Sarepta Therapeutics Completes Submission of New Drug Application Seeking Approval of Golodirsen (SRP-4053) in Patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 53

-- Golodirsen has been studied for the treatment of exon 53 amenable patients, approximately eight percent of patients with Duchenne --

-- Submission represents ongoing advancement of the company's proprietary PMO RNA-based platform --

CAMBRIDGE, Mass., December 20, 2018 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a leader in precision genetic medicine for rare diseases, announced today that it has completed the submission of its rolling New Drug Application (NDA) seeking accelerated approval for golodirsen (SRP-4053), a phosphordiamidate morpholino oligimer engineered to treat those patients with Duchenne muscular dystrophy who have genetic mutations subject to skipping exon 53 of the Duchenne gene. Duchenne is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500 - 5,000 males born worldwide.

The completion of the rolling submission for golodirsen includes data from the 4053-101 study assessing the safety, tolerability, pharmacokinetics and dystrophin expression of golodirsen in 25 boys with confirmed deletions of the DMD gene amenable to exon 53 skipping. The study demonstrated statistically significant results in favor of golodirsen on all biological endpoints, including properly exon-skipped RNA transcript using reverse transcription polymerase chain reaction, increase in quantity of dystrophin expression from baseline using Western blot and increase in dystrophin intensity as measured by immunohistochemistry.

If the golodirsen NDA is filed and granted accelerated approval, the company's ESSENCE study (4045-301) could serve as a post-marketing confirmatory study. ESSENCE, which is under way, is a global, randomized double-blind, placebo-controlled study assessing the safety and efficacy of golodirsen and casimersen, our exon 45 skipping therapy.

Golodirsen is a phosphordiamidate morpholino oligimer engineered to treat those patients with Duchenne muscular dystrophy (DMD) who have genetic mutations subject to skipping exon 53 of the DMD gene. Patients with a 53 mutation represent 8 percent of those with Duchenne.

"We are grateful for the patients and clinicians who have participated in the study with an aim to advance treatment for all patients with Duchenne," said Doug Ingram, Sarepta's president and chief executive officer. "Sarepta is committed to developing therapies to benefit the greatest possible percentage of patients affected by Duchenne. Our proprietary PMO technology remains central to our commitment to patients with Duchenne. Combined, EXONDYS 51® (eteplirsen), golodirsen, and casimersen, have the potential to treat nearly 30 percent of patients with Duchenne," added Ingram.

About Golodirsen

Golodirsen uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 53 of the DMD gene. Golodirsen is designed to bind to exon 53 of dystrophin pre-mRNA, resulting in exclusion, or "skipping," of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 53 skipping. Exon skipping is intended to allow for production of an internally truncated but functional dystrophin protein.

Golodirsen is one of the investigational candidates currently being evaluated in the ESSENCE study, a global, randomized double-blind, placebo-controlled study evaluating efficacy and safety in patients amenable to skipping exons 45 or 53.

Dystrophin is a protein found in muscle cells that, while present in extremely small amounts (about 0.002 percent of total muscle protein), is crucial in strengthening and protecting muscle fibers. A devastating and incurable muscle-wasting disease, DMD is associated with specific errors in the gene that codes for dystrophin, a protein that plays a key structural role in muscle fiber function. Progressive muscle weakness in the lower limbs spreads to the arms, neck and other areas of the body. The condition is universally fatal, and death usually occurs before the age of 30 generally due to respiratory or cardiac failure.

About Duchenne Muscular Dystrophy

DMD is an X-linked rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 - 5,000 male births worldwide.

About EXONDYS 51®

EXONDYS 51, for DMD in patients who have a confirmed mutation of the DMD gene that is amenable to exon 51 skipping, was approved under accelerated approval based on an increase in dystrophin in skeletal

muscle observed in some patients treated with EXONDYS 51. A clinical benefit of EXONDYS 51 has not been established. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

Important Safety Information About EXONDYS 51

Hypersensitivity reactions, including rash and urticaria, pyrexia, flushing, cough, dyspnea, bronchospasm, and hypotension, have occurred in patients who were treated with EXONDYS 51. If a hypersensitivity reaction occurs, institute appropriate medical treatment and consider slowing the infusion or interrupting the EXONDYS 51 therapy.

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.

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in Limb-girdle muscular dystrophy (LGMD), Charcot-Marie-Tooth (CMT) and CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs span across several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is poised to be the most meaningful precision genetic medicine company in the world and make a profound difference in the lives of patients suffering from rare neuromuscular diseases and other rare diseases. 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 submission representing ongoing advancement of Sarepta's proprietary PMO RNA-based platform; the potential of golodirsen to treat those patients with DMD who have genetic mutations subject to skipping exon 53 of the Duchenne gene; the estimated patient population suffering from DMD, and the estimated patient population with a 53 mutation; the potential of the Company's ESSENCE study to serve as a post-marketing confirmatory study, if the NDA is filed and golodirsen is granted accelerated approval; Sarepta being committed to developing therapies to benefit the greatest possible percentage of patients affected by Duchenne; Sarepta's proprietary PMO technology remaining central to Sarepta's commitment to patients with Duchenne; the potential of exondys51, golodirsen and casimersen to treat nearly 30 percent of patients with Duchenne; the potential of exon skipping to allow for production of an internally truncated but functional dystrophin protein; and Sarepta being poised to be the most meaningful precision genetic medicine company in the world and make a profound difference in the lives of patients suffering from rare neuromuscular diseases and other rare diseases.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: Sarepta may not be able to complete clinical trials required by the FDA or other regulatory authorities for approval of golodirsen; golodirsen may not result in a viable treatment 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 golodirsen results in a commercialized product, Sarepta may not achieve any significant revenues from the sale of such product; 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, such as decisions by the United States Patent and Trademark Office with respect to patents that cover Sarepta's product candidates; 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, 2017 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 2017 Annual Report on Form 10-K 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 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.

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