Sarepta Therapeutics Announces FDA Has Filed Eteplirsen NDA for the Potential Treatment of Duchenne Muscular Dystrophy for Patients Amenable to Exon 51 Skipping

- **FDA Grants Priority Review Status**
- **PDUFA Date is February 26, 2016**

CAMBRIDGE, Mass.--(BUSINESS WIRE)--August 25, 2015--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced that the U.S. Food and Drug Administration (FDA) has filed the New Drug Application (NDA) for eteplirsen for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. Approximately 13% of people with Duchenne muscular dystrophy are estimated to have a mutation addressable by Eteplirsen/exon 51 skipping.

The FDA has completed its filing review and has determined that our application is sufficiently complete to permit a substantive review. The Prescription Drug User Fee Act (PDUFA) action date for a decision on the application is February 26, 2016. The FDA has granted eteplirsen Priority Review status, which is designated to drugs that offer benefit over existing therapies, or provide a treatment where no adequate therapy exists.

“We are pleased with the FDA’s acceptance of our NDA for eteplirsen, as it represents an important milestone, not only for Sarepta, but for the Duchenne community. We look forward to continuing to work closely with the FDA during the regulatory review process,” said Edward M. Kaye, interim chief executive officer and chief medical officer. “We believe eteplirsen has the potential to make a meaningful impact on the lives of patients amenable to skipping exon 51 and we aim to build on our experience with eteplirsen to work with the FDA to inform and potentially expedite the clinical and regulatory pathway for the follow on exons, with the goal of reaching as many patients amenable to exon skipping as possible.”

About Sarepta Therapeutics

Sarepta Therapeutics is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of infectious diseases, such as drug-resistant bacteria and other rare human diseases. For more information, please visit us at www.sarepta.com.

About Eteplirsen

Eteplirsen is Sarepta's lead drug candidate and is designed to address the underlying cause of DMD by enabling the production of a functional dystrophin protein. Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable safety and tolerability profile and restoration of dystrophin protein expression.

Eteplirsen uses Sarepta's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene enabling the repair of specific genetic mutations that affect approximately 13% of the total DMD population. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from messenger RNA, or mRNA. Promoting the synthesis of a truncated dystrophin protein is intended to stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD.

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 boys born worldwide. 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. Eventually, increasing difficulty in breathing due to respiratory muscle dysfunction requires ventilation support, and cardiac dysfunction can lead to heart failure. The condition is universally fatal, and death usually occurs before the age of 30.

Forward-Looking Statements

This press release contains statements that are forward looking. 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," "may," "intends," "prepares," "looks," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements the potential DMD population that is amenable to exon 51 skipping, the potential market size for eteplirsen, the PDUFA date for the eteplirsen NDA, Sarepta's plans to continue working closely with the FDA in the regulatory review process, the potential meaningful impact of eteplirsen on the lives of patients amenable to exon 51 skipping, Sarepta's plans to build on its experience with eteplirsen and work with the FDA to inform and potentially expedite the clinical and regulatory pathway for follow-on exons and Sarepta's goal to reach as many patients with DMD amenable to exon skipping as possible.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Actual results could materially differ from those stated or implied by these forward-looking statements as a result of such risks and uncertainties. Known risk factors include the following: the estimates regarding the market size for eteplirsen or any of our product candidates may not be correct; the FDA may determine that our NDA submission for eteplirsen does not qualify for approval; an advisory committee, if any is convened, may not provide a positive recommendation to the FDA for eteplirsen; the results of our clinical trials and additional information and data we collect for the eteplirsen and our other product candidates may not be consistent with prior data or results, may not be positive and/or may not support the safety and efficacy of eteplirsen, our other product candidates and/or Sarepta's anti-sense based technology platform; there may be delays in our projected regulatory and development timelines relating to our eteplirsen NDA, clinical studies, our planned meetings and discussions with the FDA, and plans for commercializing eteplirsen and our other product candidates for various reasons including possible limitations of Company financial and other resources and regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover our product candidates; scale-up of manufacturing may not be successful and any or all of the Company's drug candidates may fail in development or may not receive required regulatory approvals for commercialization (including potentially under an accelerated pathway);

and those risks identified under the heading "Risk Factors" in Sarepta's 2014 Annual Report on Form 10-K or 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. You should not place undue reliance on forward-looking statements. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by applicable law or SEC rules.

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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