Sarepta Therapeutics, Inc.
Form 10-K
February 25, 2016

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

x ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the fiscal year ended December 31, 2015

Or

o 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<br>Suite 415<br>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 o No x

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 o No x

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 x No o

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 x No o

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. x

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

| | |
|---|---|
| Large accelerated filer x | Accelerated filer o |
| Non-accelerated filer o (Do not check if a smaller reporting company) | Smaller reporting company o |

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2015 was approximately $1,263,325,580.

The number of outstanding shares of the registrant's common stock as of the close of business on February 19, 2016 was 45,666,357.

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 2016 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.

Sarepta Therapeutics, Inc.

FORM 10-K INDEX

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. The statements contained in this Annual Report on Form 10-K that are not purely historical are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. 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 expression variations or negatives of these words. You should read these statements carefully because they discuss future expectations, contain projections of future results of operations or financial condition, or state 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 expectations regarding the timing of research, development, preclinical and clinical trial results, data and analyses relating to the safety profile and potential clinical benefits of our product candidates, including eteplirsen, our phosphorodiamidate morpholino oligomer ("PMO") chemistries, our other PMO-based chemistries and our other RNA-targeted technologies;
- our expectations regarding the Food and Drug Administration's ("FDA") interpretation of our data and information on our product candidates, PMO and PMO-based chemistries and RNA-targeted technologies and the impact of the FDA's interpretations on our FDA submissions (including our investigational new drug applications ("INDs") and new drug applications ("NDAs")), filing decisions by the FDA, potential advisory committee meeting dates and advisory committee recommendations, and FDA product approval decisions and related timelines;
- our estimates regarding how long our currently available cash, cash equivalents and investments will be sufficient to finance our operations and business plans and statements about our future capital needs;
- our current and planned investment in and activities in preparation for a potential commercial launch of eteplirsen, including continuing to negotiate and enter into commercial and supply contracts, scaling up manufacturing and hiring commercial positions and the impact of winding down or terminating these commitments if the FDA does not approve our eteplirsen NDA;
- our ability to raise additional funds to support our business plans and the impact of our credit and security agreement with MidCap Financial on our financial condition and future operations;
- our expectations regarding our ability to become a leading developer and marketer of PMO-based and RNA-targeted therapeutics and commercial viability of our product candidates, chemistries and technologies;
- the potential safety, efficacy, potency and utility of our product candidates, chemistries and technologies in the treatment of Duchenne muscular dystrophy ("DMD") and in rare, infectious and other diseases;
    - our expectations regarding the timing, completion and receipt of results from our ongoing development programs for our pipeline of product candidates including their potential consistency with prior results;
- our ability to effectively manage the clinical trial process for our product candidates on a timely basis, including our ability to conduct a placebo-controlled confirmatory study for eteplirsen in the U.S. using an exon 53-skipping product candidate;
- our expectations regarding our ability to engage a number of manufacturers with sufficient capability and capacity to meet our manufacturing needs, including with respect to the manufacture of subunits, drug substance ("APIs") and drug product, within the time frames and quantities needed to provide our product candidates, including eteplirsen, to patients in larger scale clinical trials or in potential commercial quantities, and meet regulatory and Company quality control requirements;
- the impact of regulations as well as regulatory decisions by the FDA and other regulatory agencies on our business, including with respect to our eteplirsen NDA submission as well as the development of our product candidates and our financial and contractual obligations;
- our expectations regarding the potential markets for our product candidates;
- our expectations regarding our manufacturing and scale-up techniques and our ability to synthesize and purify our product candidates to adequately support clinical development and potential commercialization;

- the potential acceptance of our product candidates, if introduced, in the marketplace;
- the possible impact of competing products on our product candidates and our ability to compete against such products;

- the impact of potential difficulties in product development, manufacturing, or the commercialization of our product candidates, including difficulties in establishing the commercial infrastructure necessary for the commercialization of eteplirsen;
- our expectations regarding partnering opportunities and other strategic transactions;
- 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;
- 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 of our product candidates;
- our ability to successfully challenge the patent positions of our competitors and successfully defend our patent positions in the actions that the United States Patent and Trademark Office ("USPTO") may take or has taken with respect to our patent claims or those of third parties, including with respect to interferences that have been declared between our patents and patent applications held by BioMarin Pharmaceuticals, Inc., relating to eteplirsen and SRP-4053 and our expectations regarding the impact of these interferences on our business plans, including our current commercialization plans for eteplirsen and SRP-4053;
- our ability to operate our business without infringing the intellectual property rights of others;
- our ability to enter into contracts, including collaborations or licensing agreements, with respect to our technology and product candidates, with third parties, including government entities;
- our estimates regarding future revenues, research and development expenses, other expenses, capital requirements and payments to third parties;
- the timing and outcomes of ongoing interference proceedings and related appeals;
- the impact of litigation on us, including actions brought by stockholders;
- 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;
- our expectations relating to potential funding from government and other sources for the development of some of our product candidates;
- the impact of the potential achievement of performance conditions and milestones relating to our restricted stock awards;
- our beliefs and expectations regarding milestone, royalty or other payments that could be due to third parties under existing agreements; and
- our succession plan, including the search for a permanent full-time CEO and the effect that the changes in management could have on the Company, its business plans and its regulatory and clinical discussions and relationships.

All forward-looking statements are based on information available to us on the date of this Annual Report on Form 10-K and we will not update any of the forward-looking statements after the date of this Annual Report on Form 10-K, except as required by law or the rules and regulations of the U.S. Securities and Exchange Commission.("SEC"). 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 certain risks and uncertainties that could cause actual results to differ materially from those anticipated in the forward-looking statements. Applicable risks and uncertainties include, among others, the fact that: the FDA may further delay our PDUF date or may not approve eteplirsen as a DMD therapeutic; we may be delayed or may not be able to comply with the FDA's requests for additional information in connection with our eteplirsen NDA; the additional information and data we collect for eteplirsen may not be consistent with prior data or results or may not support a positive advisory committee vote or recommendation relating to our eteplirsen NDA, if any, or approval of eteplirsen by the FDA; we may be delayed in and may not be able to successfully conduct or obtain positive results in our current and planned clinical trials for eteplirsen and other product candidates in our pipeline; we may not have sufficient funds to execute on our business plans and strategy; we may not be able to obtain regulatory

approvals for our product candidates in a timely manner nor achieve commercial viability; we may not be able to incorporate our PMO and other technology into therapeutic commercial products; we may not be able to successfully navigate the uncertainties related to regulatory processes; we may not be able to demonstrate acceptable levels of safety, efficacy and

quality in our product candidates through our preclinical and clinical trials; compliance with environmental laws could have a negative impact on our business if we are not able to effectively manage our real estate, manufacturing and other Company operations that may deal with hazardous materials; we rely on third parties to provide service, including the manufacturing of our product candidates, in connection with our preclinical and clinical development programs and commercialization plan and we may not be able to secure the service or quality of service we need from third parties; the pharmaceutical industry is subject to greater government scrutiny and regulation, and we may not be able to respond to changing laws and regulations affecting our industry, including any reforms to the regulatory approval process administered by the FDA or changing enforcement practices related thereto; we may not be able to obtain and maintain patent protection for our product candidates, preserve our trade secrets or prevent third parties from infringing on our proprietary rights; we may not be able to capitalize on our executive team's relationships and expertise to meet our expected timelines for regulatory submissions, clinical development plans and bringing our product candidates to market; we may not be able to hire and retain key personnel or attract qualified personnel, including a permanent full-time CEO; we may not be able to establish and maintain arrangements with third parties who are able to meet manufacturing needs for large-scale clinical trials or potential commercial needs within sufficient timelines or at acceptable costs; competitive products and pricing may have a negative impact on our business; there are uncertainties associated with our future capital needs; we may not be able to raise additional funds to execute our business plans; we may not be able to attract sufficient capital or to enter into strategic relationships; the outcome of our patent interferences, investigations and litigation and associated damages and expenses is uncertain; and those risks and uncertainties discussed in Part I, Item 1 "Business" and Item 1A "Risk Factors" of this Annual Report on Form 10-K.

PART I

Item 1. Business.

Overview

We are a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other diseases. Applying our proprietary, highly-differentiated and innovative platform technologies, we are able to target a broad range of diseases and disorders through distinct RNA-targeted mechanisms of action. We are primarily focused on rapidly advancing the development of our potentially disease-modifying DMD drug candidates, including our lead DMD product candidate, eteplirsen, designed to skip exon 51. On August 25, 2015, we announced the FDA filing of our NDA for eteplirsen for the treatment of DMD amenable to exon 51 skipping. The FDA postponed the Advisory Committee meeting for the review of the eteplirsen NDA previously scheduled for January 22, 2016 due to severe weather. On February 8, 2016, we announced that the FDA notified us that the Prescription Drug User Fee Act ("PDUFA") action date for eteplirsen has been extended to May 26, 2016 due to our submission of four-year clinical effectiveness data on January 8, 2016 to the FDA, which the FDA designated as a major amendments to the eteplirsen NDA. We are also developing therapeutics using our technology for the treatment of drug resistant bacteria and infectious, rare and other human diseases.

Objectives and Business Strategy

We believe that our highly-differentiated and proprietary RNA-targeted technology platforms can be used to develop novel pharmaceutical products to treat a broad range of diseases and address key unmet medical needs. We intend to leverage our RNA-targeted technology platforms, organizational capabilities and resources to become a leading developer and marketer of RNA-targeted therapeutics, including for the treatment of rare, infectious and other diseases, with a diversified portfolio of product candidates. In pursuit of this objective, we intend to engage in the following activities:

- advancing the development of eteplirsen and our other drug candidates for the treatment of DMD to realize the product opportunities of such candidates and potentially provide significant clinical benefits;
- further explore funding, collaboration and other opportunities to support continued development of our rare, infectious and other research and development programs; and
- leveraging our RNA-targeted technology platforms to identify product candidates in additional therapeutic areas and explore various strategic opportunities, including potential partnering, licensing or collaboration arrangements with industry partners.

Development Programs

DMD. Our lead program, with a pipeline of ten product candidates, 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. Currently, there are no approved disease-modifying therapies for DMD in the U.S. If we are successful in our development efforts, eteplirsen, our lead DMD product candidate, and our follow-on exon-skipping DMD candidates would address an unmet medical need. We are in the process of conducting several studies with eteplirsen and our follow-on DMD candidates including:

Eteplirsen

.

Study 4658-US-202 (“Study 202”) – an ongoing U.S. open label extension of our initial Phase IIb clinical trial which was completed in 2012, with over four and a half years of data collected as of February 2016 (inclusive of the primary study);

- Study 4658-301/PROMOVI (“Study 301”) – a confirmatory U.S. study, started in 2014, with a treated arm evaluating the safety and efficacy of eteplirsen in ambulatory DMD patients amenable to exon-51-skipping and an untreated concurrent control arm with patients that are not amenable to exon-51-skipping;
- Study 4658-204 (“Study 204”) – a U.S. study, started in 2014, evaluating the safety and tolerability of eteplirsen in patients with advanced stage DMD; and
- Study 4658-203 (“Study 203”) – a U.S. study, started in 2015, evaluating the safety and tolerability of eteplirsen in patients with early stage DMD.

Follow-on Exons

- Study 4053-101 (“SKIP-NMD”) – a European Union (“E.U.”) study we are conducting in collaboration with a consortium of scientific, clinical and industrial partners in the E.U. Part I of the study, started in 2014, is a dose titration,

placebo-controlled study, evaluating the safety, tolerability and pharmacokinetics of SRP-4053. Part II of the study, started in 2015, evaluates the safety and efficacy of SRP-4053 in patients with DMD amenable to exon 53 skipping;

- Study 4045-101 – a randomized, double blind, placebo controlled, dose titration study in the U.S., started in 2015, evaluating the safety, tolerability and pharmacokinetics of SRP 4045 in advanced stage patients with DMD amenable to exon 45 skipping, followed by an open label safety and efficacy evaluation.

In addition, we are currently working towards starting a second confirmatory study to support eteplirsen approval in 2016, which will evaluate the safety and efficacy of our product candidates designed to skip exons 45 and 53. We have satisfactorily responded to the FDA's inquiries on preclinical data for this study relating our exon-53 product candidate.

Infectious Diseases.

Anti-virals. The antisense technology platform has been applied to the development of potential therapeutics for Ebola and Marburg hemorrhagic fever and pandemic H1N1 influenza viral infections. Though our original discovery and development contracts from the Department of Defense ("DoD") are no longer active, we remain active partners with the National Institutes of Health ("NIH") and the National Institute of Allergy and Infectious Diseases ("NIAID") for continued development of our influenza product candidate. Following encouraging preclinical results, in February 2012, we announced Phase I results for the Ebola, Marburg and influenza product candidates which appeared to be well tolerated and showed no drug-associated safety findings in the human study subjects. All three product candidates use our PMOplus® technology. We are open to partnership possibilities and other avenues to support further development of these Ebola, Marburg and influenza product candidates; however, if we do not succeed in these efforts, we will likely curtail their further development.

Discovery and Research Programs

Our discovery and research programs include collaborations with various parties and focus on developing therapeutics in rare, genetic, anti-bacterial, neuromuscular and central nervous system diseases. We are exploring the application of our proprietary PMO platform technology in various diseases.

Proprietary Manufacturing Techniques

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential commercialization. We have entered into certain manufacturing and supply arrangements with third parties which will in part utilize these techniques to support production of certain of our product candidates and their components. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support a clinical or commercial supply of our product candidates.

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.

We have not generated any revenue from product sales to date and there can be no assurance that revenue from product sales will be achieved. Even if we do achieve revenue from product sales, we are likely to continue to incur operating losses in the near term. 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, 2015, we had approximately $204.0 million of cash, cash equivalents and investments, consisting of $80.3 million of cash and cash equivalents, $112.2 million of short-term investments and $11.5 million of 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 technology, 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 U.S. Securities and Exchange Commission ("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 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.

Lead Development Program: Pipeline of Exon-Skipping PMO Product Candidates for Duchenne Muscular Dystrophy

DMD Background

DMD is one of the most common fatal genetic disorders affecting children (primarily boys) around the world. DMD is a devastating and incurable muscle-wasting disease associated with specific mutations in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. The absence of dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and fibrotic replacement. Females are rarely affected by the disorder. In the absence of dystrophin protein, affected individuals generally experience:

- 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 in their 20s to which they typically succumb.

There is currently no approved disease modifying treatment or cure for DMD in the U.S. The yearly cost of care for individuals with DMD is high and increases with disease progression. Although DMD is a rare disease, we believe it represents a substantial product opportunity due to the severity and inexorable progression of the symptoms.

Exon-Skipping Pipeline

The table below summarizes our DMD studies including the confirmatory trials we initiated in 2015 and our planned clinical trials:

| Exon Target Treatment | Study | Duration (weeks) | U.S./E.U. | Number of Patients | Status | DMD Population |
|---|---|---|---|---|---|---|
| Exon 51 | AVI-4658-33 | Single Dose | EU | 7 | Completed | 10-17 yrs, non-amb[(b)] |
| Exon 51 | AVI-4658-28 | 12 | EU | 19 | Completed | 5-15 yrs, amb |
| Exon 51 | 4658-US-201 | 28 | US | 12 | Completed | 7-13 yrs, amb |
| Exon 51 | 4658-US-202 [(a)] | 268 | US | 12 | Dosing/Enrollment closed (Data through 236 weeks) | 7-13 yrs, amb |
| Exon 51 | 4658-301 | 96 | US | 160 | Dosing | 7-16 yrs, amb |
| Exon 51 | 4658-204 | 96 | US | 24 | Dosing/Enrollment closed | 7-21 yrs, non-amb |
| Exon 51 | 4658-203 | 96 | US | 40 | Dosing | 4-6 yrs, amb |
| Exon 51 | 4658-102 | 48 | EU/US | 12 | Planned | 6 mos - 4 yrs |
| Exon 45 | 4045-101 | 120 | US | 12 | Dosing/Enrollment closed | 7-21 yrs, non-amb |
| Exon 53 | 4053-101 | 144 | EU | 48 | Dosing | 6-15 yrs, amb |
| Exon 45/53 | 4045-301 | 48 | EU/US | 99 | Planned | 7-16 yrs, amb |

(a) Weeks presented are inclusive of 28 completed weeks in study 4658-us-201.
(b) Amb denotes ambulatory

Eteplirsen. Eteplirsen, our lead DMD product candidate, is an antisense PMO therapeutic in Phase III clinical development for the treatment of individuals with DMD who have an error in the gene coding for dystrophin that is amenable to skipping exon 51. Eteplirsen targets the most frequent series of mutations that cause DMD. Eteplirsen has been granted orphan drug designation in the U.S. and E.U. In 2007, the FDA granted eteplirsen fast track status and we are continuing to discuss with the FDA the possibility of expedited regulatory programs for eteplirsen.

For approximately four years, we have been collecting data on the safety and efficacy of eteplirsen through a Phase llb open label extension study, Study 202. In this study, biopsies were taken from patients at 48 weeks and, using different physicochemical methods on the tissue samples collected, we measured increases in novel dystrophin production. In July 2014, we announced that at 144 weeks (i) patients evaluable (n=10) on the 6-minute walk test (“6MWT”) showed a decline in walking ability at a rate slower than would be expected based on available DMD natural history data and (ii) a continued stabilization of respiratory muscle function was observed, as assessed by pulmonary function tests. In January 2015, we announced that at 168 weeks (i) continued ambulation across all patients evaluable on the 6MWT was observed, however, all patients showed a decline in distance walked on this measure since the week 144 time point, (ii) stability of respiratory muscle function was observed, as assessed by pulmonary function tests and (iii) good tolerability and no clinically significant treatment-related adverse events or serious adverse events were reported. In October 2015, we announced additional clinical efficacy and safety data that demonstrated that (i) eteplirsen provided a statistically significant advantage of 151 meters in the ability of study participants to walk at three years versus an external DMD control, (ii) eteplirsen-treated patients (n=12) experienced a slower rate of decline through week 192 than external DMD controls and (iii) the eteplirsen safety profile remained consistent with prior results. After approximately four years of treatment with eteplirsen, results of the 6MWT at 216 weeks showed continued ambulation of the 10 evaluable patients. In January 2016, we announced more than four years of data for 11 of the 13 external control patients that demonstrated 10 of the 11 patients lost ambulation, a statistically significant

difference. Please read Item 7, Management's Discussion and Analysis of Financial Condition and Results of Operations — Summary and Timeline of Eteplirsen Data Disclosure included elsewhere in this Annual Report on Form 10-K for more information.

On August 25, 2015, we announced the FDA's filing of our NDA for eteplirsen for the treatment of DMD amenable to exon 51 skipping. Eteplirsen is under priority review with a current PDUFA action date of May 26, 2016. Please read Overview and Government Regulation for additional information.

Additional DMD Product Candidates. In addition to our lead product candidate, eteplirsen, we are pursuing development of additional exon-skipping drugs, to support our broad-based development program for the treatment of DMD. Our additional nine product candidates target skipping of exons 8, 35, 43, 44, 45, 50, 52, 53 and 55 and are at various stages of development.

Exon 53. To support certain clinical proof of concept studies and investigational new drug ("IND") -enabling activities for an exon 53-skipping therapeutic, we announced in November 2012 that we are collaborating in the SKIP-NMD Consortium with University College London's scientist, Professor Francesco Muntoni, M.D., the Dubowitz Neuromuscular Centre, the Institute of Child Health and other scientists from the E.U. and the U.S. In connection with this collaboration, the Consortium received an E.U. Health Innovation-1 2012 collaborative research grant (grant agreement No. 305370) to support development of an exon 53-skipping therapeutic, based on our PMO chemistry. Targeting exon 53 with this technology will potentially address one of the most prevalent

sets of mutations in DMD that are amenable to exon-skipping (eg. deletions of exons such as 42-52, 45-52, 47-52, 48-52, 49-52, 50-52, 52 or 54-58). We completed Part I and are currently conducting Part II of a Phase I/IIa clinical trial for an exon 53-skipping product candidate in the E.U.

Exon 45. In collaboration with Children's National Medical Center ("CNMC") in Washington, D.C. and the Carolinas Medical Center ("CMC") in Charlotte, N.C., we have developed an exon 45-skipping product candidate. This collaboration is funded primarily through two grants, one from DoD's Congressionally Directed Medical Research Program to CNMC and the other from the National Institute of Neurological Disorders and Stroke to the CMC. This funding is intended to pursue the most promising treatments for DMD. The collaboration has supported a series of Good Laboratory Practice ("GLP") toxicology studies for an exon 45-skipping drug candidate based on our PMO chemistry. We have initiated and completed enrollment of a dose-ranging study for our exon 45-skipping product candidate in the U.S. (Study 4045-101).

Additionally, we are also planning to initiate a placebo-controlled confirmatory study with product candidates designed to skip exons 45 and 53 (Study 4045-301).

Exons 8, 35, 43, 44, 50, 52 and 55. Selection of lead sequences for product candidates designed to skip each of these exons are underway. Although we were previously collaborating with the NIH for the development of an exon 50 product candidate, we mutually agreed to terminate our Collaborative Research and Development Agreement in February 2013 and we are now developing an exon 50 skipping candidate utilizing our own research and development capabilities.

Our DMD program is part of our larger pan-exon strategy for the development of drug candidates to address the most prevalent exon deletions in the DMD population. Because the majority of DMD patients have exon deletions that cluster together, a small number of exon-skipping therapies will potentially be disease-modifying for a relatively large percentage of DMD patients. Approximately 75-80% of the total DMD population is potentially treatable with exon-skipping therapeutics.

Development Programs: Infectious Diseases

Our infectious diseases therapeutic programs continue to evolve, and remain based on our translation inhibition through steric hindrance technology. The applications to date have included our proprietary PMOplus® chemistry for post-exposure and therapeutic medical countermeasures to hemorrhagic fever virus infections (including Ebola and Marburg viruses), and for pandemic and seasonal influenza A infection. The contracts funding the hemorrhagic fever virus programs have ended, and while we are no longer actively developing those products, we may consider a collaborative relationship in the future. We have an active partnership with the NIAID, an institute within the NIH, for ongoing clinical development of our influenza therapeutic candidate.

As previously reported, the Phase I human safety, tolerability and pharmacokinetics single ascending dose trials of the drug candidates for Ebola, Marburg and pandemic influenza A, and the multiple ascending dose trial of the Marburg drug candidate have been completed. The Phase I single ascending and multiple ascending dose clinical trial of the pandemic influenza drug candidate has now also been completed, and data are being analyzed.

These programs were vital to informing us in all aspects of the clinical applications, chemistry and materials development of the proprietary PMOs. The funding received to support the research and development of these

antiviral candidates from the U.S. government represented substantially all of our revenues during those funding periods. As of December 31, 2014, we had completed all development activities of our contracts with the U.S. government. In 2015, key animal efficacy and human safety and pharmacokinetic data were published in the New England Journal of Medicine, Antimicrobial Agents and Chemotherapy, mBio, Antiviral Research and by the American Society of Microbiology Journals, as well as presented at a number of national and international scientific and industry conferences.

Discovery and Research Programs

Rare Diseases. We are researching the application of our proprietary peptide-conjugated PMO ("PPMO") technology to regulate progerin protein in progeria patients and in other diseases.

Anti-Bacterials. The rapid emergence of broad antibiotic resistance has underscored the urgent need for new paradigms in antimicrobial development. Our anti-bacterial program is focused on drug-resistant bacteria identified by the Centers for Disease Control and Prevention ("CDC") as urgent or serious threats to the U.S. healthcare system. Early research findings demonstrate that targeted PPMOs can successfully inhibit translation of essential structural genes such as acyl carrier protein ("acpP"), resistance proteins such as the NDM-1 metallo-b-lactamase, or those responsible for biofilm formation, which is critical for Burkholderia cepacia complex to evade host immune responses or systemic antibiotics such as cysteine protease cepI., which is responsible for biofilm expression. Additionally, though acpP alone can be bactericidal at clinically achievable concentration, data demonstrates that

co-administration of the PPMOs targeting NDM-1 can restore antibiotic activity of drugs like meropenem or imipenem to clinically achievable levels in high-level multidrug resistant Acinetobacter, E. coli, Klebsiella, and Burkholderia spp in both bench top and mouse models. Finally, we have also seen that PPMOs targeting structural genes such as acpP or quorum sensing genes such as cepI (responsible for biofilm expression) can penetrate and disrupt established biofilm; furthermore, the PPMOs targeting acpP can successfully kill the established bacterial colonies in Burkholderia cepacia models. We believe the results of this early research could have broad commercial applicability. We are exploring IND enabling studies now, and are open to partnership opportunities in the development of our anti-bacterial program.

Proprietary Platform Technology

PMO. The original PMO structure and variations of this structure that are so-called PMO-based are central to our proprietary chemistry platform. PMO and PMO-based therapeutics have been safely dosed in over 400 patients. PMO and PMO-based compounds are synthetic compounds that bind to complementary sequences of RNA by standard Watson-Crick nucleobase pairing. When targeted to mRNA, PMO and PMO-based compounds downregulate protein translation by steric blockade. The two key structural differences between PMO/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. Because of these modifications, PMO and PMO-based compounds are resistant to degradation by plasma and intracellular enzymes. Unlike the RNA-targeted technologies of siRNAs and DNA gapmers, PMO and PMO-based compounds operate by steric blockade rather than by cellular enzymatic degradation to achieve their biological effects. PMOs thus use a fundamentally different mechanism from these 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 and PMO-based compounds can 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 and PMO-based compounds significantly reduce 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 and PMO-based compounds do not exhibit coagulation and immune stimulatory effects, do not stimulate toll-like receptors (“TLRs”) 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 excellent safety and efficacy.

PPMO. The first of these novel chemistries is based on cell-penetrating PPMOs. Cellular uptake, potency, efficacy, and specificity of tissue targeting may be significantly enhanced.

PMOplus®. The second of these chemistries, PMOplus®, features the selective introduction positive charges to the phosphorodiamidate backbone. We believe that PMOplus® has potentially broad therapeutic applications, especially for anti-viral therapeutics.

PMO-X®. The third of these chemistries, PMO-X®, incorporates novel and proprietary chemical modifications to the PMO internucleoside 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.

We believe that our PMO and PMO-based technology platforms can be used to develop novel pharmaceutical products to treat a broad range of diseases and address key unmet medical needs. We intend to leverage our PMO and PMO-based technology platforms, organizational capabilities, and resources to become a leading developer and marketer of a diversified portfolio of PMO and PMO-based therapeutics, especially for the treatment of rare and infectious diseases.

Material Agreements

We believe that our RNA-targeted technology 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 November 2008, we entered into an exclusive license agreement with the University of Western Australia ("UWA"), for certain patents and technical information relating to the use of certain antisense sequences for the treatment of DMD and in April 2013, we entered into an agreement with UWA under which this license agreement 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. Our lead clinical candidate, eteplirsen, falls under the scope of the license granted under the Amended and Restated UWA License Agreement. Any future drug candidates developed for the treatment of DMD by exon skipping may or may not fall under the scope of the Amended and Restated UWA License Agreement.

Under the Amended and Restated UWA License Agreement, we are required to meet certain performance diligence obligations related to development and commercialization of products developed under the license. We believe we are currently in compliance with these obligations. In 2013, we made an initial up-front payment to UWA of $1.1 million upon execution of the Amended and Restated UWA License Agreement. We may be required to make additional payments to UWA of up to $6.0 million in aggregate based on successful achievement of certain development and regulatory milestones relating to eteplirsen and up to five additional product candidates and may also 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. As of December 31, 2015, we were not under any current obligation to make royalty payments to UWA until achievement of the first commercial sale.

Additionally, the agreement offers us the option of purchasing royalties upfront. Under this option, we may be required to make to UWA an up-front payment of $30.0 million as well as $20.0 million in aggregate contingency payments upon the successful achievement of certain commercial milestones.

The terms of the Amended and Restated UWA License Agreement will expire on a country-by-country basis on the expiration date of the last to expire valid claim or patent within the patents licensed to us under this agreement or upon the earliest to occur of the following:

- failure by us or UWA to cure a breach or default of any material obligation we each have under the agreement after notice from the non-breaching party within the specified time periods;
- a mutual agreement to terminate the agreement;
- by UWA in the event a party passes a resolution to wind-up or if a receiver, administrator, trustee or person performing similar functions is appointed by a court or liquidator over any of our assets; or
- upon our notice to UWA that we no longer desire to commercialize products covered under the agreement.

Currently, the latest date on which an issued patent covered by our agreement with UWA expires is November 2030 (not accounting for any patent term extension, supplemental protection certificate or pediatric extensions that may be available), however, patents granting from pending patent applications could result in a later expiration date.

Strategic Alliances

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, 2015, 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.

Under the terms of the sponsored research agreement, as amended, if we and any of our strategic partners elect to discontinue the development and commercialization of any product containing any molecular candidate arising or derived from the research sponsored by Charley's Fund for reasons other than safety or efficacy, we must grant to Charley's Fund an exclusive, royalty-bearing, fully-paid, worldwide license, with right of sublicense, to any such product. Depending on whether and when Charley's Fund obtains

a license to any such product, percentage royalty payments on net sales required to be made by Charley's Fund to us under the terms of the sponsored research agreement, as amended, would be in the mid-single-digits. Under the terms of the sponsored research agreement, as amended, if we are able to successfully commercialize any molecular candidate arising or derived from the research sponsored by Charley's Fund either through sales of products or through licensing or partnership arrangements with a third party that include rights for such third party to sell, distribute, promote or market such products or the underlying intellectual property, we are obligated to repay the research funds paid to us by Charley's Fund, up to an amount equal to the total amount of funds provided by Charley's Fund to us. In connection with this repayment obligation, we agreed that we would pay a mid-single-digit percentage royalty on net sales of products containing any molecular candidate arising or derived from the research sponsored by Charley's Fund and a mid-teens amount of any up-front cash and/or milestone payments received from a licensing or partnership arrangement with a third party with respect to such products (in each case, up to an amount equal to the total amount of funds provided by Charley's Fund to us). This agreement will be terminated by its own terms at the completion of the research being sponsored by Charley's Fund. Our technology upon which the agreement is based is covered by certain patents, the last of which expires following the termination of the agreement.

Previously, we noted unexpected toxicology findings in the kidney as part of our series of preclinical studies 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 studies 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.

Manufacturing

We believe we have developed proprietary state-of-the-art manufacturing and scale-up techniques that allow synthesis and purification of our product candidates to support clinical development as well as potential commercialization. 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. We currently do not have any of our own internal mid-to-large scale manufacturing capabilities to support our product candidates.

For our current development programs we have entered into supply agreements with certain large pharmaceutical manufacturing firms for the production of the custom raw materials required for PMO production and the APIs, for our product candidates.

For our DMD program, we are working with our existing manufacturers to increase our active pharmaceutical ingredient ("API") production capacity from mid-scale to large-scale. During 2016, we will also evaluate whether to increase our API production capacity to a commercial scale. This decision will depend in significant part on our discussions with the FDA in 2016 as well as our expectations regarding clinical trial needs and the potential feasibility and timing of the commercialization of eteplirsen.

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 DMD development efforts. Due to their technical expertise, experience in manufacturing our product candidates and sophistication of their manufacturing facilities and quality systems, we are considering our existing manufacturers, as well as other manufacturers with relevant expertise, for the further scale-up of the production of raw materials and APIs for our DMD program. Establishing a relationship with alternative suppliers can be a lengthy process and might cause delays in our development efforts. If we are required to seek alternative supply arrangements, the resulting delays and potential inability to find a suitable replacement could materially and adversely impact our business.

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

Sales and Marketing Strategy

We have not obtained regulatory approval for any of our product candidates. Due to the rare nature of DMD and the lack of disease-modifying treatments, patients suffering from DMD, together with their physicians, often have a high degree of organization and are well informed, which may simplify the identification of a target population for eteplirsen, our lead product candidate, if it is approved. We believe that, if approved for commercial sale, it will be possible to commercialize eteplirsen with a relatively small specialty sales force that calls on the physicians, foundations and other patient-advocacy groups focused on DMD. Our current expectation is to commercialize eteplirsen ourselves in the U.S. and we continue to take steps to establish the necessary commercial infrastructure we believe is needed for a potential marketing approval of eteplirsen. We will continue to evaluate whether to market our DMD product candidates outside of the U.S. ourselves or enter into arrangements with other pharmaceutical or biotechnology companies for the marketing and sale of our products outside the U.S. either globally or on a country-by-country basis.

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 and PMO-based technology platforms. 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 of February 9, 2016, we owned or controlled approximately 380 U.S. and corresponding foreign patents and 349 U.S. and corresponding foreign patent applications. We intend to protect our proprietary technology with additional filings as appropriate.

Our product candidates and our technology are primarily protected by composition of matter and use patents and patent applications. Currently, our clinical product candidates include eteplirsen for DMD. We have exclusively licensed patents from the UWA that provide primary patent protection for eteplirsen as follows:

| Patent Number | Country/Region | Patent Type | Expiration Date* |
|---|---|---|---|
| U.S. 7,807,816** | United States | Composition of Matter | February 23, 2026 |
| U.S. 7,960,541** | United States | Composition of Matter | June 28, 2025 |
| U.S. 8,486,907*** | United States | Methods of Use | June 28, 2025 |
| U.S. 9,018,368 | United States | Composition of Matter | June 28, 2025 |
| EP 1 766 010 B1 | Europe | Composition of Matter & Methods of Use | June 28, 2025 |

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

** Involved in U.S. Patent Interference No. 106,008.

***Involved in U.S. Patent Interference No. 106,013. Judgment dated September 29, 2015 ordered cancellation of U.S. 8,486,907. Decision dated December 29, 2015 denied our Request for Rehearing and is open to appeal.

In addition to the foregoing patents that protect eteplirsen, we either solely own or exclusively license from UWA 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., SRP-4045 and SRP-4053), 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 2034, 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 and PMO-based technologies (e.g., PPMO, PMOplus®, PMO-X®). These patents, and patent applications, if granted, expire between 2024 and 2032, 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 United States including, but not limited to, Sarepta®, Sarepta Therapeutics®, PMOplus®, PMO-X® and the Sarepta Therapeutics logo. In addition, we have multiple pending trademark applications in the United States.

Our commercial success will depend in part on obtaining and maintaining patent protection and trade secret protection for our product candidates, and successfully defending these patents against third-party challenges. Our ability to protect our product candidates from unauthorized making, using, selling, offering to sell or importing by third parties is dependent on the extent to which we have rights under valid and enforceable patents that cover these activities.

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.

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. For example, we are aware of certain claims that our competitor BioMarin has rights to in the United States that, if granted, may provide the basis for BioMarin or other parties that have rights to these claims to assert that our drug candidates, eteplirsen and/or SRP-4053, infringe on such claims. In 2014, the Patent Trial and Appeal Board ("PTAB") of the USPTO declared various patent interferences between certain patents held by Sarepta under a license from the UWA and patent applications held by BioMarin under license from Academisch Ziekenhuis Leiden ("AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. Patents held or licensed to Sarepta and included in these interference proceedings are presumed valid by statute for the duration of these proceedings and any appeals. These interferences have not changed our plans to submit an NDA for eteplirsen, continue with our clinical development plans for eteplirsen and SRP-4053 or our ability to launch eteplirsen commercially if it is approved by the FDA under an accelerated approval pathway, however, if final resolution of these interferences and related appeals, if any, are not in our favor, our current business, development and commercialization plans for eteplirsen and SRP-4053 may be negatively impacted. For details on and risks related to the interferences that PTAB has declared involving our patents, please read Risk Factors—Risks Relating to Our Business—Our success, competitive position and future revenue, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our 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.

The pharmaceutical, biotechnology and other life sciences patent situation outside the U.S. can be even more uncertain. For example, BioMarin has rights to European Patent No. EP 1619249. We opposed this patent in the Opposition Division of the European Patent Office ("Opposition Division"), and in November 2011, we announced that, although we succeeded in invalidating some of the patent's claims, the Opposition Division maintained in amended form certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46. We and BioMarin both appealed this decision in June 2013; however, pending final resolution of this matter, the patent at issue may provide the basis for BioMarin or other parties that have rights to such patent in the relevant European country to assert that our drug candidate, eteplirsen, infringes on such patent upon launching eteplirsen in such relevant European country. The outcome of the appeal cannot be predicted or determined as of the date of this report. If as part of any appeal before the European Patent Office we are unsuccessful in invalidating BioMarin's claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both eteplirsen and other therapeutic candidates, such as SRP-4045 and SRP-4053 could be materially impaired. Moreover, our ability to commercialize eteplirsen in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the European Patent Office Board of Appeals could be materially impaired. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending and in some cases are proceeding to grant. Should any patents grant from these applications, our ability to commercialize eteplirsen or our other therapeutic candidates, such as SRP-4045 and SRP-4053, could be materially impaired.

In addition to government, court and regulatory patent decisions, changes in either the patent laws or in interpretations of patent laws in the U.S. and other countries may diminish the value of our intellectual property. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in the patents that we own or have licensed or in

third-party patents. Further, since publication of discoveries in scientific or patent literature often lags behind actual discoveries, there is no assurance that we were the first creator of inventions covered by our pending patent applications, or that we were the first to file patent applications for these inventions.

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.

Drug Approval Process

To obtain FDA approval of a product candidate, we must, first and foremost, 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 application 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 placebo-controlled studies or comparison of treated group from clinical trials to data from natural history data or studies;
- 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 manufacturing site(s) in the form of pre-approval inspections; and
- FDA review and approval of the NDA.

Preclinical studies 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 studies, 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 design, particularly regarding potential safety issues of 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 I, II and III) prior to approval, a portion of these phases may overlap. A fourth post-approval phase (Phase IV) may include additional clinical studies. 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 I. Phase I 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 I studies usually involve less than 100 subjects and are conducted in healthy adult volunteers unless the drug is toxic (e.g., cytotoxics) in which case they are tested in patients.
- Phase II. Phase II 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 II studies usually involve patients with the disease under investigation and may vary in size from several dozen to several hundred.

- Phase III. If an investigational drug is found to be potentially effective and to have an acceptable safety profile in early phase studies, larger Phase III 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 III clinical trials which establish the safety and efficacy of the drug for a specific indication are required for approval of an NDA. Phase III studies usually include several hundred to several thousand patients for larger, non-orphan drug indications/diseases. However, for orphan drug indications due to their lower prevalence, clinical trials for rare or orphan diseases generally have fewer patients. For these orphan diseases, a company may also try to demonstrate efficacy and safety by comparing treated patients in clinical trials to untreated populations in placebo-controlled clinical trials or to data from natural history studies.
- Phase IV. Phase IV trials are clinical studies conducted after the FDA has approved a product for marketing. Typically there are two forms of Phase IV 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 IV 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 to the FDA the results of the preclinical and clinical trials, together with, among other things, detailed information on the manufacture and composition of the product candidate and proposed labeling, in the form of an NDA, including payment of a user fee unless the submission is for an Orphan Indication. The FDA assesses all NDAs submitted for completeness before it accepts them for filing and review. 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. Under the current NDA review goals mandated under 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. 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 refuse to approve the NDA. The FDA may also refer an application to an advisory committee, typically comprised of a panel of expert clinicians and researchers, for review, evaluation and a recommendation as to whether the application should be approved for the proposed indication. The FDA is not bound by, but typically follows, the recommendations of the advisory committee.

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 and both AVI-7288 and AVI-7537 were granted fast track status in September 2012.

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 IV 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.

While FDASIA provides certain authorities and direction to the FDA, it is unclear how the FDA will interpret and implement FDASIA provisions, in particular, in considering what the appropriate regulatory approval pathway is for eteplirsen. 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.

We had multiple meetings with the FDA during 2013 and 2014 to discuss the most appropriate regulatory pathway for early registration/approval of eteplirsen based on the Phase IIb data. In addition, we also had discussions with the FDA to finalize the confirmatory study designs for a potential accelerated approval for eteplirsen. Based on the data requirements and accelerated approval pathways defined by FDA, the eteplirsen NDA was prepared and submitted in June 2015. The eteplirsen NDA was filed by the FDA and granted priority review status in August 2015. Currently, review of the eteplirsen NDA is ongoing. The FDA postponed a meeting of the Peripheral and Central Nervous System Drugs Advisory Committee to discuss the NDA for eteplirsen previously scheduled for January 22, 2016 and notified us of the a new PDUFA date of May 26, 2016.

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; and
- continue to have quality control and manufacturing procedures conform to cGMP after 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.

Many other countries and jurisdictions have similar drug development and regulatory review processes. We have conducted clinical trials in the United Kingdom and intend to submit for marketing approval in countries other than the U.S. Therefore, we will have to comply with the legal and regulatory requirements in the countries where we conduct trials and submit for marketing approval. We will continue to evaluate, with input from the FDA and other regulatory authorities, which expedited programs are appropriate to incorporate in our regulatory approach for eteplirsen and our other DMD product candidates.

Orphan Drug Designation and Exclusivity

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. In the U.S., 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.

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. Competitors may receive approval of different drugs or biologics for the indications for which a prior approved orphan drug has exclusivity. We have been granted orphan drug designation for eteplirsen, AVI-7288, AVI-7537 and AVI-5038 in the U.S.

In Europe, Orphan Medicinal Product designation is considered by the European Medicines Agency ("EMA") for drugs intended to diagnose, prevent or treat a life-threatening or very serious condition afflicting five or fewer out of 10,000 people in the E.U., including compounds for serious and chronic conditions that would likely not be marketed without incentives due to low market return on the sponsor's development investment. The medicinal product considered should be of significant benefit to those affected by the condition as compared to previously approved products for the same indication. 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 drug for the same therapeutic indication as the orphan drug. Distinct from orphan drug exclusivity, the EMA may provide a sponsor having an approved Pediatric Investigation Plan ("PIP") or pediatric exclusivity waiver, which may lead to a two-year extension of market exclusivity beyond the original ten-year period of orphan drug exclusivity. We have been granted orphan drug designation for eteplirsen and AVI-5038 in the E.U.

Ex-U.S. Regulatory Requirements

In addition to regulation by the FDA and certain state regulatory agencies, we are also subject to a variety of foreign regulations governing clinical trials and the marketing of other medicinal products. Outside of the U.S., our ability to market a product depends upon receiving a marketing authorization from the appropriate regulatory authorities. The requirements governing the conduct of clinical trials, applications for marketing authorization, pricing and reimbursement vary widely from country to country. In any country, however, we will only be permitted to commercialize our products if the appropriate regulatory authority is satisfied that we have presented adequate evidence of safety, quality and efficacy. Whether or not FDA approval has been obtained, approval of a product by the comparable regulatory authorities of foreign countries must be obtained prior to the commencement of marketing of the product in those countries. The time needed to secure approval may be longer or shorter than that required for FDA approval. The regulatory approval and oversight process in other countries includes all of the risks associated with regulation by the FDA and certain state regulatory agencies as described above.

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.

Pharmaceutical Pricing and Reimbursement

In both U.S. and foreign markets, our ability to commercialize our products successfully and to attract commercialization partners for our products, depends in significant part on the availability of adequate financial coverage and reimbursement from third-party payers, including, in the U.S., governmental payers such as the Medicare and Medicaid programs, managed care organizations and private health insurers. Third-party payers are increasingly challenging the prices charged for medicines and examining their cost effectiveness, in addition to their safety and efficacy. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the cost effectiveness of our products. Even with the availability of such studies, our products may be considered less safe, less effective or less cost-effective than alternative products, and third-party payers may not provide coverage and reimbursement for our product candidates, in whole or in part.

Political, economic and regulatory influences are subjecting the healthcare industry in the U.S. to fundamental changes. There have been, and we expect there will continue to be, legislative and regulatory proposals to change the healthcare system in ways that could significantly affect our business, including the Patient Protection and Affordable Care Act of 2010. 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 include:

- controls on government funded reimbursement for drugs;
- 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; and
- expansion of use of managed care systems in which healthcare providers contract to provide comprehensive healthcare for a fixed cost per person.

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 have a material adverse effect on our business prospects.

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 and infectious 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, many 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 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, no disease-modifying product has been granted full approval for the treatment of DMD and no product is commercially available outside the European Economic Area (“EEA”). Companies including, but not limited to, BioMarin Pharmaceuticals, Inc. (“BioMarin”) and PTC Therapeutics, Inc., (“PTC”), have product candidates in development for the treatment of DMD. Nippon Shinyaku has reported early clinical development data for an exon 53 skipping candidate, and it is unknown if further clinical development of this or other exon-skipping compounds is planned.

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 III Ataluren Confirmatory Trial (“ACT”) DMD clinical trial result to the EMA. 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. Additionally companies such as Santhera, Summit, 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.

BioMarin has an exon 51-skipping product candidate, drisapersen. An NDA for drisapersen was filed by the FDA and a marketing authorization application 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. Drisapersen was previously owned by Prosensa. The Prosensa program commenced treatment in December 2010 in a Phase III clinical study in ambulant individuals with DMD who have a dystrophin gene mutation amenable to treatment by skipping exon 51. This randomized, placebo-controlled study was fully enrolled, with approximately 180 participants who were being dosed for 48 weeks. The primary efficacy endpoint for Prosensa's study was a measure of muscle function using the 6MWT. In September 2013, GSK and Prosensa announced that the Phase III clinical study of drisapersen did not meet the primary endpoint of a statistical significant improvement in the 6MWT compared to placebo. In September 2010, the Prosensa / GSK program commenced a Phase II double-blind, placebo-controlled study. This study is designed to assess the efficacy of two different dosing regimens of GSK2402968 administered over 24 weeks in DMD patients, and then to continue observing the patients over a second 24-week interval for a total study time frame of 48 weeks. This study completed enrollment with 54 DMD patients in October 2011 and has since concluded. Another study using GSK2402968 in non-ambulatory DMD patients has been initiated using a 6 mg/kg dose and is anticipated to enroll 20 patients. Like BioMarin, 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, or obtain marketing approval before, eteplirsen.

Additionally, several companies 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, Eli Lilly, Alnylam Pharmaceuticals, Inc., Moderna Therapeutics, Inc., Summit plc, Akashi, Catabasis and Oxford University.

Hemorrhagic Fever Virus Program Competition. No specific treatment has been proven effective, and no approved vaccine currently exists for treatment or prophylaxis of either Ebola virus or Marburg virus. These agents must be tested extensively in animals and meet strict government regulations. Investigational compounds can only be tested for efficacy in humans during outbreak situations such as the recent Ebola outbreak in West Africa that began in early 2014. The exigency and scale of the 2014 Ebola virus outbreak in West Africa has accelerated the development of both treatments and vaccines for Ebola. Several vaccine candidates have reached the clinical development stage and are actively being tested for population safety and potentially efficacious immunoprotective effect as a prophylactic agent. These include vaccine candidates sponsored by the biotechnology industry and also candidates in development by U.S. government agencies (e.g., the NIAID and the DoD). The U.S. government is also supporting early stage research on therapeutics against hemorrhagic fever viruses, including broad-spectrum therapeutics. Among the most advanced therapeutic candidates that might have utility in combating Ebola virus, are candidates being developed by the Tekmira Pharmaceutical Corp., Toyama Chemical Co. LTD, BioCryst Pharmaceuticals Inc., and Mapp Biopharmaceutical Inc. with the support of the U.S. government. Additionally, investigation of the use of convalescent plasma containing Ebola virus antibodies as a treatment modality, as well as for the potentially efficacious repurposing of drugs not intended to treat Ebola virus, remain an ongoing pursuit by the biopharmaceutical industry and several national government agencies.

Influenza Program Competition. Currently, there are three therapeutic products for influenza that have received market approval from the FDA and are recommended for use in the U.S. These are: (1) oseltamivir (Tamiflu), a Roche Holding and Gilead product; (2) zanamivir (Relenza), a GSK product; and (3) peramivir (Rapivab), a BioCryst Pharmaceuticals Inc. product. In addition to these products, Biota Pharmaceuticals and Daiichi Sankyo's laninamivir was launched in 2010 in Japan. Currently, funding from the United States Department of Health and Human Services ("DHHS") Biomedical Advanced Research and Development Authority is helping support clinical trials of, Romark Laboratories' nitazoxanide. In addition, other companies have influenza therapeutic compounds against viral and host targets in various stages of development, including Vertex Pharmaceutical and Janssen Pharmaceutical's VX-787, Biota Pharmaceutical's laninamivir, Autoimmune Technologies flufirvitide-3, Ansun BioPharma's fludase, and Toyama Chemical's favipiravir which is in a Phase II clinical trial in the United States, under a DoD contract with MediVector,

Inc., and has completed a Phase III trial in Japan. Several additional companies, including Crucell Inc., Celltrion Inc., Visterra Inc. and Genentech Inc. are also currently developing monoclonal antibodies for use against various influenza strains to confer passive or active immunotherapeutic response. DHHS is currently seeking additional antiviral therapeutics for the treatment of influenza infections.

In addition to therapeutic products, other companies are focusing development efforts on universal influenza vaccines, including BiondVax Pharmaceuticals Ltd. and Immune Targeting Systems which are in Phase II and Dynavax in Phase I clinical trials. Successful development of a universal influenza vaccine could lead to a reduction in the number of influenza cases and, therefore, the market size.

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 and Santaris Pharma A/S.

Employees

As of December 31, 2015, we had 270 employees, 114 of whom hold advanced degrees. Of these employees, 161 are engaged directly in research and development activities and 109 are in general and administration including 33 in the sales force. None of our employees are covered by collective bargaining agreements and we consider relations with our employees to be good.

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

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

Our most advanced product candidate is eteplirsen for which the FDA is reviewing a new drug application ("NDA") with a Prescription Drug User Fee Act ("PDUFA") action date of May 26, 2016. Eteplirsen is still being evaluated in several clinical studies, including a confirmatory clinical trial. The exon 53-skipping product candidate, which we are working on with the SKIP-NMD consortium, is currently in the clinic and we have completed Part I and have started Part II of a Phase I/IIa clinical trial in the E.U. We are also in the process of conducting a placebo-controlled dose titration study for our exon 45-skipping product candidate. Additionally, we are working towards initiating a clinical trial in the U.S. and the E.U. for exon 45- and 53-skipping product candidates and have satisfactorily responded to the FDA's inquiries on pre-clinical data for this study relating to our exon-53 product candidate. The remainder of our product candidates are in 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, eteplirsen, our exon 45-skipping product candidate, the exon 53-skipping product candidate we are developing with the SKIP-NMD consortium, each for DMD, and AVI-7100 in influenza are in active clinical development. AVI-7537 in Ebola and AVI-7288 in Marburg were being developed through a program with the U.S. Department of Defense (the "DoD") and further development is conditioned in part on obtaining additional funding, collaborations or emergency use. Our other product candidates, including our anti-bacterials, are in preclinical development or inactive. 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 eteplirsen and other exon-skipping candidates as part of our larger pan-exon strategy in DMD, our infectious 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.

Our RNA-targeted antisense technology has not been incorporated into a therapeutic commercial product and is still at an early stage of development.

Our RNA-targeted platforms, utilizing proprietary PMO-based technology, have not been incorporated into a therapeutic commercial product and are still at an early stage of development. This technology is used in all of our product candidates, including eteplirsen. Although we have conducted and are in the process of conducting clinical studies with eteplirsen, an exon 45-skipping product candidate and an exon 53-skipping product candidate and preclinical studies 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. 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 preclinical studies. Any failures or setbacks in developing or utilizing our PMO-based technology, 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.

We have been granted orphan drug designations in the U.S. and in the E.U. for certain of our product candidates, however, there can be no guarantee that we will maintain orphan status for these product candidates nor that we will receive orphan drug approval and hence prevent third parties from developing and commercializing products that are competitive to these product candidates in the absence of other barriers to entry.

To date we have been granted orphan drug designation under the Orphan Drug Act by the FDA for two of our product candidates in DMD (including eteplirsen), AVI-7537 for the treatment of Ebola virus and AVI-7288 for the treatment of the Marburg virus. Upon approval from the FDA of an NDA, products granted orphan drug status are generally provided with seven years of marketing exclusivity in the U.S., meaning the FDA generally will not approve applications for other product candidates for the same orphan indication that contain the same active ingredient. Even if we are the first to obtain approval of an orphan product and are granted exclusivity in the United States, there are limited circumstances under which a later competitor product may be approved for the same indication during the seven-year period of marketing exclusivity, such as if the later product is shown to be clinically superior to our product or due to an inability to assure a sufficient quantity of the orphan drug.

We also have been granted orphan medicinal product designations in the E.U. for two of our product candidates in DMD (including eteplirsen). Product candidates granted orphan status in Europe can be provided with up to 10 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 may 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.

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 United States or the E.U., our business and results of operations could be materially adversely affected. While orphan status for any of our products, if granted or maintained, would provide market exclusivity in the United States and the E.U. for the time periods specified above, we would not be able to exclude other companies from manufacturing and/or selling 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 United States or the E.U. for a product candidate that has the same active ingredient or is a similar medicinal product for the same indication as any of our product candidates for which we plan to file an NDA or marketing authorization application (“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 United States or the E.U., as applicable. Further, application of the orphan drug regulations in the United States and Europe is uncertain, and we cannot predict how the respective regulatory bodies will interpret and apply the regulations to our or our competitors’ product candidates.

Even if we receive regulatory approvals for any of our product candidates, it is possible that they may not become commercially viable products.

Even if a product candidate receives regulatory approval, the product may not gain market acceptance among physicians, patients, healthcare or third-party payers or the medical community which could limit commercialization of the product. Assuming that any of our product candidates receives the required regulatory approvals, commercial success will depend on a number of factors, including but not limited to the following:

.

demonstration and/or confirmation of clinical efficacy and safety and acceptance of the same by the medical community;

- cost-effectiveness of the product;
- the availability of adequate reimbursement by third parties, including government payers such as the Medicare and Medicaid programs, managed care organizations and private health insurers;
- the product's potential advantage over alternative or competitive treatment methods;
- whether the product can be manufactured in commercial quantities and at acceptable costs;
- marketing and distribution support for the product;
- any exclusivities or patent rights applicable to the product;
- the market-size for the product which may be different than expected; and

- our ability to achieve and sustain profitability, which may not occur if we are unable to develop and commercialize any of our product candidates, development is delayed or sales revenue from any product candidate that receives marketing approval is insufficient.

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 would materially impair our ability to generate 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 United States, 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 United States 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, including eteplirsen, on an accelerated approval (e.g., under the Food and Drug Administration Safety and Innovation Act of 2012 ("FDASIA")) or any other basis, in any jurisdiction, including in the United States, cannot be assured, may be significantly delayed or may never be achieved for various reasons including the following:

- Our preclinical, clinical, Chemistry, Manufacturing and Controls ("CMC") 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 submissions, advisory committee panels, filings or approvals. The FDA could disagree with our beliefs, interpretations and conclusions regarding data we submit in connection with an NDA submission, including the eteplirsen NDA, or other product candidates, and may delay, reject or refuse to file or approve any NDA submission we make or provide a complete response letter until we meet their additional requirements, if ever. In addition, an advisory committee could determine 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 deny approval of our product candidates based on their review of the data or other factors. Each of these risks all apply to our eteplirsen NDA which is the only NDA we have submitted to the FDA for review to date.
- 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 novel endpoints, such as natural history data and dystrophin measures, is uncertain due to, among other factors, evolving interpretations of a new therapeutic class, the broad discretion of regulatory authorities, lack of precedent, 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, including eteplirsen, will qualify for accelerated approval under FDASIA 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, we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned INDs and NDAs 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 patient muscle biopsies and dystrophin analyses), repeating or completing additional analysis of our data, or providing additional supportive data. In addition, an 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 review of our NDA or result in a decision by the Company not to proceed with development of a product candidate or an NDA submission for a product candidate based on feedback from regulators. For example, in reviewing the dystrophin data and analysis that we submitted for eteplirsen, the FDA previously expressed concerns with dystrophin as a surrogate endpoint and requested an independent assessment of dystrophin positive fibers measured in our eteplirsen Phase IIb study, which we provided. The FDA has also requested natural history data to better evaluate the ongoing clinical results of our eteplirsen 201/202 study, which we have also provided. Any material

inconsistencies between our existing data and analysis and any new analyses and additional data we provide to the FDA, including the independent assessment of dystrophin positive fibers, safety data, natural history and data from a fourth biopsy that we have provided, could negatively impact the review of our eteplirsen NDA submission. While our studies demonstrate statistical significance, the FDA may not consider our six-minute walk test ("6MWT") results, including our comparison of our 6MWT results to matched external natural history data, or, to the extent the FDA considers dystrophin a relevant biomarker, the dystrophin production observed in our studies, as demonstration of, or reasonably likely to predict a clinical benefit. Additionally, the FDA may determine, after evaluating the totality of our data and analysis package for a product candidate, or receiving the vote of an advisory committee, that such package does not support an NDA approval.

- 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 requests in a timely and satisfactory manner could significantly delay or negatively impact our placebo-controlled confirmatory study timelines and/or the development plans we have for the exon 53- and exon 45-skipping product candidates. Responding to requests from regulators and meeting requirements for clinical studies, submissions, filings, advisory committees and approvals may require substantial personnel, financial or other resources, which, as a small pre-commercial 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. For example, changes to CMC processes for the production of eteplirsen may require coordination with our third-party manufacturers, which may or may not be limited in their abilities to execute such regulatory requests. 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 related to clinical trial design and the timing of regulatory decisions with respect to any NDA 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. 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., 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 would remain subject to ongoing regulatory compliance and oversight to maintain our approval. Conducting our confirmatory studies could take years to complete, yield negative results or the FDA could determine that they 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.

Our preclinical and clinical trials may fail to demonstrate acceptable levels of safety, efficacy, and quality of our product candidates, which could prevent or significantly delay their regulatory approval.

To obtain the requisite regulatory approvals to market and sell any of our product candidates, we must demonstrate, through extensive preclinical and clinical studies that the product candidate is safe and effective in humans. Ongoing and future preclinical and clinical trials of our product candidates may not show sufficient safety, efficacy or adequate quality to obtain or maintain regulatory approvals. Furthermore, success in preclinical and early clinical trials does not ensure that the subsequent trials we plan to conduct will be successful, nor does it predict final results of a confirmatory trial. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product candidates, 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. For example, in 2012, we completed Study 201, a U.S.-based Phase IIb 12-person clinical trial for eteplirsen at 30 mg/kg and 50 mg/kg. Following completion of this study, we initiated Study 202, an ongoing open label extension study with the same participants from Study 201. These trials were initiated, in part, to further demonstrate efficacy and safety, including the production of dystrophin, and explore and identify a more consistently effective dose that may be more appropriate for future clinical trials. While Studies 201 and 202 demonstrated dystrophin production based on the measurements taken at weeks 24 and 48, respectively, and 6MWT results reported for weeks 62, 74, 84, 96 and 120 supported stabilization of disease progression, we cannot provide assurances that data from the ongoing open label extension study will continue to be positive or consistent through the study periods or that the interpretation by regulators, such as the FDA, of the data we collect for our product candidates, including for eteplirsen, will be consistent with our interpretations. For example, on July 10, 2014, we announced that the 6MWT results for week 144 in Study 202 showed a change in decline from 5%, which was observed prior to 144 weeks, to

approximately 8.5%. Additionally, on January 12, 2015, we announced results for week 168 in Study 202, which showed continued ambulation across all patients evaluable on the test, however patients showed a decline in distance walked on this measure since the week 144 time point. Further, on October 1, 2015 we announced additional clinical efficacy and safety data that demonstrated that (i) eteplirsen provided a statistically significant advantage of 151 meters in the ability of study participants to walk at three years versus an untreated external DMD control, (ii) eteplirsen-treated patients (n=12) experienced a slower rate of decline through week 192 versus untreated external DMD controls and (iii) the eteplirsen safety profile remained consistent with prior results. In January 2016, the FDA made public our eteplirsen Briefing Document Addendum (the “January 2016 Addendum”), which disclosed that at four years, 10 out of 12 patients on eteplirsen remained ambulatory while 10 out of 13 untreated patients in the external control had lost ambulation (one patient in the external control was still ambulatory at year four, while two patients in the external control were missing data at four years), a statistically significant difference. In addition, the January 2016 Addendum disclosed a statistically significant advantage of 162 meters in the ability of study participants to walk (as measured by the 6MWT) at four years.

If we do not obtain the required approvals to initiate the confirmatory trial for eteplirsen using our exon 45- and 53-skipping product candidates, the data from the confirmatory studies for eteplirsen do not produce the safety and efficacy data required by the FDA for obtaining or maintaining marketing approval, or the FDA does not accept the results of our eteplirsen confirmatory studies as supporting evidence of efficacy, we may need to continue working with the FDA on the design and subsequent execution of any further studies or analysis we plan to conduct or that may be required to obtain and maintain approval of eteplirsen or our other DMD product candidates. Any significant delays or negative developments in the confirmatory studies for eteplirsen could delay or otherwise negatively impact our development plans for our follow-on DMD product candidates. For example, in October 2014, we received meeting minutes from a Type B pre-NDA meeting that took place in September 2014 in which the FDA provided updated guidance regarding the information to be provided as part of, or at the time of, our NDA submission for eteplirsen. The guidance stated that the FDA was requiring additional data as part of the NDA submission, including the results from an independent assessment of dystrophin images, the 168 week clinical data from Study 202, and additional safety data from new patients exposed to eteplirsen, specifying the minimum number of patients and minimum duration of exposure. Additionally, the guidance also required patient-level natural history data to be obtained by us from independent academic institutions and requested MRI data from a recent study conducted by an independent group. Although we continue to work to provide the FDA with the additional data and information requested, it may not support or result in a positive recommendation of an advisory committee or the approval of our eteplirsen NDA submission.

We currently rely on third parties in the manufacturing process to produce our product candidates and our dependence on these parties, including any inability on our part to accurately anticipate product demand and timely secure manufacturing capacity to meet actual clinical or commercial product demand may impair the advancement of our research and development programs and potential commercialization of our product candidates.

We currently do not have the internal ability to undertake the manufacturing process for our product candidates in the quantities needed to conduct our research and development programs, supply clinical trials or meet commercial demand. 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), drug substance (“API”) and drug product, as well as to perform additional steps in the manufacturing process, such as the filling and labeling of vials and storage of our product candidates. There are a limited number of third parties with facilities and capabilities suited for the manufacturing process of 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. 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, could result in the cancellation of shipments, loss of product in the manufacturing process or a shortfall in available 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 have our product candidates manufactured in sufficient quality and quantity required for planned preclinical testing, clinical trials and potential commercial use would be adversely affected.

Sarepta, through its third party manufacturers, has produced or is in the process of producing clinical and commercial supply, including for eteplirsen, based on its current understanding of market demands and planned clinical studies. In light of the limited number of third parties with the expertise to produce 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 manufacturing arrangements on the commercially reasonable terms necessary to provide adequate supply of our product candidates to meet demands that exceed our clinical or commercial assumptions. Further, we may not be able to obtain the significant financial capital that may be required

in connection with such arrangements. Even after successfully engaging third parties to execute the manufacturing process for our 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 regulatory approval process and potential commercialization. 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 of any of our product candidates.

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

Our contract manufacturers are required to produce our materials, APIs and drug products under current Good Manufacturing Practice regulations ("cGMP"). We and our contract manufacturers are subject to periodic unannounced inspections by the FDA and corresponding state and foreign authorities to ensure strict compliance with cGMP and other applicable government regulations and corresponding foreign requirements. We do not have 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 our product candidates in a compliant manner on the schedule we require for clinical trials or for potential

commercial use. The failure to achieve and maintain high quality compliance, including failure to detect or control anticipated or unanticipated manufacturing errors, could result in patient injury or death or product recalls. Any difficulties or delays in our contractors' manufacturing and supply of product candidates, or any failure of our contractors to maintain compliance with the applicable regulations and requirements could increase our costs, make us postpone or cancel clinical trials, prevent or delay regulatory approvals by the FDA and corresponding state and foreign authorities, prevent the import and/or export of our products, cause us to lose revenue, result in the termination of the development of a product candidate, or have our product candidates recalled or withdrawn from use.

We may not be able to successfully scale up manufacturing of 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 delay or prevent us from developing or commercializing our product candidates.

As we prepare for larger and later stage clinical trials for our product candidates and the potential commercialization of eteplirsen, we are working to increase future manufacturing capacity and scale up production of some of the components of our drug products. In 2016, our focus remains on (i) achieving larger-scale manufacturing capacity for eteplirsen throughout the manufacturing supply chain and (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. 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 or is cost-effective, or in a time frame required to meet our timelines for clinical trials, potential commercialization and other business plans, or at all. cGMP 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 a product candidate itself or the 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 product and demonstrate stability of product candidates for use in late stage clinical trials (and any subsequent drug products for commercial use), our analytical methods must be validated in accordance with regulatory guidelines. We may not be able to successfully validate, or maintain validation of, our analytical methods or demonstrate adequate purity, stability or comparability of the product candidates in a timely or cost-effective manner, or at all. If we are unable to successfully validate our analytical methods or to demonstrate adequate purity, stability or comparability, the development of our product candidates and regulatory approval or commercial launch for any resulting drug products may be delayed, which could significantly harm our business.

During work with our third-party manufacturers to increase manufacturing capacity and scale up production, it is possible that they could make improvements in the manufacturing and scale-up processes for our product candidates. We may not own or be able to secure ownership of such improvements or may have to share the intellectual property rights to those improvements. Additionally, 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 our product candidates could cause significant delays in our business plans or prevent commercialization of our product candidates.

We are winding down our expired U.S. government contract, and 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, including our Ebola and Marburg programs. The July 2010 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. We may explore and evaluate options to continue advancing the development of our Ebola and Marburg product candidates, which may or may not include funding through U.S. government programs. As a result of government budgetary cuts, appropriations and sequestration, among other reasons, the viability of the government and its agencies as a partner for further development of our Ebola and Marburg programs, or other programs, is uncertain. The options for us to further develop product candidates that were previously developed under contracts with the U.S. government with third parties may be limited or difficult in certain respects given that, after termination or expiration of a U.S. government contract, the government has broad license rights in intellectual property developed under such contract. Therefore, the U.S. government may have the right to develop all or some parts of product candidates 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 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;
    - participant enrollment and retention 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 IRBs, 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. In addition, terms may vary significantly among different trial sites and CROs and may subject 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 $220.2 million for the year ended December 31, 2015. Our accumulated deficit was $899.1 million as of December 31, 2015. Substantially all of our revenue to date has been derived from research and development contracts with the DoD, the last of which expired in July 2014. We have not yet generated any revenue from product sales and have generally incurred expenses related to research and development of our technology 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 as we:

- continue our research, preclinical 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;
- establish a sales, marketing and distribution infrastructure to commercialize any products for which we may obtain marketing approval;
- 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.

Our ability to achieve and maintain profitability depends on various factors including our ability to raise additional capital, partner with third parties for one or more of our programs, complete development of our product candidates, obtain regulatory approvals and market our approved products, if any. It is uncertain when, if ever, we will become profitable and if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of the Company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations.

If the FDA does not approve our eteplirsen NDA by the currently planned PDUFA date, or at all, our business may be negatively impacted and we may suffer financial losses in connection with winding down and terminating contracts, manufacturing commitments and employees hired in connection with our current and planned activities in preparation for a potential commercial launch.

Given the potential commercialization timelines, we have commenced certain pre-launch and commercialization investments and activities including, but not limited to, negotiating and entering into supply and other commercial agreements, scaling up manufacturing and hiring certain positions needed for pre-launch and commercial activities and operations. If the FDA delays or does not provide approval for our eteplirsen NDA by the currently planned PDUFA date of May 26, 2016, or at all, or we need to delay or discontinue our development and commercialization plans for eteplirsen for other reasons, our business and the development of our follow-on DMD product candidates may be negatively impacted and we may incur financial losses in connection with delaying, winding down or terminating the investments, contracts and commitments we enter into for the purpose of positioning ourselves for a commercial launch of eteplirsen.

We will need additional funds to conduct our planned research, development and manufacturing efforts. If we fail to attract significant capital on acceptable terms or fail to enter into strategic relationships, we may be unable to continue to develop our product candidates.

We will likely require additional capital from time to time in the future in order to continue the development of product candidates in our pipeline and to expand our product portfolio. The actual amount of funds that we may need will be determined by many factors, some of which are beyond our control. These factors include the success of our research and development efforts, the status of our preclinical and clinical testing, costs and timing relating to securing regulatory approvals and obtaining patent rights, regulatory changes, competitive and technological developments in the market 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.

We would expect to seek additional financing from the sale and issuance of equity or equity-linked or debt securities, 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. In addition, if the FDA delays or ultimately denies approval of our eteplirsen NDA, raising additional funds may be difficult. 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. For example, on October 9, 2015, we sold 3,250,000 shares of our common stock in an underwritten public offering at a price to the public of $39.00 per share. Additional debt 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.

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 preclinical collaborations with academic or research institutions and government entities for the development of additional exon-skipping product candidates for the treatment of DMD and clinical collaboration for a product candidate for the treatment of influenza, 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 credit and security agreement with MidCap Financial could adversely affect our financial condition or restrict our future operations.

On June 26, 2015, the Company entered into a credit and security agreement with MidCap Financial that provides a senior secured term loan of $20.0 million, which may be increased by an additional $20.0 million upon the acceptance by the FDA of the NDA for eteplirsen. This indebtedness could have important consequences, including:

- requiring the Company to maintain pledged cash in favor of MidCap Financial equal to not less than the lesser of the outstanding term loans or (a) $15.0 million prior to the increase in the term loan by an additional $20.0 million and (b) $30.0 million thereafter;
- 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 credit and security agreement.

Any of these factors could materially and adversely affect our business, financial condition and results of operations. In addition, if we incur additional indebtedness, the risks related to our business and our ability to service our indebtedness would increase.

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 United States. 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 those related to revenue recognition, accrued expenses, assumptions in the valuation of stock-based compensation and accounting for and valuation of liability classified warrants. 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.

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 of 1986, as amended, 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 than would have otherwise been required if such limitations were not in effect and could cause such net operating losses and tax credits to expire unused, in each case reducing or eliminating the benefit of such net operating losses and tax credits and potentially adversely affecting our financial position. Similar rules and limitations may apply for state income tax purposes.

We rely on third parties to provide services in connection with our preclinical 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 preclinical 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.

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.

Our former CEO and President resigned on March 31, 2015 and we have appointed an interim CEO. No assurance can be made about the impact that this change in management will have on the Company and its business plans (including our regulatory and clinical plans and relationships) nor as to when we will hire a permanent CEO. The existing management team is actively managing the business in accordance with a business strategy approved by the board of directors.

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 advancing our product candidates. 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 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, if any, depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our technologies 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 United States as well as other countries. We anticipate filing additional patent applications both in the United States 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 candidates and platform technology with a basis for exclusivity, we and our collaborators may not be able to develop or commercialize such 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 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. For example, in July 2014, the Patent Trial and Appeal Board (the "PTAB") of the USPTO declared patent interferences between certain patents held by Sarepta (under license from the University of Western Australia, "UWA") and patent applications held by BioMarin (under license from Academisch Ziekenhuis Leiden, "AZL") related to exon 51 and exon 53 skipping therapies designed to treat DMD. In particular, the PTAB declared Interference No. 106,008, which identifies Sarepta's/UWA's U.S. Patent Nos. 7,807,816 and 7,960,541, both covering eteplirsen, as interfering with BioMarin's/AZL's U.S. Application No. 13/550,210. The PTAB also declared Interference No. 106,007, which identifies Sarepta's/UWA's U.S. Patent No. 8,455,636, covering SRP-4053, as interfering with BioMarin's/AZL's U.S. Application No. 11/233,495. In September 2014, the PTAB declared a third patent interference relating to certain methods concerning the exon 51 skipping therapies that are the subject of Interference No. 106,008. In particular, the PTAB declared Interference No. 106,013, which identifies Sarepta's/UWA's U.S. Patent No. 8,486,907, which covers certain methods of using eteplirsen, as interfering with BioMarin's/AZL's U.S. Application No. 14/198,992. In addition, in a September 2014 Order in Interference No. 106,007, the PTAB authorized us to file a motion with the PTAB, which we filed in November 2014, requesting the declaration of a fourth interference relating to certain methods concerning the exon 53 skipping therapies that are the subject of Interference No. 106,007, including SRP-4053, and between Sarepta's/UWA's U.S. Patent No. 8,455,636 and BioMarin's/AZL's U.S. Application No. 14/248,279. On September 29, 2015, we received notice that the PTAB had issued a decision in Interference No. 106,013 that resulted in a judgment against Sarepta and an order for the cancellation of Sarepta's/UWA's U.S. Patent No. 8,486,907 that covers certain methods of using eteplirsen thereby leaving open the possibility of BioMarin's/AZL's competing U.S. Application No. 14/198,992 to issue and, if so, potentially provide a basis for BioMarin to allege that our product candidate, eteplirsen, infringes a patent granting from this application. We filed a Request for Rehearing that requests the PTAB to continue this interference, and the PTAB denied our Request on December 29, 2015. We intend to appeal this decision to an appropriate appeals court. We cannot make any assurances about the outcome of the two remaining proceedings (Interference No. 106,007 and Interference No. 106,008) or appeals of any of these three interferences. Any additional adverse rulings, which, in the case of Interference No. 106,007 and Interference No. 106,008 could come at any time and, if negative, could adversely affect our business and result in a decline in our stock price. If final resolution of the interferences and related appeals are not in our favor, then the Sarepta/UWA patents involved in these interferences and any other Sarepta/UWA patents or applications also found to be interfering may be invalidated, and as a result, we may not have any patent-based exclusivity available for our product candidates, which may have a material negative impact on our business plans. In addition, if final resolution of the interferences or related appeals are not in our favor, the USPTO may issue the BioMarin/AZL patent applications resulting in the grant of one or more patents that may provide a basis for BioMarin to allege that our product candidates, eteplirsen and/or SRP-4053, infringe such patents. In addition, these interferences, appeals and any subsequent litigation may require significant financial resources that we may have planned to spend on other Company objectives, resulting in delays or other negative impacts on such other objectives. In addition, BioMarin may continue to evaluate other opportunities to challenge our intellectual property rights or seek to broaden their patent positions in an attempt to cover our product candidates in the United States and in other jurisdictions. We are also aware of certain pending and granted claims that are held by BioMarin in Japan, Europe and certain other countries that may provide the basis for BioMarin or other parties to assert that eteplirsen infringes on such claims. Because we have not yet initiated an invalidation proceeding in these countries, the outcome and timing of any such proceeding cannot be predicted or determined as of the date of this report.

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 United States might have less restrictive patent laws than the United States, 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, or 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 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 our product candidates in important commercial markets.

If 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 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. BioMarin, which is developing competitive pipeline products, has rights to patent claims that, absent a license, may preclude us from commercializing eteplirsen in several jurisdictions. BioMarin has rights to European Patent No. EP 1619249, for example. We opposed this patent in the Opposition Division of the European Patent Office (“EPO”), and the Opposition Division maintained certain claims of this patent relating to the treatment of DMD by skipping dystrophin exons 51 and 46, which may provide a basis to maintain that commercialization of eteplirsen in a European country where BioMarin has a patent corresponding to EP 1619249 would infringe on such patent. Both we and BioMarin have appealed the Opposition Division decision, submitted briefs in support of our respective positions and have also submitted responses to each other’s briefs. BioMarin filed arguments with the EPO in response to Sarepta’s previously filed briefs. The Opposition Division decision, if maintained at the appeals level, could have a substantial negative effect on our business and leaves open the possibility that BioMarin or other parties that have rights to such patent could assert that our product candidate, eteplirsen, infringes on such patent in a relevant European country. The timing and outcome of the appeal cannot be predicted or determined as of the date of this report. If as part of any appeal before the European Patent Office we are unsuccessful in invalidating BioMarin’s claims that were maintained by the Opposition Division or if claims previously invalidated by the Opposition Division are restored on appeal, our ability to commercialize both eteplirsen and other therapeutic candidates could be materially impaired. Moreover, our ability to commercialize eteplirsen in a European country where BioMarin has a patent related to EP 1619249 while the appeal process remains ongoing before the European Patent Office Board of Appeals could be materially impaired. In addition, we are aware of various divisional applications relating to EP 1619249 that are being pursued by BioMarin, which are pending and in some cases are proceeding to grant. Should any patents grant from these applications, our ability to commercialize eteplirsen or our other therapeutic candidates, such as SRP-4045 and SRP-4053, could be materially impaired.

We are also aware of existing patent claims BioMarin is pursuing in the United States, including those involved in the interferences declared by the USPTO in July 2014 and September 2014 and discussed in these risk factors, and others that it has or is pursuing in other countries, that where granted may provide the basis for BioMarin or other parties to assert that commercialization of eteplirsen and certain other of our product candidates would infringe on such claims.

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 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 RNA 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 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) and Nippon Shinyaku Co. Ltd. share a focus on RNA-targeted drug discovery and development. Competitors with respect to our exon-skipping DMD program, or eteplirsen, include BioMarin (which acquired Prosensa), Nippon Shinyaku, Daiichi Sankyo and Shire plc; and other companies such as PTC Therapeutics and Summit plc have also been working on DMD programs. Additionally, several companies 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, Pfizer, Inc., Bristol-Myers Squibb, Biogen Idec, Inc., Ionis Pharmaceuticals, Inc., Alexion Pharmaceuticals, Inc., Sanofi, Eli Lilly, Alnylam, Moderna Therapeutics, Inc., Summit plc, Akashi, Catabasis, and Oxford University. Although BioMarin received a complete response letter for KyndrisaTM (drisapersen) for the treatment of DMD amenable to exon 51 skipping on January 14, 2016, BioMarin continues to be a competitor for us on the development of DMD exon-skipping product candidates. BioMarin announced that its ongoing Kyndrisa extension studies will continue, as will the ongoing clinical trials for other exon-skipping oligonucleotides, BMN 044, BMN 045 and BMN 053, while BioMarin is exploring next steps for this application. If BioMarin is successful in obtaining regulatory approval for any of its exon-skipping 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 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 technology obsolete or noncompetitive. Our competitors, including BioMarin, 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.

We currently have no products that have been approved for commercial sale; however, the current and future use of our product candidates by us and our collaborators in clinical trials, expanded access programs, the sale of any products in the future, 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 if we obtain

marketing approval for any of our product candidates. 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.

Despite the implementation of security measures, our internal computer systems and those of third parties with which we contract are vulnerable to damage from cyber-attacks, computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. System failures, accidents or security breaches could cause interruptions in our operations, and could result in a material disruption of our clinical activities and business operations, in addition to possibly requiring substantial expenditures of resources to remedy. The loss of clinical trial data could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur a liability and our research and development programs and the development of our product candidates could be delayed.

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 twelve months, our stock has increased as much as 60% in a single day or decreased as much as 55% in a single day. We expect that our stock could have a material swing in its trading price in connection with competitor developments, any advisory committee meeting/recommendation or FDA decision relating to our eteplirsen NDA, including a decline in trading price if research analysts, investors or others who follow us view the results of any developments related to these events as negative for Sarepta. 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:

- discussions at, the outcome of and other matters related to the advisory committee meeting for eteplirsen;
-

the timing of our submissions to regulatory authorities and regulatory decisions and developments including any decision by the FDA regarding our NDA for eteplirsen;

- 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 our product candidates;
- delays in beginning and completing preclinical and clinical studies for potential product candidates;
- delays in entering or failing to enter into strategic relationships with respect to development and/or commercialization of 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 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 such as the resignation of our former CEO and appointment of an interim CEO in 2015; 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 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.

We expect our operating results to fluctuate in future periods, which may adversely affect our stock price.

Our quarterly operating results have fluctuated in the past, and we believe they will continue to do so in the future. Our operating results may fluctuate due to the variable nature of our revenue and research and development expenses. Likewise, our research and development expenses may experience fluctuations as a result of the timing and magnitude

of expenditures incurred in

support of our DMD and other proprietary drug development programs. 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, 2015, there were approximately 45.6 million shares of common stock outstanding and outstanding awards to purchase 6.8 million shares of common stock under various incentive stock plans. Additionally, as of December 31, 2015, there were 1.8 million shares of common stock available for future issuance under our Amended and Restated 2011 Equity Incentive Plan, 0.1 million shares of common stock available for issuance under our 2013 Employee Stock Purchase Plan and 1.0 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.

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, Andover, Massachusetts and Corvallis, Oregon 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 02142 | 88,329 | 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 97333 | 53,000 | December 2020 | Laboratory and office space | Primarily lab space |
| 1749 SW Airport Avenue, Corvallis, OR 97333 | 36,150 | N/A – facility is owned; land lease expires February 2042 | Acquired with intention of providing future expansion space for the manufacture of potential products and components | Approximately 25,000 square feet leased and the remaining space unoccupied* |

* In November 2011, the tenant, Perpetua Power Source Technologies, Inc. (“Perpetua”), agreed to lease approximately 25,000 square feet of the building until March 2017. Perpetua has the option to extend the lease for an additional year if notice is provided no less than 12 months prior to the expiration date. Perpetua also has a right of first refusal relating to the lease of the remaining space at the building and was granted an option to purchase the building during the term of the lease, provided there is no uncured default by Perpetua at the time of exercise. If the purchase option is exercised, the price for the building was $2.0 million until February 2015 and $2.1 million from March 2015 until February 2016, and will be $2.2 million from March 2016 through the remainder of the initial lease term. If Perpetua exercises its extension option, the purchase price will be $2.3 million during the term of the extension.

** Currently, this facility is not ready for use.

Item 3. Legal Proceedings.

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, and plaintiffs were afforded 28 days to file a consolidated amended complaint. The plaintiffs' consolidated amended complaint, filed on July 21, 2014, sought to bring claims on behalf of themselves and persons or entities that purchased or acquired securities of the Company between July 10, 2013 and November 11, 2013. The consolidated amended complaint alleged that Sarepta and certain of its officers violated the federal securities laws in connection with disclosures related to eteplirsen, the Company's lead therapeutic candidate for DMD, and seeks damages in an unspecified amount. Pursuant to the court's June 23, 2014 order, Sarepta filed a motion to dismiss the consolidated amended complaint on August 18, 2014, and argument on the motion was held on March 12, 2015. On March 31, 2015, the Court dismissed plaintiffs' amended complaint. On April 30, 2015, plaintiffs in the Corban suit filed a motion for leave seeking to file a further amended complaint, which the Company opposed. Following a hearing on August 12, 2015, the Court denied this motion, and on September 22, 2015, the Court dismissed the case. The plaintiffs filed a Notice of Appeal in the Court of Appeals for the First Circuit on September 29, 2015. On January 27, 2016, the plaintiffs filed a motion to vacate the District Court's order denying leave to amend and dismissing the case. Defendants filed their opposition with the District Court on February 11, 2016, and oral argument on the plaintiffs' motion was held on February 25, 2016. The plaintiffs' appellate brief is due to the First Circuit on March 22, 2016.

Another complaint was filed in the U.S. District Court for the District of Massachusetts on December 3, 2014 by 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), asserting violations of Section 10(b) of the Exchange Act and Securities and Exchange Commission Rule 10b-5 against the Company, Christopher Garabedian and Sandesh Mahatme. Plaintiffs' amended complaint, filed on March 20, 2015, alleges that the 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 a new drug application ("NDA") for eteplirsen and the likelihood of the Food and Drug Administration ("FDA") accepting the NDA based on that data. Plaintiffs seek compensatory damages and fees. The Company received service of the complaint on January 5, 2015. Sarepta filed a motion to dismiss the complaint on May 11, 2015, pursuant to the scheduling order entered on February 20, 2015, which plaintiffs have opposed. Oral argument on the motion has been scheduled for March 2, 2016.

In addition, two derivative suits were filed based upon the Company's disclosures related to eteplirsen. On February 5, 2015, a derivative suit was filed against the Company's Board of Directors in the 215th Judicial District of Harris County, Texas (David Smith, derivatively on behalf of Sarepta Therapeutics, Inc., v. Christopher Garabedian et. al, Case 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. On March 24, 2015, the parties agreed to abate the case pending the resolution of both suits pending in federal court in the District of Massachusetts, Corban and Kader. Additionally, on February 24, 2015, a derivative suit was filed against the Company's Board of Directors with the Court of Chancery of the State of Delaware (Ira Gaines, and the Ira J.

Gaines Revocable Trust U/A, on behalf of nominal defendant Sarepta Therapeutics, Inc., vs. Goolsbee et. al., No. 10713). The claims allege that the defendants participated in making material misrepresentations or omissions during the period of April 21, 2014 through October 27, 2014, regarding the sufficiency of the Company's data for submission of the NDA for eteplirsen and the likelihood of the FDA accepting the NDA based on that data. Plaintiffs seek unspecified compensatory damages, punitive damages, actions to reform and improve corporate governance and internal procedures, and attorneys' fees. On March 26, 2015, the parties agreed to stay the case pending the resolution of Kader, pending in federal court in the District of Massachusetts.

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 vs. 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 CEO, 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. We have reached an agreement in principle with the parties in the McDonald suit and do not believe that disposition of the McDonald suit should have a material financial impact on the Company.

Item 4. Mine Safety Disclosures.

Not applicable.

PART II

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

Market Information

Our Common Stock is quoted on The NASDAQ Global Select Market under the symbol "SRPT." Prior to January 2, 2014, our Common Stock was quoted on The NASDAQ Global Market. 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, 2015 | | |
| First Quarter | $15.74 | $11.33 |
| Second Quarter | $33.16 | $12.01 |
| Third Quarter | $41.47 | $28.19 |
| Fourth Quarter | $41.97 | $23.09 |
| Year Ended December 31, 2014 | | |
| First Quarter | $31.28 | $17.50 |
| Second Quarter | $40.00 | $20.89 |
| Third Quarter | $31.35 | $18.59 |
| Fourth Quarter | $24.95 | $12.58 |

Holders

As of February 19, 2016, we had 240 stockholders of record of our common stock.

Dividends

We did not declare or pay cash dividends on our common stock in 2015, 2014 or 2013. 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, 2010 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 U.S. Securities and Exchange Commission 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.

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, | | | | |
|---|---|---|---|---|---|
| | 2015 | 2014 | 2013 | 2012 | 2011 |
| | (in thousands, except per share amounts) | | | | |
| Operations data: | | | | | |
| Revenue | $1,253 | $9,757 | $14,219 | $37,329 | $46,990 |
| Research and development | 146,394 | 94,231 | | | |