additionally, the Company's failure to be compliant with the affirmative or negative covenants or make payments when they become due will result in an event of default.

After paying off certain debt issuance costs, the Company received net proceeds of $29.1 million related to the July 2017 Term Loan, $9.2 million of which was used to pay off the outstanding balance of the term loan that was taken out in June 2015 (“June 2015 Term Loan”). In connection with the July 2017 Term Loan, the Company recorded $30.0 million as long-term debt in the consolidated balance sheet as of December 31, 2017. In addition, debt issuance costs of $1.1 million related to the July 2017 Term Loan were recorded as a direct deduction to the carrying value of the July 2017 Term Loan in the consolidated balance sheet as of December 31, 2017. These costs are being amortized to interest expense using the effective interest method over the term of the loan. The following table summarizes term loans for each of the periods indicated:

	As of December 31	
	2017	2016
	(in thousands)	
Principal amount of the 2017 Term Loan	$ 30,000	$ —
Principal amount of the 2015 Term Loan	—	15,000
Unamortized debt issuance expense	(918)	(223)
Net carrying value of term loan	$ 29,082	$ 14,777

Revolving Line of Credit

In July 2017, the Company entered into a revolving credit and security agreement (the “Revolving Credit Agreement”) which provides an aggregate revolving loan commitment of $40.0 million (which may be increased by an additional tranche of $20.0 million) with MidCap (“Revolver”). Borrowings under the Revolving Credit Agreement bear interest at a rate of 3.95%, plus the one-month LIBOR. In addition to paying interest on the outstanding principal under the Revolving Credit Agreement, the Company paid $0.2 million of origination fee, which was 0.50% of the amount of the revolving loan. The Company recognized this origination fee as other asset and it is being amortized to interest expense over the term of the line-of-credit. Additionally, the Company is liable for unused line fees, minimum balance fees, collateral fees, deferred revolving loan original fees, etc. This facility matures in July 2021. The Company may voluntarily prepay the outstanding revolving loans under the Revolving Credit Agreement in whole or in part provided that the prepayment shall be in certain amounts as specified therein. As of December 31, 2017, the outstanding balance of the revolving line of credit is less than $0.1 million.

Mortgage Loans

The Company has two mortgage loans outstanding which bear interest at 4.75% with the original maturity date of February 2027 and are collateralized by the Airport Facility in Corvallis, Oregon. At December 31, 2017, these loans had unpaid principal balances of $0.8 million and $0.5 million, for a total indebtedness of $1.3 million, and were presented as current portion of long-term debt on the consolidated balance sheet. In connection with the sale of the Airport Facility in January 2018, the two long-term mortgage loans were paid off.

As of December 31, 2017, the Company recorded approximately $6.2 million as current portion of long-term debt and approximately $424.9 million as long-term debt on the consolidated balance sheets related to the convertible debt, the term loan, the revolver and the mortgage loans. Related to these debt facilities, for the years ended December 31, 2017, 2016 and 2015, the Company recorded $5.8 million, $1.9 million and $1.3 million of interest expenses.

The following table summarizes the total gross payments due under the Company’s debt arrangements:

	As of December 31, 2017
	(in thousands)
2018	$ 6,328
2019	10,000
2020	10,000
2021	5,000
2022	—
Thereafter	570,000
Total Payments	$ 601,328

13. EQUITY FINANCING

In July 2017, the Company sold approximately 8.8 million shares of common stock through an underwritten public offering, including 1.2 million shares sold to the underwriters. The offering price was $42.50 per share. The Company received net proceeds of approximately $354.0 million from the offering, net of commission and offering expenses of approximately $20.0 million.

In September 2016, the Company sold approximately 5.8 million shares of common stock through an underwritten public offering at a price of $59.75 per share. The Company received aggregate net proceeds of approximately $327.4 million from the offering net of commission and offering expenses of approximately $17.6 million.

In June 2016, the Company sold approximately 2.1 million shares of common stock through an underwritten public offering at a price of $17.84 per share. The implied underwriting discount and commission was $1.60 per share. The Company received aggregate net proceeds of approximately $37.3 million from the offering net of offering expense of approximately $0.2 million.

In October 2015, the Company sold approximately 3.3 million shares of common stock at an offering price of $39.00 per share. The Company received aggregate net proceeds of approximately $119.9 million, after deducting the underwriting discounts and offering related transaction costs of approximately $6.8 million.

14. STOCK-BASED COMPENSATION

In June 2011, the Company's stockholders approved the 2011 Equity Incentive Plan ("2011 Plan"). The 2011 Plan, which authorized 13.0 million shares of common stock to be issued, allows for the grant of stock options, SARs, RSAs, RSUs, performance shares and performance units. In June 2016 and 2015, shareholders authorized the issuance of an additional 1.3 million and 1.7 million shares, respectively, of common stock under the 2011 Plan. As of December 31, 2017, 2.3 million shares of common stock remain available for future grant under the 2011 Plan.

In June 2013, the Company's stockholders approved the 2013 ESPP with approximately 0.3 million shares of common stock available to be issued. In June 2016, the Company's stockholders approved an additional approximately 0.3 million shares of common stock available to be issued to be added to the 2013 ESPP. As of December 31, 2017, 0.2 million shares of common stock remain available for future grant under the 2013 ESPP.

In September 2014, the Company initiated the 2014 Employment Commencement Incentive Plan ("2014 Plan") with approximately 0.6 million shares of common stock available to be issued. In October 2015 and June 2017, the 2014 Plan was increased by 1.0 million 3.8 million shares of common stock available to be issued. As of December 31, 2017, 0.6 million shares of common stock remain available for future grant under the 2014 Plan.

Stock Options

In general, stock options have a ten-year term and vest over a four-year period, with one-fourth of the underlying shares vesting on the first anniversary of the grant and 1/48th of the underlying shares vesting monthly thereafter, such that the underlying shares will be fully vested on the fourth anniversary of the grant, subject to the terms of the applicable plan under which they were granted.

The fair values of stock options granted during the periods presented were measured on the date of grant using the Black-Scholes-Merton option-pricing model, with the following assumptions:

	For the Year Ended December 31,		
	2017	2016	2015
Risk-free interest rate (1)	1.6 - 2.1%	1.1 – 1.8%	1.1 – 1.7%
Expected dividend yield (2)	—	—	—
Expected terms (3)	4.2 - 4.8 years	4.2 – 4.8 years	4.7 – 5.0 years
Expected volatility (4)	54.0 - 63.0%	78.2 – 137.1 %	94.3 – 111.1 %

(1) The risk-free interest rate is estimated using an average of Treasury bill interest rates over a historical period commensurate with the expected term of the option that correlates to the prevailing interest rates at the time of grant.
(2) The expected dividend yield is zero as the Company has not paid any dividends to date and does not expect to pay dividends in the future.
(3) The expected terms are estimated using historical exercise behavior.

(4) Upon commercialization of EXONDYS 51 in the U.S., the Company's future risk profile changed. As a result, starting January 1, 2017, the Company estimates expected volatility by using only implied volatility in exchange-traded options of the Company's common stock. Prior to January 1, 2017, the expected volatility was estimated using a blend of calculated volatility of the Company's common stock over a historical period and implied volatility in exchange-traded options of the Company's common stock.

The amounts estimated according to the Black-Scholes-Merton option-pricing model may not be indicative of the actual values realized upon the exercise of these options by the holders.

The following tables summarize the Company's stock option activity for each of the periods indicated:

	For the Year Ended December 31,					
	2017		2016		2015	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	5,436,951	$ 22.70	6,515,976	$ 23.91	5,216,203	$ 24.45
Granted	4,805,722 (1)	35.09	1,285,051	14.89	2,830,078	20.28
Exercised	(792,845)	17.40	(1,056,821)	18.31	(816,696)	12.26
Cancelled	(643,624)	25.44	(1,307,255)	24.61	(713,609)	26.78
Grants outstanding at end of the period	8,806,204	$ 29.74	5,436,951	$ 22.70	6,515,976	$ 23.91
Grants exercisable at end of the period	3,288,712	$ 24.76	2,942,624	$ 24.42	2,617,167	$ 23.85
Grants vested and expected to vest at end of the period	6,910,022	$ 28.49	5,116,409	$ 22.83	5,908,213	$ 23.94

(1) In June 2017, the Company granted its new CEO 3,300,000 options with service and market conditions. These options have a five-year cliff vesting schedule. The fair value of $13.48 for these options was determined by a lattice model with Monte Carlo simulations. The remaining are service-based options which have a four-year vesting schedule with 25% vesting on the first anniversary and 1/48 monthly thereafter.

The weighted-average grant date fair value per share of stock options granted during the years ended December 31, 2017, 2016 and 2015 was $14.78, $12.46 and $14.98, respectively.

	Aggregate Intrinsic Value (in thousands)	Weighted Average Remaining Contractual Life (Years)
Options outstanding at December 31, 2017	$ 228,081	7.6
Options exercisable at December 31, 2017	$ 101,559	5.1
Options vested and expected to vest at December 31, 2017	$ 187,582	7.1

The following table summarizes the Company's stock options vested and exercised for each of the periods indicated:

	For the Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Aggregate grant date fair value of stock options vested	$ 18,225	$ 30,651	$ 27,858
Aggregate intrinsic value of stock options exercised	$ 20,922	$ 30,610	$ 18,138

In February 2016, the Company granted to executives approximately 0.3 million stock options with service- and performance-based conditions ("February 2016 performance grants"). Vesting is achieved based upon the FDA approval of EXONDYS 51 and submission of MAA for eteplirsen to the EMA and continuing service over a four-year period. As a result of both milestones achieved in 2016, 50% of the February 2016 performance grants were triggered to be vested immediately and the remaining 50% are subject to the remaining service conditions of the awards.

In June 2013, the Company granted to executives approximately 0.4 million stock options with service- and performance-based conditions ("June 2013 performance grants"). Vesting is achieved based upon various regulatory approval for EXONDYS 51 and filings of investigational new drug ("IND") submissions for other drug candidates and continuing service over a four-year period. Through the submission of two IND applications during 2014 and the approval of EXONDYS 51 in the U.S. in 2016, cumulatively, 80% of the June 2013 performance grants vested or are eligible to vest subject to the remaining service conditions of the awards. The remaining 20% were forfeited as the performance criteria was not met.

For the years ended December 31, 2017, 2016 and 2015, the Company has recognized approximately $0.9 million, $5.0 million and $0.5 million in stock-based compensation expense related to the options with performance-based criteria, respectively.

As of December 31, 2017, the total stock-based compensation expense related to non-vested awards with only service-vesting conditions not yet recognized is approximately $47.5 million and those with service- and performance-based conditions approximates $0.4 million.

Restricted Stock Awards

The Company grants RSAs to members of its board of directors and certain employees. The following table summarizes the Company's RSA activity for each of the periods indicated:

	For the Year Ended December 31,					
	2017		2016		2015	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	153,170	$ 34.53	161,320	$ 21.50	6,000	$ 34.92
Granted	341,500	34.58	117,553	41.22	181,783	20.80
Vested	(63,264)	14.60	(59,703)	13.62	(24,463)	20.21
Forfeited	(19,625)	43.02	(66,000)	33.51	(2,000)	13.90
Grants outstanding at end of the period	411,781	$ 37.23	153,170	$ 34.53	161,320	$ 21.50

In September 2016, the Company granted executives RSAs with certain sales targets. If the sales targets are achieved within the required time frame, the number of RSAs may be increased from 71,925 to 89,906 shares. In December 2017, the Company modified the expiration date of these RSAs from June 30, 2018 to December 31, 2018. As a result of this modification, the grant date fair value was changed to $54.29. As of December 31, 2017, the sales targets related to these RSAs were not deemed probable. If and when deemed probable that such performance milestones may be achieved within the required time frame, the Company may recognize up to $4.9 million of stock-based compensation related to these grants. In September 2015, the Company granted executives 65,000 RSAs with performance conditions. As a result of the performance conditions not being met, these grants were cancelled during 2016.

Restricted Stock Units

The Company also grants RSUs to members of its board of directors and certain employees. The following table summarizes the Company's RSU activity for the period indicated:

	For the Year Ended December 31, 2017	
	Shares	Weighted Average Exercise Price
Granted	181,029	$ 33.03
Vested	(78,017)	32.63
Forfeited	(36,460)	32.63
Grants outstanding at end of the period	66,552	$ 33.72

In March 2017, the Company granted executives 156,029 RSUs with certain sales target and regulatory milestones. In June 2017, one performance condition of these RSUs was achieved. As a result, 50% of these RSUs became immediately vested and, accordingly, the Company recorded $2.5 million of stock-based compensation expenses for the year ended December 31, 2017. As of September 30, 2017, it has become probable that the second performance milestone will be achieved within the required timeline. Accordingly, for the year ended December 31, 2017, the Company recognized approximately $0.4 million of stock-based compensation expenses and there is remaining unrecognized stock-based compensation expense of $0.3 million. The third performance milestone was deemed as not probable of being achieved as of December 31, 2017. If and when deemed probable that the last performance milestone may be achieved within the required time frame, the Company may recognize up to $0.7 million of stock-based compensation related to the third performance milestones of these grants.

Stock Appreciation Rights

The Company issues SARs to employees on the same terms as options granted to employees. The grant date fair value of the SARs is determined using the same valuation assumptions as for stock options described above. Stock-based compensation expense is recognized on a straight-line basis over the vesting period of the SARs.

In August 2012, 70,000 SARs were granted to the Company's former President and CEO and have an exercise price of $10.08 per share. In November 2012, 100,000 SARs were granted to the Company's Executive Vice President and CFO and have an exercise price of $23.85 per share. The SARs are classified as equity as the agreements require settlement in shares of stock.

The following table summarizes the Company's SAR activity for each of the periods indicated:

	For the Year Ended December 31,					
	2017		2016		2015	
	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price	Shares	Weighted Average Exercise Price
Grants outstanding at beginning of the period	100,000	$ 23.85	170,000	$ 18.18	170,000	$ 18.18
Exercised	—	—	(67,812)	10.08	—	—
Forfeited	—	—	(2,188)	10.08	—	—
Grants outstanding at end of the period	100,000	$ 23.85	100,000	$ 23.85	170,000	$ 18.18
Grants exercisable at end of the period	100,000	$ 23.85	100,000	$ 23.85	141,249	$ 17.59
Grants vested and expected to vest at end of the period	100,000	$ 23.85	100,000	$ 23.85	167,813	$ 18.29

	Aggregate Intrinsic Value (in thousands)	Weighted Average Remaining Contractual Life (Years)
SARs outstanding, exercisable and vested at December 31, 2017	$ 3,179	4.9

2013 Employee Stock Purchase Plan

Under the Company's ESPP, participating employees purchase common stock through payroll deductions. The purchase price is equal to 85% of the lower of the closing price of the Company's common stock on the first business day and the last business day of the relevant purchase period. The 24-month award period will end between February 28, 2018 and August 31, 2019. The following table summarizes the Company's ESPP activity for each of the periods indicated:

	For the Year Ended December 31,		
	2017	2016	2015
Number of shares purchased	102,698	137,113	75,539
Proceeds received (in millions)	$ 1.4	$ 1.6	$ 0.9

Stock-based Compensation Expense

For the years ended December 31, 2017, 2016 and 2015, total stock-based compensation expense was $30.5 million, $30.0 million and $32.1 million, respectively. Included in the amounts for the year ended December 31, 2017 and 2015 are $2.1 million and $8.6 million of stock-based compensation expense incurred in connection with the resignation of the Company's former CEOs, respectively. The following table summarizes stock-based compensation expense by function included within the consolidated statements of operations and comprehensive loss:

	For the Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Research and development	$ 8,542	$ 9,499	$ 10,403
Selling, general and administrative	21,923	20,463	21,714
Total stock-based compensation	$ 30,465	$ 29,962	$ 32,117

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

	For the Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Stock options	$ 23,416	$ 26,320	$ 29,014
Employee stock purchase plan	1,754	2,380	2,165
Restricted stock awards/units	5,295	872	446
Other	—	390	492
Total stock-based compensation	$ 30,465	$ 29,962	$ 32,117

15. 401 (K) PLAN

The Company sponsors a 401 (k) Plan ("the Plan") which is a defined contribution plan. It is available to all employees who are age 21 or older. Participants may make voluntary contributions and the Company makes matching contributions according to the Plan's matching formula. For employees who started before January 1, 2016, matching contributions fully vest after one year of service. For employees who started on and after January 1, 2016, matching contributions vest on a straight line basis over three years. The expense related to the Plan primarily consists of the Company's matching contributions.

Expense related to the Plan totaled $1.4 million, $1.2 million and $0.9 million for the years ended December 31, 2017, 2016 and 2015, respectively.

16. OTHER INCOME AND LOSS

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

	For the year ended December 31,		
	2017	2016	2015
	(in thousand)		
Interest expenses	$ (5,781)	$ (1,892)	$ (1,365)
Interest income	1,809	507	890
Other income	1,982	850	629
Gain from sale of Priority Review Voucher	125,000	—	—
Total other income (loss)	$ 123,010	$ (535)	$ 154

17. INCOME TAXES

On December 22, 2017, the Tax Cuts and Jobs Act (the “Act") was enacted in the U.S. The Act reduces the U.S. federal corporate tax rate from 35% to 21%, requires companies to pay a one-time transition tax on earnings of certain foreign subsidiaries that were previously tax deferred and creates new taxes on certain foreign sourced earnings. On December 22, 2017, the SEC issued guidance under Staff Accounting Bulletin No. 118, Income Tax Accounting Implications of the Tax Cuts and Jobs Act (“SAB 118”). SAB 118 directs taxpayers to consider the impact of the U.S. legislation as “provisional” when it does not have the necessary information available to prepare or analyze (including computations) in reasonable detail to complete its accounting for the change in tax law.

At December 31, 2017, the Company made reasonable estimates of the effects of the Act on its existing U.S. deferred tax balances. These estimates may be impacted by the need for further analysis and future clarification and guidance regarding available tax accounting methods and elections, earnings and profits computations, and state tax conformity to federal tax changes.

The Company incurred a $1.8 million state income tax liability in 2017. The state tax liability relates to tax on income of Sarepta U.S. in states where the Company had a profit in excess of net operating losses.

The Company incurred a $0.2 million current federal income tax for alternative minimum tax (“AMT”). Under the Act, corporate AMT is repealed for tax years beginning after December 31, 2017. Any AMT credit carryovers to tax years after that date generally may be utilized to the extent of the taxpayer’s regular tax liability. In addition, under the Act, any excess AMT credits are partially refundable in 2018, 2019, and 2020, and fully refundable in 2021. The Company has recorded a noncurrent receivable for the amount of expected refund of the AMT.

The Company did not incur any federal, state, or foreign income taxes in 2016 and 2015.

For the year ended December 31, 2017, the Company incurred domestic and foreign pre-tax losses of $45.7 million and $2.9 million.

The following table summarizes the reconciliation between the Company’s effective tax rate and the income tax rate for each of the periods indicated:

	For the Year Ended December 31,		
	2017	2016	2015
Federal income tax rate	34.0 %	34.0 %	34.0 %
State taxes	(27.7)	—	—
Research and development and other tax credits	8.5	1.5	0.3
Valuation allowance	(93.2)	(17.3)	(19.1)
Permanent differences	6.4	(3.4)	(1.7)
Sarepta International C.V. return to provision	62.1	—	—
Impact of tax reform, net of valuation allowance	5.9	—	—
Foreign rate differential	(0.6)	(14.8)	(13.5)
Other	0.4	—	—
Effective tax rate	(4.2)%	— %	— %

As a result of the Act, the Company remeasured all deferred tax assets and liabilities based on the rates at which they are anticipated to reverse in the future, which is generally 21%. This reduction to the Company’s deferred tax assets by approximately $87.0 was fully offset by a corresponding decrease in its valuation allowance, resulting in no net tax impact to the Company.

Permanent differences affecting the Company’s effective tax rate primarily include excess tax deductions, net of non-deductible stock-based compensation.

In December 2012, the Company licensed certain intellectual property of Sarepta Therapeutics, Inc. to its wholly owned subsidiary, Sarepta International C.V. The parties also entered into a contract research agreement under which Sarepta Therapeutics, Inc. performs research services for Sarepta International C.V. In January 2016, Sarepta Therapeutics, Inc. entered into a manufacturing and distribution agreement as well as service agreement with Sarepta International C.V. In conjunction with its recent filings, it was determined that Sarepta International C.V. in 2016 and 2017 were effectively connected with the conduct of a trade or

business by the entity in the U.S. and, accordingly, the 2016 and 2017 losses are subject to U.S. income taxes. For the years ended December 31, 2017 and 2016, Sarepta International C.V. incurred losses of $172.8 million and $214.3 million, respectively.

The following table summarizes the analysis of the deferred tax assets and liabilities for each of the periods indicated:

	As of December 31,	
	2017	2016
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforwards	$ 144,897	$ 174,569
Difference in depreciation and amortization	1,210	2,589
Research and development and other tax credits	44,306	37,812
Stock-based compensation	14,392	16,166
Deferred rent	1,210	2,306
Deferred revenue	904	1,315
Capitalized inventory	22,127	36,282
Other	4,071	3,047
Total deferred tax assets	233,117	274,086
Deferred tax liabilities:		
Debt discount	(28,548)	—
Total deferred tax liabilities	(28,548)	—
Valuation allowance	(204,569)	(274,086)
Net deferred tax assets	$ —	$ —

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which are comprised principally of U.S. federal and state net operating loss carryforwards, research and development tax credit carryforwards, stock-based compensation expense, capitalized inventory and intangibles. Under the applicable accounting standards, management has considered the Company's history of losses and concluded that it is more likely than not that the Company will not recognize the benefits of net federal and state deferred tax assets. Accordingly, a full valuation allowance of the net deferred tax asset had been established at December 31, 2017 and 2016. The net change in the valuation allowance for deferred tax assets was a decrease of $69.5 million and an increase of $54.2 million for the years ended December 31, 2017 and 2016, respectively. This decrease for the year ended December 31, 2017 was primarily due to a reduction in the federal tax rate from 34% to 21% under the Act signed into law on December 22, 2017 and the utilization of federal and state net operating losses.

As of December 31, 2017, the Company had federal and state net operating loss carryforwards of $249.3 million and $174.8 million, respectively, available to reduce future taxable income, which will expire between 2018 and 2037. In addition, the Company had $387.1 million of federal net operating loss carryforwards generated by its wholly-owned subsidiary, Sarepta International C.V., which will expire between 2036 and 2037. Utilization of these net operating losses could be limited under Section 382 of the Internal Revenue Code and similar state laws based on ownership changes and the value of the Company's stock. Additionally, the Company has $34.3 million and $11.5 million of federal and state research and development credits, respectively, available to offset future taxable income. These federal and state research and development credits begin to expire between 2018 and 2037 and between 2018 and 2032, respectively. The Company also has foreign net operating loss carryforwards of $2.9 million, which will expire in 2024.

The follow table summarizes the reconciliation of the beginning and ending amount of total unrecognized tax benefits for each of the periods indicated:

	For the Year Ended December 31,		
	2017	2016	2015
	(in thousands)		
Balance at beginning of the period	$ 4,644	$ 3,706	$ —
Increase related to current year tax positions	735	801	613
Increase related to prior year tax positions	—	137	3,093
Decrease related to prior year tax position	(245)	—	—
Balance at end of the period	$ 5,134	$ 4,644	$ 3,706

The balance of total unrecognized tax benefits at December 31, 2017, if recognized, would not affect the effective tax rate on income from continuing operations, due to a full valuation allowance against the Company's deferred tax assets. The Company does not expect that the amount of unrecognized tax benefits to change significantly in the next twelve months. The Company, including its domestic subsidiaries, files consolidated U.S. federal and state income tax returns. The Company's policy is to recognize interest and/or penalties related to income tax matters in income tax expense. It had no accrual for interest or penalties on its balance sheet at December 31, 2017 or 2016 and has not recognized interest and/or penalties in the statement of operations for years ended December 31, 2017, 2016 or 2015.

18. 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. Given that the Company recorded a net loss for each of the periods presented, there is no difference between basic and diluted net loss per share since the effect of common stock equivalents would be anti-dilutive and are, therefore, excluded from the diluted net loss per share calculation.

	For the Year Ended December 31,		
	2017	2016	2015
	(in thousands, except per share amounts)		
Net loss	$ (50,688)	$ (267,265)	$ (220,030)
Weighted-average number of shares of common stock and common stock equivalents outstanding:			
Weighted-average number of shares of common stock outstanding for computing basic loss per share	58,818	48,697	42,290
Dilutive effect of outstanding stock awards and stock options after application of the treasury stock method*	—	—	—
Weighted-average number of shares of common stock and dilutive common stock equivalents outstanding for computing diluted loss per share	58,818	48,697	42,290
Net loss per share — basic and diluted	$ (0.86)	$ (5.49)	$ (5.20)

* For the year ended December 31, 2017, stock options, RSAs, RSUs and SARs to purchase approximately 9.4 million shares of common stock, respectively, were excluded from the 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 loss per share using the if-converted method as they may be settled in cash or shares at the Company's option. The 2024 Notes have no effect on diluted net loss per share until the Company's stock price exceeds the conversion price of $73.42 per share. In the period of conversion, the 2024 Notes will have no impact on diluted net loss if they are settled in cash and will have an impact on diluted loss per share if the Notes are settled in shares upon conversion.

For the years ended 2016 and 2015, stock options, RSAs and SARs to purchase approximately 5.7 million and 6.8 million shares of common stock, respectively, were excluded from the net loss per share calculation as their effect would have been anti-dilutive.

19. COMMITMENTS AND CONTINGENCIES

Lease Obligations

In June 2013, the Company entered into a lease agreement ("Cambridge lease") for its headquarters located in Cambridge, Massachusetts. As of December 31, 2017, the Company had entered into six amendments to the Cambridge lease, increasing its total rental space for its headquarters to 88,459 square feet. The Cambridge lease and its amendments will expire in January 2021. The agreement calls for a security deposit in the form of a letter of credit totaling $0.6 million. The Company purchased a certificate of deposit ("CD") to meet the requirement and it is recorded as a long-term restricted investment in the consolidated balance sheets as of December 31, 2017 and 2016.

In June 2014, the Company entered into an agreement to sublease from an unrelated third party 10,939 square feet of office space. The sublease was terminated on December 31, 2016.

In January 2014, the Company entered into an agreement to sublease 15,077 square feet of office space to an unrelated third party. The sublease expired in July 2015. In August 2015, the Company entered into an agreement to sublease this space to another unrelated third party. The sublease expired in March 2017.

In February 2015, the Company entered into an agreement to sublease 7,461 square feet of office space to an unrelated third party. The sublease expired February 2016. In December 2016, the Company entered into an agreement to sublease this space to another unrelated third party. The sublease expired in December 2017.

The Company also leases laboratory and office space in Corvallis, Oregon which will expire in December 2020. The second floor and the first floor of the facility were vacated and closed and made available for sub-leasing in December 2016 and April 2017, respectively.

The following table summarizes the aggregate non-cancelable future minimum payments under the Company's leases:

	As of December 31, 2017 (in thousands)
2018	4,946
2019	4,986
2020	5,089
2021	332
Total minimum lease payments	$ 15,353

Royalty Obligations

The Company is obligated to pay royalties on net sales of certain of its products. The royalty rates are in the low-single-digit to high teens percentages for both inside and outside the U.S. Under the collaboration agreement with Summit signed in October 2016, the Company may be required to make tiered royalty payments ranging from a low to high teens percentage of net sales on a product-by-product basis in the Licensed Territory. Under the license agreement with BioMarin signed in July 2017, the Company is required to make a mid-single-digit percentage of royalty on the net product sales inside the U.S. and maybe required to make a high-single-digit percentage of royalty on net product sales outside the U.S. Under the Under the Amended and Restated License Agreement with UWA signed in April 2013, the Company may be obligated to pay a low-single-digit percentage of royalty on the net sales of products exceeding certain amounts, which includes EXONDYS 51. For the year ended December 31, 2017, the Company recorded $4.7 million royalty payments to BioMarin on its consolidated statements of operations and comprehensive loss.

Milestone Obligations

The Company has collaboration and license agreements for which it could be obligated to pay up-front, development, regulatory and commercial milestones as a product candidate proceeds from the submission of an investigational new drug application through approval for commercial sale and beyond. As of December 31, 2017, the Company may be obligated to make up to $808.5 million of future development, up-front royalty and sales milestone payments associated with its collaboration and license agreements. For the years ended December 31, 2017, 2016 and 2015, the Company recognized approximately $22.0 million, $47.6 million and $0.2 million relating to certain up-front and development milestone payments as research and development expense, respectively, under these agreements. As of December 31, 2017 and 2016, the Company also recorded $6.6 million and $1.0 million as an in-licensed rights, respectively, corresponding to the execution of the settlement and license agreements with BioMarin and the first sale of EXONDYS 51.

Other Funding Commitments

The Company has several on-going clinical trials in various clinical trial stages. Its most significant clinical trial expenditures are to contract research organizations ("CROs"). The CRO contracts are generally cancellable at the Company's option. As of December 31, 2017, the Company has approximately $52.9 million in cancellable future commitments based on existing CRO contracts. For the years ended December 31, 2017, 2016 and 2015, the Company recognized approximately $13.9 million, $8.1 million and $8.5 million, respectively, for expenditures incurred by CROs.

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, purported class action complaints were filed against the Company and certain of its officers in

the U.S. District Court for the District of Massachusetts on January 27, 2014 and January 29, 2014. The complaints were consolidated into a single action (*Corban v. Sarepta, et. al., No. 14-cv-10201*) by order of the court on June 23, 2014. Plaintiffs' consolidated amended complaint, filed on July 21, 2014, asserted violations of Section 10(b) of the Securities Exchange Act of 1934, as amended (the "Exchange Act") and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian, Sandy Mahatme, and Ed Kaye ("Individual Defendants," and collectively with the Company, the "Corban Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Corban Defendants made material misrepresentations or omissions during the putative class period of July 24, 2013 through November 12, 2013, regarding a data set for a Phase 2b study of eteplirsen and the likelihood of the FDA accepting the Company's new drug application for eteplirsen for review based on that data set. Plaintiffs sought compensatory damages and fees. On August 18, 2014, the Corban Defendants filed a motion to dismiss, which the Court granted on March 31, 2015. Plaintiffs subsequently sought leave to file a second amended complaint, which the Corban Defendants opposed. On September 2, 2015, the Court denied Plaintiffs' motion for leave to amend as futile. Plaintiffs filed a notice of appeal on September 29, 2015, seeking review of the Court's March 31, 2015 order dismissing the case and the Court's September 2, 2015 order denying leave to amend. On January 27, 2016, Plaintiffs filed in the district court a motion for relief from judgment pursuant to Federal Rule of Civil Procedure 60(b)(2), arguing that the FDA Briefing Document published on or about January 15, 2016, was material and would have changed the Court's ruling. On February 26, 2016, the First Circuit stayed the appeal pending the district court's ruling on the 60(b)(2) motion. Defendants opposed the 60(b)(2) motion, and on April 21, 2016, the Court denied Plaintiffs' motion for relief from judgment. On May 19, 2016, Plaintiffs filed a motion to alter or amend the April 21, 2016 order pursuant to Federal Rule of Civil Procedure 59(e). On May 20, 2016, the Court denied Plaintiffs' motion, and Plaintiffs filed a notice of appeal of the Court's April 21, 2016 denial of their 60(b)(2) motion and May 20, 2016 denial of their 59(e) motion. On June 13, 2016, the First Circuit granted Plaintiffs' motion to consolidate the two appeals. Oral argument took place on March 7, 2017 and the First Circuit affirmed the District Court's dismissal of this case on August 22, 2017. Plaintiffs filed a Petition for Panel Rehearing and Rehearing *En Banc*, which the First Circuit denied on October 11, 2017. As such, the risk of loss is not deemed probable.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 styled William Kader, Individually and on Behalf of All Others Similarly Situated v. Sarepta Therapeutics Inc., Christopher Garabedian, and Sandesh Mahatme (*Kader v. Sarepta et.al 1:14-cv-14318*). On March 20, 2015, Plaintiffs filed an amended complaint asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, and Chris Garabedian and Sandy Mahatme ("Individual Defendants," and collectively with the Company, the "Kader Defendants"), and violations of Section 20(a) of the Exchange Act against the Individual Defendants. Plaintiffs alleged that the Kader Defendants made material misrepresentations or omissions during the putative class period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of an NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs sought compensatory damages and fees. The Kader Defendants moved to dismiss the amended complaint on May 11, 2015. On April 5, 2016, following oral argument on March 29, 2016, the Court granted Defendants' motion to dismiss. On April 8, 2016, Lead Plaintiffs filed a motion for leave to file an amended complaint, which Defendants opposed. On January 6, 2017, the Court denied Plaintiffs' motion for leave to amend and dismissed the case. Plaintiffs filed a notice of appeal on February 3, 2017. Appellants' brief was filed April 24, 2017 and Appellee's brief was filed May 24, 2017. Oral argument took place on December 4, 2017. A decision has not yet been issued by the First Circuit. As such, the risk of loss is not deemed probable.

On February 5, 2015, a derivative suit was filed in the 215th Judicial District of Harris County, Texas against the Company's Board of Directors (*David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et al., No. 2015-06645*). The claims allege that Sarepta's directors caused Sarepta to disseminate materially false and/or misleading statements in connection with disclosures concerning the Company's submission of the NDA for eteplirsen. Plaintiff seeks unspecified compensatory damages, actions to reform and improve corporate governance and internal procedures, disgorgement of profits, benefits and other compensation obtained by the directors, and attorneys' fees. The parties have agreed to stay the case pending resolution of the *Corban* and *Kader* cases. As such, the risk of loss is not deemed probable.

On March 16, 2016, a derivative suit was filed in the U.S. District Court for the District of Massachusetts against the Company's Board of Directors (*Dawn Cherry, on behalf of nominal defendant Sarepta Therapeutics, Inc., v. Behrens et al., No. 16-cv-10531*). The claims allege that the defendants authorized the Company to make materially false and misleading statements about the Company's business prospects in connection with its development of eteplirsen from July 10, 2013 through the date of the complaint. Plaintiffs seek unspecified damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. The parties have agreed to stay the case pending resolution of the *Corban* and *Kader* cases. As such, the risk of loss is not deemed probable.

Additionally, on September 23, 2014, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (*Terry McDonald, derivatively on behalf of Sarepta Therapeutics, Inc., et al. v. Goolsbee et al., No. 10157*). The claims allege, among other things, that (i) the Company's non-employee directors paid themselves excessive compensation fees for 2013, (ii) that the compensation for the Company's former Chief Executive Officer, Christopher Garabedian,

was also excessive and such fees were the basis for Mr. Garabedian's not objecting to or stopping the excessive fees for the non-employee directors and (iii) that the disclosure in the 2013 proxy statement was deficient. The relief sought, among others, includes disgorgement and rescindment of allegedly excessive or unfair payments and equity grants to Mr. Garabedian and the directors, unspecified damages plus interest, a declaration that the Company's Amended and Restated 2011 Equity Plan at the 2013 annual meeting was ineffective and a revote for approved amendments, correction of misleading disclosures and plaintiff's attorney fees. The parties have agreed to a Memorandum of Understanding concerning the settlement terms and do not believe that disposition of the McDonald suit will have a material financial impact on the Company. The parties have now completed the confirmatory discovery process that will allow plaintiffs' counsel to represent to the court that the terms of the settlement are fair.

Purchase Commitments

The Company has entered into long-term contractual arrangements from time to time for the provision of goods and services.

The following table presents non-cancelable contractual obligations arising from these arrangements:

	As of December 31, 2017 (in thousands)
2018	81,677
2019	13,939
Total purchase commitments	$ 95,616

In connection with an amendment to a supply agreement, in September 2015, the Company issued an irrevocable standby letter of credit totaling $10.7 million to a contract manufacturing vendor. The obligation secured by the letter of credit will be fulfilled upon full payment of all deposits and purchase payments by February 2017. To meet the requirement of the letter of credit, the Company purchased $10.7 million in a certificate of deposit with a September 2017 maturity date. The Company recorded this $10.7 million as a restricted investment on the consolidated balance sheet as of December 31, 2016, which matured in September 2017.

20. FINANCIAL INFORMATION BY QUARTER (UNAUDITED)

	2017 for Quarter Ended			
	December 31	September 30	June 30	March 31
	(in thousands)			
Revenues:				
Product, net	$ 57,277	$ 45,954	$ 35,011	$ 16,342
Total revenues	57,277	45,954	35,011	16,342
Operating expenses:				
Cost of sales (excluding amortization of in-licensed rights)	3,546	3,078	505	224
Research and development	44,441	34,239	58,908	29,119
Selling, general and administrative	32,221	28,176	36,069	26,216
Settlement and license charges	—	25,588	2,839	—
Amortization of in-licensed rights	216	780	29	28
Total cost and operating expenses	80,424	91,861	98,350	55,587
Operating loss	(23,147)	(45,907)	(63,339)	(39,245)
Other (loss) income:				
Interest (expense) income and other, net	(2,693)	184	184	335
Gain from sale of Priority Review Voucher	—	—	—	125,000
Total other (loss) income	(2,693)	184	184	125,335
(Loss) income before income tax (benefit) expense	(25,840)	(45,723)	(63,155)	86,090
Income tax (benefit) expense	(1,842)	2,011	(109)	2,000
Net (loss) income	$ (23,998)	$ (47,734)	$ (63,046)	$ 84,090
Net income (loss) per share:				
Basic (loss) earnings per share	$ (0.37)	$ (0.78)	$ (1.15)	$ 1.53
Diluted (loss) earnings per share	$ (0.37)	$ (0.78)	$ (1.15)	$ 1.50
Weighted average number of shares of common stock used in calculating:				
Basic (loss) earnings per share	64,277	61,528	54,976	54,850
Diluted (loss) earnings per share	64,277	61,258	54,976	56,012

	2016 for Quarter Ended			
	December 31	September 30	June 30	March 31
	(in thousands)			
Revenues:				
Product, net	$ 5,421	$ —	$ —	$ —
Total revenues	5,421	—	—	—
Cost and operating expenses:				
Cost of sales (excluding amortization of in-licensed rights)	101	—	—	—
Amortization of in-licensed rights	29	—	—	—
Research and development	70,749	34,349	44,348	38,826
Selling, general and administrative	22,937	22,184	17,752	20,876
Total operating expenses	93,816	56,533	62,100	59,702
Operating loss	(88,395)	(56,533)	(62,100)	(59,702)
Other (loss) income:				
Interest expense and other, net	(57)	(209)	(201)	(68)
Total other loss	(57)	(209)	(201)	(68)
Net loss	$ (88,452)	$ (56,742)	$ (62,301)	$ (59,770)
Net loss per share—basic and diluted	$ (1.62)	$ (1.18)	$ (1.35)	$ (1.31)
Shares used in per share calculations—basic and diluted	54,619	48,254	46,157	45,697

Exhibit 10.58

April 27, 2017

[Executive Name]
c/o Sarepta Therapeutics, Inc.
215 First Street, Suite 415
Cambridge, MA 02142

Dear [_____]:

We are pleased to confirm our agreement relating to your continued employment with Sarepta Therapeutics, Inc. (the "Company") pursuant to the terms and conditions as set forth in this letter agreement (this "letter agreement"). Defined terms that are used but not defined herein shall have the meanings given to such terms as shown on Annex A. All payments and benefits payable pursuant to this letter agreement will be subject to applicable withholding taxes.

1. Severance.

(a) Subject to Section 1(b), Annex B and the other terms and conditions of this letter agreement, in the event that, during the Termination Period, your employment is terminated without Cause or you resign for Good Reason (any such termination during the Termination Period, a "Qualifying Termination"), in addition to any unpaid salary, accrued but unpaid bonus for the year preceding the year of termination, and vested benefits (including, but not limited to, reimbursement for reimbursable business expenses incurred prior to such termination, unused vacation, and unused sick days) owed to you as of the date of such termination, you (or in the event of your death following a Qualifying Termination, your beneficiary) shall be entitled to:

(i) a severance payment ("Severance Payment") equal to:

(A) in the case of a Qualifying Termination during Protection Period One, 1.5 times of the sum of (x) your then annual base salary and (y) your target bonus for the year of termination, which Severance Payment shall be paid to you, with respect to the salary component, in substantially equal installments in accordance with the Company's regular payroll policies over a period of eighteen (18) months following the date of such termination, and with respect to the bonus component, in a lump sum; or

(B) in the case of a Qualifying Termination during Protection Period Two, 1.0 times of the sum of (x) your then annual base salary and (y) your target bonus for the year of termination, which Severance Payment shall be paid to you, with respect to the salary component, in substantially equal installments in accordance with the Company's regular payroll policies over a period of twelve (12) months following the date of such termination (such twelve (12) month period with respect to Protection Period Two and the eighteen (18) month period described in Section 1(a)(i)(A) with respect to Protection Period One, each a "Severance Period"), and with respect to the bonus component, in a lump sum;

(ii) a monthly payment of an amount equal to the monthly premiums for continuation coverage under the Company's group health plans (in which you and your applicable covered dependents participated immediately prior to your Qualifying Termination) for the period beginning on your employment termination date and ending on the earlier of (x) the expiration of the applicable Severance Period and (y) the date you become eligible for group health insurance coverage through a new employer (the "COBRA Payments") (subject to your timely completion and submission of the necessary election forms, and further subject to your co-payment of the monthly premiums (if any) at the applicable active employees' rate and any administrative fee); provided that if such continuation coverage violates federal non-discrimination laws or rules applicable to such group health insurance plan(s) in a manner that adversely affects the Company or any of its affiliates, as reasonably determined by the Company in its sole discretion, you and the Company will work together to identify an alternative arrangement that provides substantially the same economic benefit as these COBRA Payments without any increase in cost to the Company; and

(iii) vesting of each outstanding equity award granted to you by the Company that is listed in Annex C ("Equity Awards"), to the extent provided below ("Equity Vesting"):

(A) except with respect to Equity Awards that are shares of restricted stock of the Company, for your unvested Equity Awards that have no performance requirements ("Time-Vested Awards") and for your unvested Equity Awards that have performance requirements and/or milestones ("Performance-Vested Awards") for which the performance requirements and/or milestones were satisfied as of the date of your Qualifying Termination, continued vesting during the applicable Severance Period (twelve or eighteen months, as the case may be under Section 1(a)(i));

(B) with respect to Equity Awards that are shares of restricted stock of the Company for which performance requirements and/or milestones were satisfied or no performance requirements or milestones were required as of your Qualifying Termination, accelerated vesting of the portion of the shares of restricted stock that would have otherwise vested during the applicable Severance Period had you not had a Qualifying Termination and had you continued to be employed by the Company during the applicable Severance Period;

(C) for your Performance-Vested Awards for which the performance requirements and/or milestones were not satisfied as of the date of your Qualifying Termination (other than the September 2016 performance-based restricted stock award subject to a performance milestone based on achievement of a specified quarterly revenue run rate target through June 30, 2018 (the "September 2016 RSA"), accelerated vesting of the portion of the Performance-Vested Awards that vest within six (6) months following your Qualifying Termination based on the actual achievement of the applicable performance requirements and/or milestones and without regard to any further time-based vesting requirement; and

(D) for your September 2016 RSA, accelerated vesting of that award on the date of the actual achievement of the performance milestone by June 30, 2018 and without regard to any further time-based vesting requirement.

Equity Awards that are not vested by the dates set forth above shall be forfeited and cancelled in their entirety on such applicable dates.

(b) Notwithstanding anything herein to the contrary, the Company's (or any of its affiliates') obligations to pay you the Severance Payments, pay you the COBRA Payments, and provide you the Equity Vesting as described in Section 1(a)(iii) shall be conditioned upon your execution, delivery, and non-revocation of a valid and enforceable release of claims in favor of the Company and its affiliates that is substantially in the form attached hereto as Annex D (the "Release") which Release, within 60 days after your termination date, has become effective and is no longer subject to revocation under applicable law. Subject to the foregoing and the provisions set forth herein, the Severance Payments and COBRA Payments will commence to be paid to you on the 61st day following your employment termination date, and shall include any Severance Payments and COBRA Payments that were otherwise scheduled to be paid prior thereto. Subject to the foregoing and the provisions set forth herein, vesting and forfeiture of Equity Awards shall be suspended during the sixty (60) day period following the date of your Qualifying Termination ("Suspension Period"), and such Equity Awards shall vest only (including those scheduled to vest during the Suspension Period) at the end of and following the Suspension Period in accordance with Section 1(a)(iii) if you timely execute and do not revoke the Release during the Suspension Period.

(c) Notwithstanding anything herein to the contrary, a Qualifying Termination shall occur only if such termination occurs on or following the Company's hiring of a new Chief Executive Officer of the Company who is not currently employed by the Company ("New CEO") or if such termination is in connection with, or otherwise related to the hiring of a New CEO. Any termination that occurs as a result of any action by the Company's current Chief Executive Officer shall not be a Qualifying Termination under this letter agreement. Notwithstanding anything in this Section 1 to the contrary, in the event your employment is terminated by the Company without Cause or by you for Good Reason on or after the execution date of this letter agreement but before the Commencement Date, and your termination follows, is in connection with, or is otherwise related to, the hiring of a New CEO, you shall be deemed for purposes of this letter agreement to have had a Qualifying Termination on the Commencement Date and therefore entitled to the Severance Payments, COBRA Payments, and Equity Vesting under Section 1 (in addition to any salary or bonus earned, health benefits provided, or Equity Award vesting from the date of your actual termination of employment to the Commencement Date), subject to the terms and conditions set forth therein.

2. Equity Exercise Period and Payments. In the event of your Qualifying Termination, you shall (a) have no less than ninety (90) days from the end of the applicable Severance Period (twelve or eighteen months, as the case may be under Section 1(a)(i)) (but in no event beyond the remaining term of such equity awards) to exercise any exercisable Equity Awards (i.e., options) already vested as of the last date of the applicable Severance Period; and (b) have the right to payment for non-exercisable Equity Awards that vest during the applicable Severance Period, payable within thirty (30) days following the end of the last vesting date provided under Section 1(a)(iii). Unless otherwise approved in writing by the Board of Directors or its designee, in the event of your termination during the Termination Period for any reason other than without Cause or Good Reason, the time period to exercise or receive payment for any equity awards already vested as of the date of termination shall be as set forth in the Equity Award plan documents or award agreements.

3. Company's Property. Within five business days of your termination of employment with the Company for any reason (including, without limitation, due to a Qualifying Termination) (or at any time prior thereto at the Company's request), you shall return all property belonging to the Company or its affiliates (including, but not limited to, any Company-provided laptops, computers, cell phones, wireless electronic mail devices or other equipment, or documents and property belonging to the Company). To the extent any Company records reside on personal devices you shall promptly delete such records unless otherwise prohibited by law. You may retain your rolodex and similar address books provided that such items only include contact information. To the extent that you are provided with a cell phone number by the Company during employment, the Company shall cooperate with you in transferring such cell phone number to your individual name following the date of termination.

4. Confidentiality; Non-Interference; Non-Solicitation; and Non-Disparagement Covenants.

(a) Confidentiality. During your employment with the Company and at any time thereafter you will remain subject to the terms of the Confidentiality Agreement provided, however, the foregoing shall not apply to information that (i) was known to the public prior to its disclosure to you; (ii) becomes generally known to the public subsequent to disclosure to you through no wrongful act of you or any representative of you; or (iii) you are required to disclose by applicable law, regulation or legal process (provided that you provide the Company with prior notice of the contemplated disclosure and cooperates with the Company at its expense in seeking a protective order or other appropriate protection of such information). In addition, nothing in this Agreement shall be construed to prohibit you from reporting possible violations of federal or state law or regulations to any governmental entity or self-regulatory organization with oversight responsibility for the Company, or making other disclosures that are protected under whistleblower or other provisions of any applicable federal or state law or regulations. Prior authorization of the Company is not required to make any such reports or disclosures, and you are not required to notify the Company that he has made such reports or disclosures.

(b) Non-Interference. During your employment with the Company and for a period of one (1) year thereafter plus any additional period during which you may be receiving the Severance Payments, the COBRA Payments, or the Equity Vesting (such one (1) year period plus additional period, "Restriction Period"), you agree that you shall not, except in the furtherance of your duties hereunder, directly or indirectly, individually or on behalf of any other person, firm, corporation or other entity, solicit, aid or induce any customer of the Company or any of its subsidiaries or affiliates to purchase goods or services then sold by the Company or any of its subsidiaries or affiliates from another person, firm, corporation or other entity or assist or aid any other persons or entity in identifying or soliciting any such customer.

(c) Non-Solicitation and Non-Disparagement. During your employment with the Company and for the Restriction Period, you agree that you shall not, except in the furtherance of your duties hereunder, directly or indirectly, individually or on behalf of any other person, firm, corporation or other entity, (A) solicit, aid or induce any employee, representative or agent of the Company or any of its subsidiaries or affiliates to leave such employment or retention or to accept employment with or render services to or with any other person, firm, corporation or other entity unaffiliated with the Company or hire or retain any such employee, representative or agent, or take any action to materially assist or aid any other person, firm, corporation or other entity in identifying, hiring or soliciting any such employee, representative or agent, (B) interfere, or aid or induce any other person or entity in interfering, with the relationship between the Company or any of its subsidiaries or affiliates and any of their respective vendors, joint venturers, or licensors; or (C) either publicly or privately, disparage, criticize or defame the Company, its affiliates and their respective affiliates, directors, officers, agents, partners, stockholders, individuals or the Company's, products, services, technology or business. An employee, representative or agent shall be deemed covered by this Section

4 while so employed or retained and for a period of six (6) months thereafter, Notwithstanding the foregoing, the provisions of this Section 4(c) shall not be violated by (A) general advertising or solicitation not specifically targeted at Company-related persons or entities, (B) you serving as a reference, upon request, for any employee of the Company or any of its subsidiaries or affiliates, or (C) actions taken by any person or entity with which you are associated if you are not personally involved in any manner in the matter and have not identified such Company-related person or entity for soliciting or hiring.

5. Non-Competition Covenant. You acknowledge that you perform services of a unique nature for the Company that are irreplaceable, and that your performance of such services to a competing business will result in irreparable harm to the Company. Accordingly, during your employment hereunder and for the Restriction Period, you agree that you will not, directly or indirectly, own, manage, operate, control, be employed by (whether as an employee, consultant, independent contractor or otherwise, and whether or not for compensation) or render services to any person, firm, corporation or other entity, in whatever form, engaged in the research, development or sale of Duchenne Muscular Dystrophy treatments ("DMD"), oligonucleotide based therapies with respect to DMD, or chemistry platforms with respect to DMD that compete with Company or any of its subsidiaries or affiliates or in any other material business in which the Company or any of its subsidiaries or affiliates is engaged on the date of termination or in which they have planned, on or prior to such date, to be engaged in on or after such date, in any locale of any country in which the Company conducts business. Notwithstanding the foregoing, nothing herein shall prohibit you from being a passive owner of not more than one percent (1%) of the equity securities of a publicly traded corporation engaged in a business that is in competition with the Company or any of its subsidiaries or affiliates, so long as you have no active participation in the business of such corporation. In addition, the provisions of this Section 5 shall not be violated by you commencing employment with a subsidiary, division, or unit of any entity that engages in a business in competition with the Company or any of its subsidiaries or affiliates so long as you and such subsidiary, division or unit do not engage in a business in competition with the Company or any of its subsidiaries or affiliates.

6. Tolling. In the event you violate any of the provisions of Section 3, 4, or 5, you acknowledge and agree that the post-termination restrictions contained in Sections 3, 4, and 5 shall be extended by a period of time equal to the period of such violation, it being the intention of the parties hereto that the running of the applicable post-termination restriction period shall be tolled during any period of such violation.

7. Cooperation. Upon the receipt of reasonable notice from the Company (including outside counsel), you agree that while employed by the Company and thereafter, you will respond and provide information with regard to matters in which you have knowledge as a result of your employment with the Company, and you will provide reasonable assistance to the Company, its affiliates, and their respective representatives in defense of any claims that may be made against the Company or its affiliates, and you will assist the Company and its affiliates in the prosecution of any claims that may be made by the Company or its affiliates, to the extent that such claims may relate to the period of your employment with the Company. You agree to promptly inform the Company if you become aware of any lawsuits involving such claims that

may be filed or threatened against the Company or its affiliates. You also agree to promptly inform the Company (to the extent that you are legally permitted to do so) if you are asked to assist in any investigation of the Company or its affiliates (or their actions), regardless of whether a lawsuit or other proceeding has then been filed against the Company or its affiliates with respect to such investigation, and shall not do so unless legally required. Upon presentation of appropriate documentation, the Company shall pay or reimburse you for all reasonable out-of-pocket travel, duplicating, and telephonic expenses incurred by you in complying with this Section 7.

8. Remedies. You hereby acknowledge and agree that monetary damages may not be a sufficient remedy for any breach of this letter agreement, including, without limitation, a breach of the covenants contained in this letter agreement and that the Company (or its affiliates) shall be entitled, without waiving any other rights or remedies, to obtain legal, injunctive, or equitable relief as may be deemed proper by a court of competent jurisdiction, without obligation to post any bond. In the event of a material breach of this letter agreement by you, as determined by a court of competent jurisdiction, of Section 3, 4, 5, 6, or 7 hereof, any Severance Payments, COBRA Payments, Equity Vesting, and any other benefit of any type being paid or provided to you pursuant to this letter agreement or otherwise shall immediately cease upon such written judgment, and solely to the extent determined by a court of competent jurisdiction, you shall immediately repay the Company for the value of any Severance Payments, COBRA Payments, Equity Vesting (determined as of the date of termination), and any other benefit of any type that is or was paid or provided to you pursuant to this letter agreement (other than $1,000).

9. Reasonableness. You acknowledge and agree that the restrictions contained in this letter agreement are reasonable and will not prevent you from finding other employment if your employment with the Company ends. You also acknowledge and agree that if you use the Company's (or its affiliates') confidential information, or compete with the Company (or its affiliates) in violation of the terms of this letter agreement, that you will be causing the Company and its affiliates irreparable harm.

10. Severability; Revision by Court. The provisions of this letter agreement shall be deemed severable and the invalidity or unenforceability of any provision shall not affect the validity or enforceability of the other provisions hereof. If any provision in this letter agreement is found by a court of competent jurisdiction to be unenforceable or unreasonable as written, you and the Company hereby specifically and irrevocably authorize and request said court to revise the unenforceable or unreasonable provisions in a manner that shall result in the provision being enforceable while remaining as similar as legally possible to the purpose and intent of the original.

11. Miscellaneous. The terms and conditions of Annexes A, B, C, D, and E are incorporated herein and made a part hereof by reference as if fully set forth herein. This letter agreement along with the Confidentiality Agreement and the CIC Severance Agreement constitute the entire agreement and understanding of the parties hereto with respect to the obligations addressed herein and supersedes all prior or contemporaneous oral or written agreements regarding the subject matter hereof. Any addition or modification to this letter agreement, or waiver of any provision hereof, must be in writing and signed by the parties hereto.

12. Successors and Assigns. You understand and agree that you cannot assign or otherwise transfer any of your obligations under this letter agreement. You also understand and agree that the Company or its affiliates may, at its option, assign or transfer its rights under this letter agreement to another organization or individual. You understand and agree that if there is an assignment or transfer of the Company's (or any of its affiliates') rights under this letter agreement, then this letter agreement will continue to be effective, will continue to bind you, and will inure to the benefit of the organization or individual to whom the transfer or assignment is made.

13. Governing Law; Jurisdiction. This letter agreement shall be governed by the laws of the Commonwealth of Massachusetts without regard to conflicts of laws principles. Any action or proceeding seeking to enforce any provision of, or based on any right arising out of, this letter agreement may be brought against either party only in the courts of the Commonwealth of Massachusetts. Both parties hereby irrevocably consent to the jurisdiction of any such court in any such action or proceeding and waive any objection to venue laid in such courts.

14. No Mitigation. In no event shall you be obligated to seek other employment or take any other action by way of mitigation of the amounts payable to you under any of the provisions of this Agreement, nor shall the amount of any payment hereunder be reduced by any compensation earned by you as a result of employment by a subsequent employer, except as provided in Section 1(a)(ii)(y) hereof.

15. Survival of Provisions**.** The obligations contained in Sections 3, 4, 5, 6, 7, and 8 hereof, as well as those set forth in the Confidential Proprietary Rights and Non-Disclosure Agreement attached as Annex E (the "Confidentiality Agreement") shall survive the termination of your employment with the Company and shall be fully enforceable thereafter

17. Termination During Change in Control Period**.** You entered into a Change in Control Agreement with the Company as of [_____] (the "CIC Severance Agreement"). In the event of a termination of your employment with the Company during the Change in Control Period (as defined in the CIC Severance Agreement), then you shall be entitled to severance benefits under the CIC Severance Agreement in accordance with its terms and conditions, and you shall have no rights or entitlements under Section 1 of this letter agreement, including, without limitation, the Severance Payments, COBRA Payments, and Equity Vesting. With respect to the application and effectiveness of the CIC Severance Agreement, that agreement is intended to apply solely if a Change in Control thereunder is a change in control event as defined under the Treasury Regulations under Code Section 409A.

19. Limits and Termination of this Letter Agreement. This letter agreement does not apply to any termination of employment that occurs after the end of the Termination Period. In the event that, during the Termination Period, your employment is not terminated without Cause or you do not resign for Good Reason or there is no New CEO by January 31, 2019, this letter agreement shall expire at the end of the Termination Period.

20. Legal Counsel. You have been advised and encouraged to seek your own counsel to assist you with this letter agreement. **YOU ACKNOWLEDGE THAT YOU HAVE HAD THIS LETTER AGREEMENT IN YOUR POSSESSION FOR AT LEAST FORTY-EIGHT (48) HOURS PRIOR TO SIGNING IT, YOU HAVE HAD REASONABLE OPPORTUNITY TO READ IT AND TO OBTAIN INDEPENDENT LEGAL ADVICE AS TO ITS PROVISIONS, AND FULLY UNDERSTAND ITS CONTENTS.**

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

If the terms of this letter agreement are acceptable to you, please confirm your agreement by executing below and returning a signed copy of it to me.

Sincerely,

Sarepta Therapeutics, Inc.

By: _____
Edward Kaye, MD
President and Chief Executive Officer

Acknowledged and Agreed this 27th day of April, 2017

_____

Annex A

Definitions

"Board of Directors" means: the Board of Directors of the Company.

"Cause" means:

(a) Your substantial and repeated failure to attempt in good faith to perform your duties or follow the reasonable and legal written direction of the Chief Executive Officer (after the Company has provided you with written notice of such failure and your failure to cure within thirty (30) days of your receipt of such notice);

(b) Your willful material misconduct with respect to any material aspect of the business of the Company;

(c) Your indictment for, conviction of, or pleading of guilty or nolo contendere to, a felony or any crime involving moral turpitude;

(d) Your performance of any material act of theft, fraud, or malfeasance in connection with the performance of your duties to the Company;

(e) The triggering of a disclosure obligation under the federal securities laws, as reasonably determined by the Company based upon advice of outside counsel, arising out of your act or omission before or during employment by the Company resulting from (i) you receiving notice from any governmental entity or self-regulatory organization alleging a violation of law, rule, or regulation and notifying you that it plans to indict or formally charge you or that you are indicted or formally charged, by any governmental entity or self-regulatory organization alleging a violation of a law, rule, or regulation; (ii) the Securities and Exchange Commission sending you a "Wells Notice"; or (iii) you being or becoming a party to any civil matter or a subject or target in any criminal matter, in each case by any governmental entity or self-regulatory organization alleging a violation of a law, rule, or regulation; or

(f) A material breach of this Agreement or a material violation of the Company's code of conduct or other written material policy (after the Company has provided you with written notice of such breach or violation and your failure to cure within thirty (30) days of your receipt of such notice).

"Code" means: the Internal Revenue Code of 1986, as amended.

"Commencement Date" means: August 1, 2017.

"Good Reason" means: the occurrence of any of the following events, without your express written consent, unless such events are cured by the Company within thirty (30) days following written notification by you to the Company that you intend to terminate your employment for one of the reasons set forth below:

(a) material diminution in your base salary at the rate or your bonus target at the percentage, in each case, as in effect immediately prior to the reduction or the failure to pay you any salary or any earned and due bonus or incentive payments; or

(b) material diminution in your duties, authorities or responsibilities (other than temporarily while physically or mentally incapacitated or as required by applicable law and other than in connection with any service on any informal management committees associated with the Company or its affiliates); or

(c) a change by the Company in the location at which you perform your principal duties for the Company to a new location that is more than fifty (50) miles from the Company's current location in Cambridge, Massachusetts.

You shall provide the Company with a written notice detailing the specific circumstances alleged to constitute Good Reason within thirty (30) days after the first occurrence of such circumstances (or any claim of such circumstances as "Good Reason" shall be deemed irrevocably waived by you), and in no event shall you be entitled to resign for "Good Reason" more than one hundred and eighty (180) days following the occurrence of any event alleged to constitute "Good Reason."

"Protection Period One" means: the period starting on the Commencement Date and ending on May 31, 2018.

"Protection Period Two" means: the period starting on June 1, 2018 and ending on January 31, 2019.

"Termination Period" means: period starting on the first day of Protection Period One and ending on the last day of Protection Period Two.

Annex B

Code Section 409A & 4999/280G Matters

Section 409A Matters

a. It is intended that the provisions of the letter agreement comply with, or be exempt from, Code Section 409A, and all provisions of the letter agreement shall be construed in a manner consistent with the requirements for avoiding taxes or penalties under Code Section 409A. Notwithstanding the foregoing, the Company shall have no liability with regard to any failure to comply with Code Section 409A so long as it has acted in good faith with regard to compliance therewith.

b. If, under the letter agreement, an amount is to be paid in two or more installments, for purposes of Code Section 409A, each installment shall be treated as a separate payment.

c. A termination of employment shall not be deemed to have occurred for purposes of any provision of the letter agreement providing for the payment of amounts or benefits upon or following a termination of employment unless such termination is also a "Separation from Service" within the meaning of Code Section 409A and, for purposes of any such provision of the letter agreement, references to a "resignation," "voluntary termination," "termination," "termination of employment" or like terms shall mean Separation from Service.

d. If you are deemed on the date of termination of your employment to be a "specified employee" within the meaning of that term under Section 409A(a)(2)(B) of the Code and using the identification methodology selected by the Company from time to time, or if none, the default methodology, then:

   i. With regard to any payment, the providing of any benefit or any distribution of equity upon Separation from Service that constitutes "deferred compensation" subject to Code Section 409A, such payment, benefit or distribution shall not be made or provided prior to the earlier of (i) the expiration of the six-month period measured from the date of your Separation from Service or (ii) the date of your death; and

   ii. On the first day of the seventh month following the date of your Separation from Service or, if earlier, on the date of your death, all payments delayed pursuant to this Section (d) (whether they would otherwise have been payable in a single sum or in installments in the absence of such delay) shall be paid or reimbursed to you in a lump sum, and any remaining payments and benefits due under the letter agreement shall be paid or provided in accordance with the normal dates in accordance with the terms of the letter agreement.

   In determining the amounts that are subject to the six-month delay requirement described above, the Company shall use all exclusions from the six-month delay rule that are available to the payments made to you. Please be advised that the Company reserves the right to adopt an alternate method of complying with the six-month delay requirement which may result in you being deemed a specified employee.

e. Whenever a payment under the letter agreement specifies a payment period with reference to a number of days (e.g., "payment shall be made within thirty (30) days following the date of termination"), the actual date of payment within the specified period shall be within the sole discretion of the Company.

Section 4999/280G Matters

If any payment or benefit (including payments and benefits pursuant to this letter agreement) that you would receive from the Company or in connection with a change of effective ownership or control of the Company ("Transaction Payment") would (i) constitute a "parachute payment" within the meaning of Section 280G of the Code, and (ii) but for this provision, be subject to the excise tax imposed by Section 4999 of the Code (the "Excise Tax"), then the Company shall cause to be determined, before any amounts of the Transaction Payment are paid to you, which of the following two alternative forms of payment would result in your receipt, on an after-tax basis, of the greater amount of the Transaction Payment notwithstanding that all or some portion of the Transaction Payment may be subject to the Excise Tax: (1) payment in full of the entire amount of the Transaction Payment (a "Full Payment"), or (2) payment of only a part of the Transaction Payment so that you receive the largest payment possible without the imposition of the Excise Tax (a "Reduced Payment").

For purposes of determining whether to make a Full Payment or a Reduced Payment, the Company shall cause to be taken into account all applicable federal, state and local income and employment taxes and the Excise Tax (all computed at the highest applicable marginal rate, net of the maximum reduction in federal income taxes which could be obtained from a deduction of such state and local taxes). If a Reduced Payment is made, (x) you shall have no rights to any additional payments and/or benefits constituting the Transaction Payment, and (y) reduction in payments and/or benefits shall occur in the manner that results in the greatest economic benefit to you as determined in this paragraph. If more than one method of reduction will result in the same economic benefit, the portions of the Payment shall be reduced pro rata.

The independent registered public accounting firm engaged by the Company as of the day prior to the effective date of the change of ownership or control of the Company shall make all determinations required to be made under this Annex B. If the independent registered public accounting firm so engaged by the Company is serving as accountant or auditor for the individual, entity or group effecting the change of control, the Company shall appoint a nationally recognized independent registered public accounting firm that is reasonably acceptable to you (and such acceptance shall not be unreasonably withheld) to make the determinations required hereunder. The Company shall bear all reasonable expenses with respect to the determinations by such independent registered public accounting firm required to be made hereunder. The independent registered public accounting firm engaged to make the determinations under this Annex B shall provide its calculations, together with detailed supporting documentation, to the Company and you within fifteen (15) calendar days after the date on which your right to a Transaction Payment is triggered or such other time as reasonably requested by the Company or you. If the independent registered public accounting firm determines that no Excise Tax is payable with respect to the Transaction Payment, either before or after the application of the Reduced Amount, it shall furnish the Company and you with detailed supporting calculations of its determinations that no Excise Tax will be imposed with respect to such Transaction Payment. Any good faith determinations of the accounting firm made hereunder shall be final, binding, and conclusive upon the Company and you.

Annex C

Outstanding Equity Awards

Grant Date	**Type of Equity Award**	**Vesting Schedule and Performance Measure (if any)**

Annex D

Form of Waiver and Release Agreement

Annex E

Confidential Proprietary Rights and Non-Disclosure Agreement

EXHIBIT 21.1

Sarepta Therapeutics, Inc.

Subsidiaries of the Registrant

Name	Jurisdiction of Incorporation
ST International Holdings, Inc.	Delaware, USA
STIH Two, Inc.	Delaware, USA
Sarepta Securities Corp.	Massachusetts, USA
Sarepta International CV	Netherlands

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

The Board of Directors
Sarepta Therapeutics, Inc.:

We consent to the incorporation by reference in the registration statements (Nos. 333-105412, 333-109015, 333-133211, 333-138299, 333-150021, 333-160922, 333-180258, 333-184807, 333-209709, 333-45888, 333- 68502, 333-86039, 333-86778, and 333-93135) on Form S-3 and (Nos. 333-101826, 333-172823, 333- 175031, 333-192287, 333-199037, 333-209710, 333-213022, 333-34047, 333-49994, 333-49996, and 333-221271) on Form S-8 of Sarepta Therapeutics, Inc. and subsidiaries of our reports dated March 1, 2018, with respect to the consolidated balance sheets of Sarepta Therapeutics, Inc. and subsidiaries as of December 31, 2017 and 2016, and the related consolidated statements of operations and comprehensive loss, stockholders' equity, and cash flows for each of the years in the three-year period ended December 31, 2017, and the related notes collectively, the "consolidated financial statements", and the effectiveness of internal control over financial reporting as of December 31, 2017, which reports appear in the December 31, 2017 annual report on Form 10-K of Sarepta Therapeutics, Inc. and subsidiaries.

(signed) KPMG LLP

Cambridge, Massachusetts
March 1, 2018

EXHIBIT 31.1

CERTIFICATION

I, Douglas S. Ingram, certify that:

1. I have reviewed this Annual Report on Form 10-K 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.

March 1, 2018

/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 Annual Report on Form 10-K 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.

March 1, 2018

/s/ Sandesh Mahatme
Sandesh Mahatme
Executive Vice President, Chief Financial 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 the Annual Report of Sarepta Therapeutics, Inc. on Form 10-K for the fiscal year ended December 31, 2017, 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 Annual Report on Form 10-K fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

March 1, 2018

/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 Report on Form 10-K 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 the Annual Report of Sarepta Therapeutics, Inc. on Form 10-K for the fiscal year ended December 31, 2017, 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 Annual Report on Form 10-K fairly presents, in all material respects, the financial condition and results of operations of Sarepta Therapeutics, Inc.

March 1, 2018

/s/ Sandesh Mahatme

Sandesh Mahatme,
Executive Vice President, Chief Financial 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 Report on Form 10-K 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.