**Sarepta Therapeutics Announces Plan to Submit a New Drug Application (NDA) for Accelerated Approval of Golodirsen (SRP-4053) in Patients with Duchenne Muscular Dystrophy (DMD) Amenable to Skipping Exon 53**

-- The Company met with the FDA Division of Neurology Products in February to obtain guidance on the regulatory pathway for golodirsen --

-- The Company intends to complete a rolling NDA submission for golodirsen by year-end 2018 --

CAMBRIDGE, Mass., March 12, 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, announced today that it recently received final minutes from a February 2018 Type C meeting held with the Division of Neurology Products, United States Food and Drug Administration (the Division), to solicit the Division's guidance on the development pathway for Sarepta's therapeutic candidate, golodirsen, 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.

"Sarepta is thankful for the FDA Neurology Division's thoughtful and direct guidance regarding golodirsen," said Doug Ingram, Sarepta's president and chief executive officer. "Obviously, whether golodirsen will obtain accelerated approval is a review decision that will come after the submission and review of our NDA. But we greatly appreciate the willingness of the Neurology Division to engage and provide clear direction to us on the steps necessary to support an NDA submission for accelerated approval."

As previously announced in the third quarter of 2017, Sarepta's 4053-101 study – a Phase 1/2 study to assess the safety, tolerability, pharmacokinetics and efficacy of golodirsen in 25 boys with confirmed deletions of the *DMD* gene amenable to exon 53 skipping – demonstrated statistically significant results in favor of golodirsen on all biological endpoints, including properly exon-skipped RNA transcript using reverse transcription polymerase chain reaction, quantity of dystrophin expression using Western blot and dystrophin intensity pursuant to immunohistochemistry.

Based on the results of Study 4053-101 and informed now by FDA's feedback, Sarepta intends to complete a rolling submission of a golodirsen NDA by year-end 2018, seeking accelerated approval of golodirsen based on an increase in dystrophin protein as a surrogate endpoint.

Among other guidance:

- The Division reported that in light of the precedent of eteplirsen's approval, based on an increase in dystrophin protein as a surrogate endpoint reasonably likely to predict clinical benefit, a statistically significant increase in de novo, truncated dystrophin protein in Study 4053-101, based on a scientifically sound experimental design and rigorous analytical methods, may serve as a basis for accelerated approval of golodirsen for the treatment of Duchenne muscular dystrophy, assuming that Sarepta provides substantial evidence of the effect of golodirsen on dystrophin from a single study.
- Sarepta proposed that its Study 4045-301 (ESSENCE), a Phase 3 ongoing placebo-controlled clinical trial assessing the efficacy of golodirsen and casimersen, serve as the post-marketing confirmatory study. The Division confirmed that ESSENCE could possibly serve as a confirmatory study if golodirsen is granted accelerated approval, with the understanding that it is incumbent upon Sarepta to describe how it will successfully enroll and complete the ESSENCE study in light of an accelerated approval.
- The Division indicated that it is willing to accept a rolling submission of the NDA. The complete submission must include long-term animal toxicology studies, which will be completed in the fourth quarter of 2018. Hence, Sarepta anticipates the NDA submission will be complete in late 2018.

**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 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.

**Sarepta 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 submit an NDA for accelerated approval of golodirsen in patients with DMD who are amenable to exon 53 skipping based on an increase in dystrophin protein as a surrogate endpoint; Sarepta's intention to complete a rolling NDA submission for golodirsen by year-end 2018; the possibility of a statistically significant increase in de novo, truncated dystrophin protein in Study 4053-101 based on a scientifically sound experimental design and rigorous analytical methods to serve as a basis for accelerated approval of golodirsen for the treatment of DMD; the ability to demonstrate substantial evidence of the effect of golodirsen on dystrophin using a single study; the possibility of the ESSENCE study to serve as a confirmatory study if golodirsen is granted accelerated approval; the possibility of the FDA accepting our rolling NDA submission; our ability to complete toxicology studies in the fourth quarter of 2018;*

*the possibility of receiving accelerated approval for golodirsen; golodirsen's potential 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; and exon skipping's intention to allow for production of an internally truncated but functional dystrophin protein.*

*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 completing the NDA submission and Sarepta may not be able to submit the NDA on time or at all for various reasons, including any negative or inconsistent safety and efficacy data and regulatory, court or agency decisions; 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; 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 other Securities and Exchange Commission (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 filed with the 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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