



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 51

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 ≥ 30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in $\geq 10\%$ 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 talented employees to execute on Sarepta's plans and deliver on its commitments; Sarepta accelerating its plans in 2018, moving 16 programs through development, and planning for multiple milestones and inflection points; and 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 development 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 ex-

pected 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. 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 www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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