Sarepta Therapeutics Announces FDA Clearance of IND for the Company's PPMO Exon 51 Candidate, SRP-5051

-- Sarepta to immediately initiate a Phase 1/2a clinical trial in patients with Duchenne muscular dystrophy (DMD) amenable to skipping exon 51 --

CAMBRIDGE, Mass., November 7, 2017 (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, announced today that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for the Company's peptide phosphorodiamidate morpholino oligomer (PPMO) exon 51 candidate, SRP-5051. Sarepta will immediately initiate its Phase 1/2a clinical trial and begin screening patients with DMD amenable to skipping exon 51.

Results of the Phase 1/2a trial will inform the multi-center, double blind, placebo-controlled, multi-dose efficacy study to evaluate dystrophin expression and clinical outcomes, and is planned to initiate by mid-year 2018 or as soon as a therapeutic dose has been identified.

Sarepta's next-generation PPMO platform is designed around a proprietary cell-penetrating peptide conjugated to the phosphorodiamidate morpholino oligomer (PMO) backbone, with the goal of increasing tissue penetration.

"Our PMO platform, on which EXONDYS 51 is based, represents precision genetic medicine that is designed to elegantly engineer the expression of dystrophin through modification of mRNA in muscle cells. Our next-generation platform, PPMO, greatly advances this science by using a proprietary peptide technology to act as a transporter of the PMO into the muscle cell, which in animal models has substantially increased mRNA modification and dystrophin production," said Douglas Ingram, Sarepta's president and chief executive officer. "SRP-5051, the first clinical candidate from our PPMO platform, represents a dec-

ade's long investment and tireless work by Sarepta and its scientists. Preclinical models point to the potential of this next-generation class of chemistry to substantially increase efficacy while reducing the frequency of dosing."

Mr. Ingram continued, "DMD is a cruel and, at least today, an invariably fatal disease. The clearance of this IND to bring SRP-5051 to the clinic is not merely a success for Sarepta. More importantly, it represents a potential approach for a better life for children living with DMD. For that reason, we intend to move urgently in further pursuit of our goal of translating promising science into potentially life-saving and life-enhancing medicines. Without delay, Sarepta will begin enrolling patients in the study to identify a safe and therapeutic dose of SRP-5051. Beyond that, we have crafted an ambitious strategy to sequence and rapidly advance multiple PPMO-based therapies designed to change the course of DMD. In parallel, if we have positive signals in our first trial, we will explore the potential of applying our PPMO technology to a broad range of other neuromuscular diseases."

About SRP-5051

SRP-5051 uses Sarepta's PPMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051 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. PPMO is Sarepta's next-generation chemistry platform designed around a proprietary cell-penetrating peptide conjugated to the PMO backbone, with the goal of increasing tissue penetration, increasing exon skipping and significantly increasing dystrophin production. If successful, the PPMO offers the potential for improved efficacy and less frequent dosing for patients.

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 ≥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 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 Sarepta's plan to immediately initiate a Phase 1/2a clinical trial in patients with DMD amenable to skipping exon 51; the study design; the results of the Phase 1/2a trial informing the multi-center, double blind, placebo-controlled, multi-dose efficacy study to evaluate dystrophin expression

and clinical out-comes, and the plan to initiate such study by mid-year 2018 or as soon as a therapeutic dose has been identified; the goal of Sarepta's next-generation PPMO platform of increasing tissue penetration; the PMO platform representing precision genetic medicine that is designed to elegantly engineer the expression of dystrophin through modification of mRNA in muscle cells and the PPMO greatly advancing this science; the potential of PPMO to substantially increase efficacy while reducing the frequency of dosing; bringing SRP-5051 to the clinic representing a potential approach for a better life for children living with DMD; Sarepta's intention to move urgently in further pursuit of its goal of translating promising science into potentially life-saving and life-enhancing medicines; Sarepta's plan to begin enrolling patients in the study to identify a safe and therapeutic dose of SRP-5051; Sarepta's ambitious strategy to sequence and rapidly advance multiple PPMO-based therapies designed to change the course of DMD and to explore the potential of applying the PPMO technology to a broad range of other neuromuscular diseases; PPMO's goal of increasing tissue penetration, increasing exon skipping and significantly increasing dystrophin production; and the potential of PPMO for improved efficacy and less frequent dosing for patients.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: there may be delays in the study timelines and Sarepta may not be able to successfully complete the study for various reasons, including any negative or inconsistent safety and efficacy data; the study data and results may not provide support for an NDA filing; Sarepta's PPMO technology, including SRP-5051, may not result in any viable treatments suitable for commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates; and even if PPMO results in commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; 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