Sarepta Announces FDA Acceptance of Golodirsen (SRP-4053) New Drug Application for Patients with Duchenne Muscular Dystrophy Amenable to Skipping Exon 53

-- FDA Grants Priority Review Status --

-- Regulatory Action Date is August 19, 2019 --

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

CAMBRIDGE, Mass., Feb. 14, 2019 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, announced the Food and Drug Administration, Division of Neurology (the Division), has accepted its New Drug Application (NDA) seeking accelerated approval for golodirsen (SRP-4053) and provided a regulatory action date of August 19, 2019. Golodirsen is a phosphordiamidate morpholino oligomer engineered to treat those individuals with Duchenne muscular dystrophy (Duchenne) who have genetic mutations subject to skipping exon 53 of the dystrophin gene. Duchenne is a fatal genetic neuromuscular disorder affecting an estimated one in approximately every 3,500 - 5,000 males born worldwide.

The company completed its NDA at the end of 2018 as part of a rolling submission and requested priority review, which was granted. The company previously received orphan drug designation for golodirsen. The NDA 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 dystrophin 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 approved, golodirsen will serve up to another 8 percent of the Duchenne community, bringing us closer to helping as many Duchenne patients as possible,” said Doug Ingram, president and chief executive

officer, Sarepta. "We look forward to working with the FDA toward advancing this important therapy and rapidly bringing it to individuals with Duchenne who are amenable to exon 53 skipping."

Golodirsen is also being studied in Sarepta's ongoing ESSENCE study (4045-301), a global, randomized double-blind, placebo-controlled study assessing the safety and efficacy of golodirsen and casimersen, our exon 45 skipping agent. The Division has previously confirmed that the ESSENCE study could possibly serve as a post-marketing confirmatory study.

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.

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 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 gene therapies for 5 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based 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 expected regulatory action date of August 19, 2019; the estimated number of patients suffering from Duchenne and golodirsen's potential to serve up to another 8 percent of the Duchenne community; the possibility of ESSENCE serving as a post-marketing confirmatory study; the potential of exon skipping to allow for production of an internally truncated but functional dystrophin protein; and Sarepta's mission to profoundly improve and extend the lives of patients with rare genetic-based 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 for approval of golodirsen; priority review designation by the FDA for golodirsen may not lead to faster development or regulatory review or approval process, and it does not increase the likelihood that golodirsen will receive marketing approval; 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; if the actual number of exon 53 amenable patients is smaller than estimated, Sarepta's revenue and ability to achieve profitability may be adversely affected; 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 web-site at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Source: Sarepta Therapeutics, Inc.

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