Sarepta Therapeutics Announces Publication of Long-Term Pulmonary Function of Eteplirsen-Treated Patients Compared to Natural History of Duchenne Muscular Dystrophy in *The Journal of Neuromuscular Diseases*

CAMBRIDGE, Mass., December 27, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases, today announced that the pulmonary function results from eteplirsen-treated Duchenne muscular dystrophy (DMD) patients (N=12) in Study 201/202 compared to natural history were published in the December 20, 2017 online edition of *The Journal of Neuromuscular Diseases*. A statistically significant and clinically meaningful reduction in pulmonary decline as measured by forced vital capacity percent predicted (FVC%p) was observed for eteplirsen-treated patients as compared to natural history data published in the scientific literature.

In eteplirsen-treated patients, the mean FVC%p decreased from 97.7% to 85.3% over 216 weeks, a decrease of 2.8% per study year. In an age-adjusted mixed-model repeated-measures (MMRM) analysis of FVC%p, an annual decrease of 2.3% was observed for eteplirsen-treated patients compared to an annual decrease of 4.1% observed in a natural history cohort with a similar age range from the United Dystrophinopathy Project (UDP).

“Patients treated with eteplirsen in Study 201/202 experienced significantly less deterioration of respiratory muscle function than natural history would predict,” said Douglas Ingram, Sarepta’s president and chief executive officer. “The results included both ambulant and non-ambulant patients who received eteplirsen. Our mission is to develop and bring to the community precision genetic therapies that can improve the lives of those suffering from DMD, a cruel degenerative disease.”

About Eteplirsen

Eteplirsen uses Sarepta’s proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. Eteplirsen 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 eteplirsen 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 eteplirsen has not been established.

Important Safety Information About Eteplirsen

Adverse reactions in DMD patients (N=8) treated with eteplirsen 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 (eteplirsen, 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 eteplirsen is not recommended.

In the 88 patients who received ≥30 mg/kg/week of eteplirsen for up to 208 weeks in clinical studies, the following events were reported in ≥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 eteplirsen 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 medicines 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 the results of Study 201/202 and Sarepta's mission to develop and bring to the community precision genetic therapies that can improve the lives of those suffering from DMD.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the results of Sarepta's studies for eteplirsen, or other product candidates, *may not be positive or consistent with prior results or demonstrate a safe treatment benefit; Sarepta may not be able to meet expectations with respect to the sales of its therapy for the treatment of DMD; Sarepta may not be able to execute on its business plans, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its products to U.S. and ex-U.S. markets for various reasons including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, and regulatory, court or agency decisions; 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. We caution 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.

Source: Sarepta Therapeutics, Inc.

Media and Investors:
Sarepta Therapeutics, Inc.

Ian Estepan, 617-274-4052

iestepan@sarepta.com

or

W2O Group

Brian Reid, 212-257-6725

breid@w2ogroup.com