Sarepta Therapeutics Announces Positive Results in Its Study Evaluating Gene Expression, Dystrophin Production, and Dystrophin Localization in Patients with Duchenne Muscular Dystrophy (DMD) Amenable to Skipping Exon 53 Treated with Golodirsen (SRP-4053)

-- Study achieved statistical significance on all primary and secondary biological endpoints --

-- Results further validate the Company's exon-skipping platform for the treatment of DMD --

CAMBRIDGE, Mass., September 6, 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 muscle biopsy results from its 4053-101 study, a Phase 1/2 first-in-human study conducted in Europe to assess the safety, tolerability, pharmacokinetics, and efficacy of golodirsen in 25 boys with confirmed deletions of the *DMD* gene amenable to skipping exon 53. The study comprised two parts. In Part 1, 12 patients were randomized to receive a dose titration of golodirsen (8 patients) or placebo (4 patients). At the end of Part 1 (dose titration), all 12 patients continued on golodirsen and an additional 13 patients started golodirsen (Part 2). In Part 2, all 25 patients were treated for an additional 48 weeks at the time of muscle biopsy. The analysis included biopsies of the bicep muscle at baseline and on-treatment at the Part 2 Week 48 time point. All 25 participants displayed an increase in skipping exon 53 ($p < 0.001$) over baseline levels, representing a 100 percent response rate as measured by RT-PCR and demonstrating proof of mechanism. Mean dystrophin protein increased to 1.019 percent of normal compared to a mean baseline of 0.095 percent of normal ($p < 0.001$) as measured by Western blot, the primary biological endpoint in the study, representing a 10.7 fold increase from baseline. The study also showed a statistically significant increase in dystrophin immunofluorescence as measured by immunohistochemistry (IHC), the secondary biological endpoint in the study, confirming sarcolemma-associated protein expression and distribution.

Francesco Muntoni, principal investigator for this study and Pediatric Neurologist, Great Ormond Street Hospital for Children NHS Foundation Trust and the UCL Great Ormond Street Institute of Child Health, said, "All treated boys showed the anticipated exon skipping after treatment and this resulted in a mean increase of dystrophin protein, as measured by Western blot, from 0.095 percent at baseline to 1.019 percent of normal after at least one-year of treatment with golodirsen."

"These data were also supported by the highly statistically significant increase of dystrophin expression at the sarcolemma, as measured by recently developed validated methodology. This is now the second exon-skipping agent to have shown a statistically significant increase in dystrophin production, validating the exon-skipping approach to treating DMD boys with amenable mutations."

Professor Muntoni is also Director of the Dubowitz Neuromuscular Center, a leading clinical and research institution for children affected by neuromuscular disorders, and Deputy Director, for the MRC Neuromuscular Translational Research Centre at University College London.

"These data demonstrate statistically significant exon skipping, dystrophin production and localization, which further validate the broad application of our exon-skipping platform and aligns with our strategic imperative to expand and improve the treatment choices for the majority of patients with DMD," said Douglas Ingram, Sarepta Therapeutics' president and chief executive officer. "Additionally, the rigor with which we designed our methods and executed this study speaks to our commitment to continuous improvement and scientific excellence."

The full biological results from the 4053-101 study will be presented at an upcoming medical meeting or scientific conference. 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.

Golodirsen uses Sarepta Therapeutics' 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 dystrophin protein.

About the 4053-101 Study

The 4053-101 study was conducted as part of the SKIP-NMD project, which was originally funded by a European Union grant that allowed the completion of the first phase of the Phase 1/2 4053-101 study. In addition, the SKIP-NMD international project was coordinated by Professor Muntoni and involved a consortium of 10 academic partners across Europe (UK, France, Belgium and Italy) and the U.S. Several companies including Sarepta Therapeutics, Consultants for Research and Imaging (CRIS), and SYSNAV (expertise in indoor/outdoor robust navigation and positioning systems) provided new advances and techniques in translational Duchenne research, as did six patient organizations: Action Duchenne, Association Française contre les Myopathies, Duchenne Family Support Group, Duchenne Parent Project France, Duchenne Parent Project Onlus, and Muscular Dystrophy UK.

The study is titled, "*2-Part, Randomized, Double-Blind, Placebo-Controlled, Dose-Titration, Safety, Tolerability, and Pharmacokinetics Study (Part 1) Followed by an Open-Label Efficacy and Safety Evaluation (Part 2) of SRP-4053 in Patients With Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping.*"

Part 1 is a randomized, placebo-controlled dose-titration to assess safety, tolerability and pharmacokinetics of four dose levels of SRP-4053 in genotypically-confirmed DMD patients with deletions amenable to exon 53 skipping.

Part 2 is an open-label evaluation of SRP-4053 in patients from Part 1, along with newly enrolled DMD patients with deletions amenable to exon 53 skipping. Paired muscle biopsies of the biceps brachii at baseline and on-treatment were obtained to evaluate the biological endpoints from 25 patients treated with 30 mg/kg of SRP-4053 administered weekly by intravenous infusion during Part 2 of the trial. The study is scheduled to continue for 144 weeks to evaluate safety and clinical endpoints.

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 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 safety and efficacy of golodirsen (SRP-4053); study data achieving statistical significance on endpoints and validating Sarepta's exon-skipping platform and approach for the treatment of DMD boys with amendable mutations; the data demonstrating statistically significant exon skipping, dystrophin production and localization, which further validate the broad application of Sarepta's exon-skipping platform and align with Sarepta's strategic imperative to expand and improve the treatment choices for the majority of patients with DMD; the rigor with which Sarepta designed the methods and executed this study speaking to Sarepta's commitment to continuous improvement and scientific excellence; the full biological results being presented at an upcoming medical meeting or scientific conference; golodirsen continued evaluation as part of the ESSENCE study and its designed mechanism of action.

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 our ongoing research and development efforts and clinical trials for golodirsen (SRP-4053) and our other product candidates may not be positive or consistent with these or prior results or demonstrate a safe clinical benefit; the data presented in this release may not be consistent with the final data set and analysis thereof or result in a safe or effective treatment benefit; there may be delays in Sarepta's projected development or regulatory timelines for golodirsen (SRP-4053) and its other products candidates for various reasons, some of which may be outside of Sarepta's control, including regulatory, court or agency decisions, and any or all of Sarepta's product candidates may fail in development or may not receive required regulatory approvals for commercialization; and those risks identified under the heading "Risk Factors" in Sarepta's 2016 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2017 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 Sarepta'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 the Company's filings with the SEC. 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 web-site at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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