### **UNITED STATES** SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

### FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 8, 2018

# Sarepta Therapeutics, Inc. (Exact name of registrant as specified in its charter)

Delaware (State or other jurisdiction of incorporation)

001-14895 (Commission File Number) 93-0797222 (IRS Employer Identification No.)

215 First Street Suite 415 Cambridge, MA 02142 (Address of principal executive offices, including zip code)

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

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):	
	Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
	Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
	Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
	Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))
multi-cate by sheet made whether the registration as marging growth commons or defined in Dule 405 of the Committee Act of 1022 (\$220.405 of this sheeten) or Dule 12b. 2 of the Committee Evaluation	

Emerging growth company  $\; \square \;$ 

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

### Item 2.02 Results of Operations and Financial Condition.

On January 8, 2017, Douglas S. Ingram, President and Chief Executive Officer of Sarepta Therapeutics, Inc. (the "Company") disclosed certain preliminary financial information for the year ended December 31, 2017 during the Company's presentation at the 36th Annual J.P. Morgan Healthcare Conference (the "Conference") and in discussions with third parties at the Conference. Specifically, the Company disclosed that the Company generated approximately \$57.3 million in revenue (unaudited) in the year ended December 31, 2017 from sales of EXONDYS 51® (eteplirsen) Injection. The Company also issued a press release disclosing such information. A copy of the slide presentation associated with this announcement is furnished as Exhibit 99.1 and is incorporated herein by reference. A copy of the press release is furnished as Exhibit 99.2 and is incorporated herein by reference.

The information in this Item 2.02 is unaudited and preliminary, and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2017 and its results of operations for the three months and year ended December 31, 2017. The audit of the Company's financial statements for the year ended December 31, 2017 is ongoing and could result in changes to the information in this Item 2.02.

### Item 7.01 Regulation FD Disclosure.

The disclosure in Item 2.02 above is hereby incorporated by reference into this Item 7.01.

The slides presented by Mr. Ingram at the Conference on January 8, 2018 are furnished with this report as Exhibit 99.1, which is incorporated herein by reference.

The information in this report and Exhibits 99.1 and 99.2 to this report is furnished pursuant to Items 2.02 and 7.01 and shall not be deemed "filed" for the purposes of Section 18 of the Securities and Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section. It may only be incorporated by reference in another filing under the Exchange Act or the Securities Act of 1933, as amended, if such subsequent filing specifically references the information furnished pursuant to Items 2.02 and 7.01 of this report.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit Number Description

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99.1 Sarepta Therapeutics, Inc. Presentation at the 36th Annual J.P. Morgan Healthcare Conference, dated January 8, 2018.

99.2 <u>Press Release dated January 8, 2018.</u>

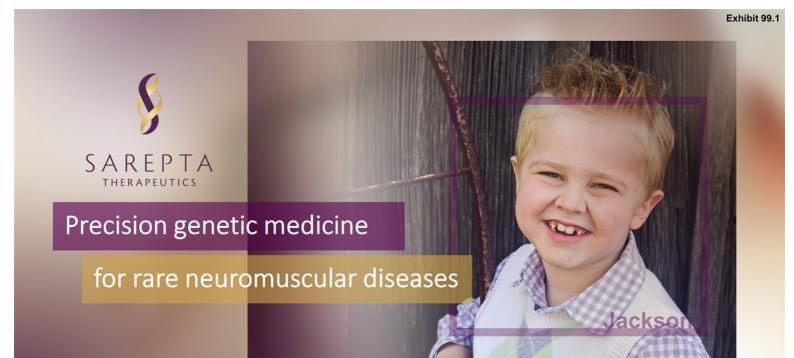
SIGNATURES

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

Sarepta Therapeutics, Inc.

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

Date: January 8, 2018



JANUARY 8, 2018 NASDAQ: SRPT

36TH ANNUAL JP MORGAN HEALTHCARE CONFERENCE SAN FRANCISCO, CALIFORNIA

### FORWARD-LOOKING STATEMENTS

This presentation contains "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Statements that are not historical facts or words such as "believes," "anticipates," "palnas," "expects," "will," "intends," "potential," "possible," "goal," "strategy," "may," "should," "project," "estimate," and similar expressions are intended to identify forward-looking statements in this presentation include but are not limited to: Sarepta's aspiration to be a global top-five rare-disease focused company, applying its expertise in precision genetic medicine to address a variety of neuromuscular conditions; Sarepta having all the elements in place for success; Sarepta's goal to develop life-changing precision genetic medicines to treat 100% of individuals with DMD and apply its therapeutic approach to other rare neuromuscular diseases; Sarepta's new clinical studies evaluating potential life-changing modalities; Sarepta's raised capital intending to accelerate R&D, expand talent base and support partnering; 2018 being a year of transformation for Sarepta; Sarepta's key milestones and inflection points for 2018, including FDA meeting on golodirsen in Q1, CHMP decision for eteplirsen in mid-2018, submitting an IND for SRP-5053 in H2 2018, and having certain readouts and generating data from studies; Sarepta's plan to develop treatments for all eligible individuals with DMD; Sarepta building a global franchise in neuromuscular diseases and its's product development strategy and possibilities; Sarepta's pipeline, technologies and next-generation approaches and their respective potential benefits, including PPMO potentially being a transformative approach to treating DMD and slowing disease progression in DMD with potentially less frequent dosing in the clinic and application in other therapeutic areas, and the potential of Sarepta's programs with its partners to address all individuals with DMD, including the promise of micro-dystrophin gene thera

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control and are based on Sarepta's current beliefs, expectations and assumptions regarding it business. Actual results and financial condition could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties and could materially and adversely affect Sarepta's business, results of operations and trading price. Potential known risk factors include, among others, the following: the audit of our financial statements for the year ended December 31, 2017 is ongoing and could result in changes to the information; we may not be able to meet expectations with respect to EXONDYS 51 sales or attain the net revenue we anticipate for 2018, profitability or positive cash-flow from operations; we may not be able to achieve our expected base case and the expected year-over-year revenue growth; we may not be able to comply with all FDA post-approval commitments/requirements with respect to EXONDYS 51 in a timely manner or at all; we may not be able to obtain regulatory approval for eteplicien in jurisdictions outside of the U.S., including from the EMA; the results of our ongoing research and development efforts, including those with strategic partners, and clinical trials for our product candidates, including, PPMO, golodirsen, casimersen and gene therapy, may not be positive or consistent with prior results or demonstrate a safe treatment benefit which could negatively impact our business; we may not be able to execute on our business plans and goals, including meeting our expected or planned regulatory milestones and timelines, clinical development plans, and bringing our product candidates to market, for various reasons including possible limitations of our financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, results of research and development efforts and/or clinical trials may not be posit

Sarepta Therapeutics aspires to be a **global top- five rare-disease focused** company, applying its
expertise in **precision genetic medicine** to address
a variety of **neuromuscular conditions**.

## ALL THE ELEMENTS IN PLACE FOR SUCCESS



pipeline



Funded for the future



**Strong foundation** 



**Urgent** mission

## **DRIVEN BY AN URGENT MISSION**

Our goal is to develop lifechanging precision genetic medicines to treat 100 percent of individuals with Duchenne muscular dystrophy (DMD) and apply our therapeutic approach to other rare neuromuscular diseases where it is likely to have the most benefit



## 2017: SETTING THE STAGE FOR SUCCESS









R&D

**FINANCIAL** 

**EXECUTED** 

TOP 5

SIGNED

INITIATED

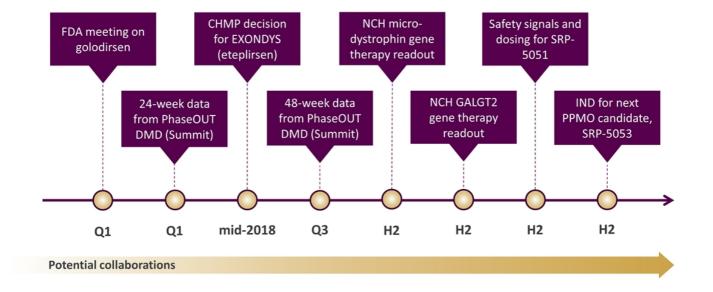
clinical studies evaluating potential lifechanging modalities

**ADDED** 

to accelerate R&D, expand talent base, and support partnering

## 2018: A YEAR OF TRANSFORMATION

**KEY MILESTONES/INFLECTION POINTS** 



## OUR PATH TO DEVELOPING TREATMENTS FOR ALL ELIGIBLE INDIVIDUALS WITH DMD

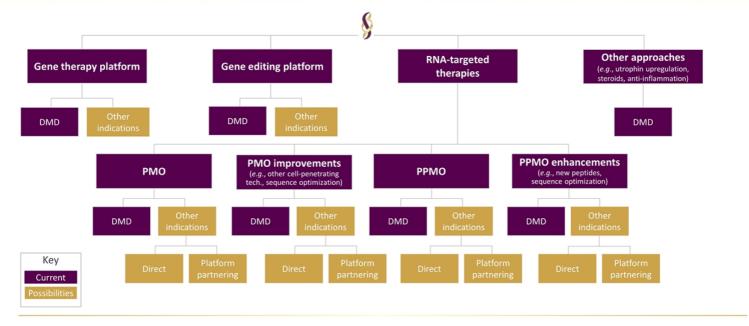


External Collaborations

<sup>\*</sup>EXONDYS 51 received accelerated approval in the U.S., confirmatory studies required \*\*Other exon targets in development: 8, 35, 43, 44, 50, and 55

## BUILDING A GLOBAL FRANCHISE IN NEUROMUSCULAR DISEASES

**DEVELOPMENT STRATEGY** 



### WHAT IS DMD?1-2

### AFFECTS 1 IN 3,500-5,000 MALES BORN WORLDWIDE

- Rare, progressive neuromuscular genetic disease that is 100 percent fatal
- Average lifespan of mid- to late-20s; typical diagnosis occurs between ages 4-5
- Caused by gene mutation that encodes dystrophin, a protein that exists in infinitesimally small amounts in the body (0.002 percent of muscle), but plays a key structural role in muscle fiber production
- Even small amounts of dystrophin production have shown significant benefits (e.g. Becker Muscular Dystrophy and exon 44 amenable individuals)



<sup>1.</sup> Emery AE, Population frequencies of inherited neuromuscular diseases—a world survey. Neuromuscul Disord. 1991;1(1):19–29pm

<sup>2.</sup> Emery AE. The muscular dystrophies. BMJ. 1998;317(7164):991



## USING PRECISION RNA SPLICING TO CORRECT GENETIC DEFECTS

Our RNA platform results in genetic medicine precisely engineered to induce pre-mRNA splicing, which is designed to transform mRNA and produce a truncated but functional protein

### Sarepta Therapeutics is currently advancing two RNA-targeted technologies

# Phosphorodiamidate Morpholino Oligomer (PMO)

- Synthetically designed structures modeled after the natural framework of RNA
- Near-term opportunity with potential to treat 29 percent of individuals with DMD by 2019



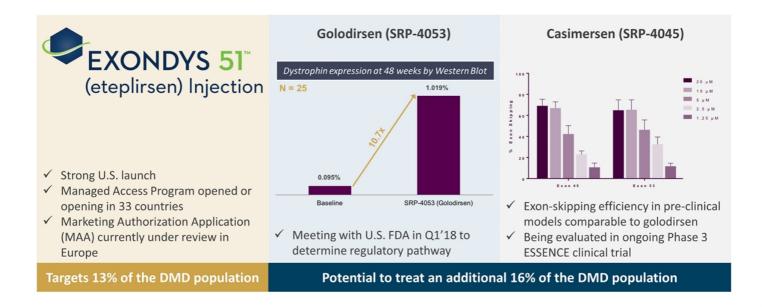
### Peptide Phosphorodiamidate Morpholino Oligomer (PPMO)

- Designed to enhance tissue targeting, intracellular delivery, target selectivity, and potency
- Potentially transformative approach to slowing disease progression in DMD with application in other therapeutic areas



## PMO PROGRAMS: SEVERAL POTENTIAL NEAR-TERM CATALYSTS

APPROVED THERAPY AND LATE-STAGE CANDIDATES TARGET 29 PERCENT OF INDIVIDUALS WITH DMD



## PPMO: A TRANSFORMATIVE APPROACH TO TREATING DMD



### SRP-5051: first PPMO candidate in the clinic

STATUS • Phase 1/2a clinical trial initiated in November 2017

• Multi-center, double blind, placebo-controlled, multi-dose efficacy portion of study to initiate by mid-2018 or as soon as a therapeutic dose has been identified



Superior delivery vehicle



Induces significant increases in dystrophin levels



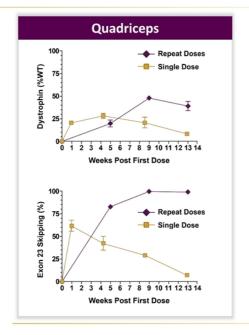
Potentially less frequent dosing in the clinic

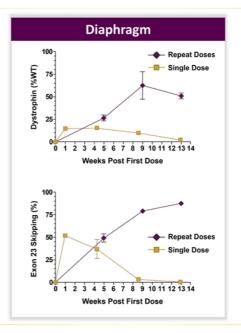


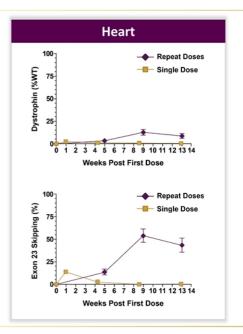
Achieves delivery in the three major muscle groups affected in DMD

Sources: Internally generated data 14

# REPEAT PPMO DOSES INCREASED AND SUSTAINED DYSTROPHIN PRODUCTION IN VIVO

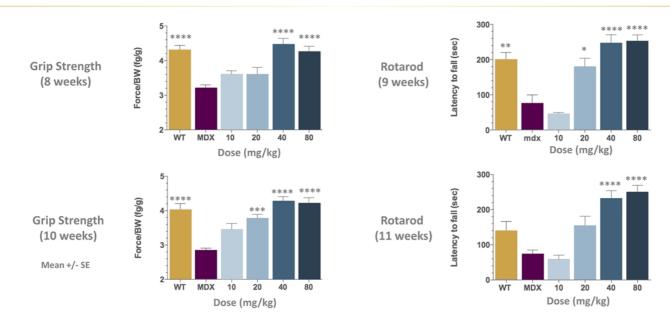






mdx (DMD) mice were treated with a three monthly IV doses of PPMO @ 40 mg/kg; the single dose PPMO cohort was included as a comparator

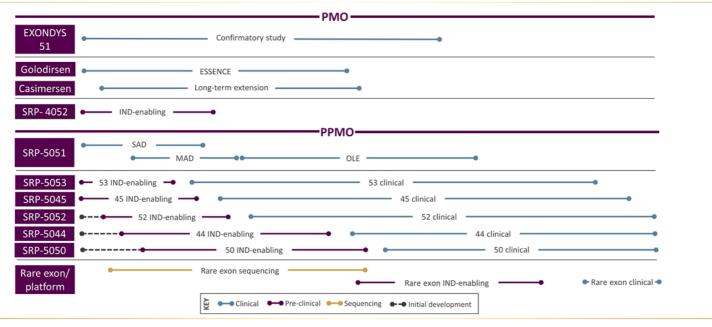
## PPMO TREATMENT IMPROVED MUSCLE FUNCTION IN MDX MICE



- mdx (DMD) mice at 7 weeks of age were treated with a single IV dose of saline or PPMO @ 10, 20 40 or 80 mg/kg and WT mice at 7 weeks of age were treated with a single IV dose of saline
  Mice were tested for grip strength at 8 weeks (1 week post-injection, pi) and 10 weeks (3 weeks pi) of age, and for rotarod performance at 9 weeks (2 weeks pi) and 11 weeks (4 weeks pi) of age (n=10 per group)
  Graphs are mean +/- SE; Statistics performed was the One Way Anova Tukey Multiple Comparison Test and the significant values shown are versus mdx saline (\*p<0.05, \*\*p<0.01, \*\*\*p<0.001, \*\*\*p<0.001)

## RNA-TARGETED THERAPY FRANCHISE DEVELOPMENT STRATEGY\*

POTENTIAL FOR UP TO SEVEN APPROVED THERAPIES IN THE U.S. BY 2022



\*timeline not to scale



## LEADERS IN GENE THERAPY AND EDITING FOR DMD

POTENTIAL TO ADDRESS ALL INDIVIDUALS WITH DMD











## MICRO-DYSTROPHIN GENE THERAPY OVERVIEW

PHASE 1/2A CLINICAL TRIAL UNDERWAY; INTERIM SAFETY AND EFFICACY DATA EXPECTED IN 2H'18

Program developed and led by gene therapy pioneers Jerry Mendell, M.D. and Louise Rodino-Klapac, Ph.D.



Uses an adeno-associated virus vector to deliver a shortened version of the dystrophin gene to replace the missing protein in individuals with DMD

### THE PROMISE OF MICRO-DYSTROPHIN GENE THERAPY IN DMD

GENETHON DATA PUBLISHED IN NATURE COMMUNICATIONS

- Study conducted in 12 dogs naturally affected by DMD and treated with Genethon's microdystrophin gene therapy
- At two-year follow-up, muscle function was significantly restored and clinical symptoms had stabilized
- Dystrophin expression had returned to a high level in the high-dose group
- No immunosuppressive treatment was administered beforehand, and no side-effects were observed



Video courtesy of:

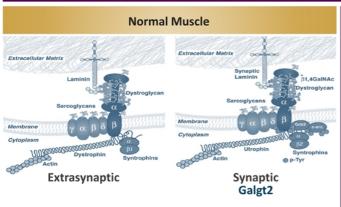


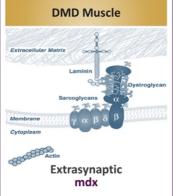
Discovered and developed with Genethon

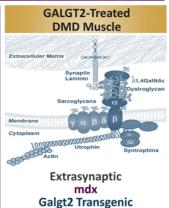
## **GALGT2 GENE THERAPY OVERVIEW**

### POTENTIAL TO RESTORE MUSCLE FUNCTION DESPITE THE ABSENCE OF THE DYSTROPHIN PROTEIN

TARGET Targets the dystroglycan complex to enhance utrophin expression—a largely homologous protein to dystrophin—and preserve muscle function regardless of underlying genetic mutation







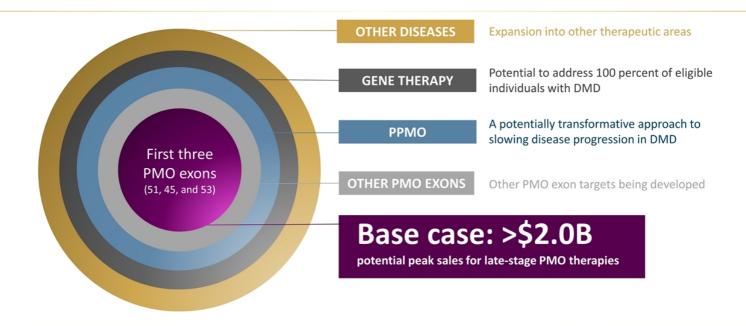
### **STATUS**

- Dose escalation trial beginning with the minimal efficacious dose as determined by pre-clinical studies
- Interim read-out assessing safety and efficacy signals in 2H'18

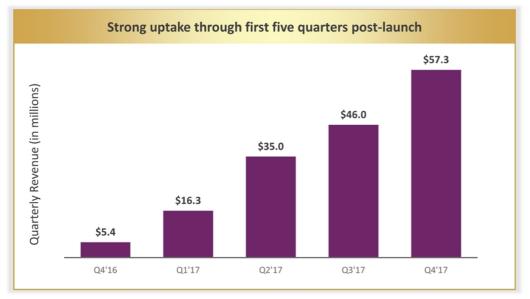
Adapted from Flanigan 2014 PPMD presentation



## **OUR BASE CASE REPRESENTS A SIGNIFICANT OPPORTUNITY**



# **EXONDYS 51: STRONG QUARTERLY GROWTH**





\*Unaudited

# **EXONDYS 51: STRONG GROWTH IN 2018**



\*Unaudited 26





### Sarepta Therapeutics Pre-Announces Fourth Quarter 2017 Revenue and Provides Full-Year 2018 Revenue Guidance for EXONDYS 51® (eteplirsen), Representing Approximately 100 Percent Year-over-Year Growth

- Fourth quarter 2017 EXONDYS 51 unaudited revenue of \$57.3 million -
- Full-year 2017 EXONDYS 51 unaudited revenue of \$154.6 million —
- Full-year 2018 EXONDYS 51 revenue guidance of \$295 \$305 million —
- 16 programs in development –

SAN FRANCISCO, Calif., January 8, 2018 (GLOBE NEWSWIRE) — Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, today pre-announced its fourth quarter 2017 revenue and also provided full-year 2018 revenue guidance for EXONDYS 51 during the Company's presentation at the 36th Annual J.P. Morgan Healthcare Conference. Sarepta's president and chief executive officer, Douglas Ingram, who presented on behalf of the Company, stated that revenue for the fourth quarter will total \$57.3 million and \$154.6 million for the full-year of 2017. EXONDYS 51 revenue guidance for 2018 will be in the range of \$295 – \$305 million.

"2017 was a remarkable year for Sarepta," said Douglas Ingram, Sarepta's president and chief executive officer. "Our full-year 2017 revenue, representing one of the most successful rare disease launches in history, speaks to the value of EXONDYS 51 and also to the ability of our talented colleagues at Sarepta to execute on our plans and deliver on our commitments."

Mr. Ingram continued, "In 2017, we set the stage by successfully launching our first therapy, entering into important collaborations and ensuring we have ample resources to invest in our impressive pipeline. In 2018, we are accelerating our plans, moving 16 programs through various stages of development, and planning for multiple milestones and inflection points. And we are doing all of this to serve our lofty but achievable aspiration: to improve and extend the lives of the thousands of children suffering from DMD,

expand our platform to other rare diseases, and in so doing, to become one of the most important global leaders in precision genetic medicine to reduce human suffering and treat disease."

#### About EXONDYS 5

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. Data from clinical studies of EXONDYS 51 in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of EXONDYS 51 has not been established.

### Important Safety Information About EXONDYS 51

Adverse reactions in DMD patients (N=8) treated with EXONDYS 51 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (EXONDYS 51, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

In the 88 patients who received <sup>3</sup>30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in <sup>3</sup>10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

There have been reports of transient erythema, facial flushing, and elevated temperature occurring on the day of EXONDYS 51 infusion.

For further information, please see the full Prescribing Information.

#### About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

#### Forward-Looking Statements

This press release contains forward-looking statements. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding Sarepta's revenue from EXONDYS 51 in the fourth quarter of 2017 and in the year 2017; Sarepta's full-year 2018 EXONDYS 51 revenue guidance and approximately 100 percent year-over-year growth; Sarepta's full year 2017 revenue representing one of the most successful rare disease launches in history, and speaking to the value of EXONDYS 51 and also to the ability of Sarepta's left the successful rare disease launches in history, and speaking to the value of EXONDYS 51 and also to the ability of Sarepta's lofty but achievable aspiration to improve and extend the lives of the thousands of children suffering from DMD, expand its platform to other rare diseases and become one of the most important global leaders in precision genetic medicine to reduce human suffering and treat disease.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the audit of Sarepta's financial statements for the year ended December 31, 2017 is ongoing and could result in changes to the information; Sarepta may not be able to meet expectations with respect to EXONDYS 51 sales or attain the net revenues it anticipates for 2018, profitability or positive cash-flow from operations; Sarepta may not be able to comply with all FDA post-approval commitments/requirements with respect to EXONDYS 51 in a timely manner or at all; Sarepta may not be able to obtain regulatory approval for eteplirsen in jurisdictions outside of the U.S., including from the EMA; the results of Sarepta's ongoing research and evelopment efforts, including those with strategic partners, and clinical trials for Sarepta's product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit which could negatively impact its business; Sarepta may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its product candidates to market, for various reasons including possible limitations of Sarepta's financial and other

resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, results of research and development efforts and/or clinical trials may not be positive, and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2016 and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's 2016 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2017 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta cautions investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

### Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at <a href="https://www.sarepta.com">www.sarepta.com</a>. We encourage investors and potential investors to consult our website regularly for important information about us.

Source: Sarepta Therapeutics, Inc.

Media and Investors: Sarepta Therapeutics, Inc. Ian Estepan, 617-274-4052 <u>iestepan@sarepta.com</u>

OI

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