Sarepta Therapeutics Completes NDA Submission to FDA for Eteplirsen for the Treatment of Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

CAMBRIDGE, Mass.--(BUSINESS WIRE)--June 29, 2015-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced the completion of the rolling submission of a New Drug Application (NDA) to the United States Food and Drug Administration (FDA) for eteplirsen on June 26, 2015. Eteplirsen, the Company's lead drug candidate, targets the underlying cause of Duchenne muscular dystrophy and is designed to enable the production of a functional internally truncated dystrophin protein in patients with mutations amenable to exon 51 skipping. Approximately 13% of people with Duchenne muscular dystrophy are estimated to have a mutation targeted by Eteplirsen/exon 51 skipping.

"The completion of our NDA submission for eteplirsen represents the culmination of the efforts of our employees, investigators, clinical trial sites, and most importantly the patients and families of the Duchenne community," said Edward M. Kaye, interim chief executive officer and chief medical officer. "We look forward to working with the FDA during the regulatory process in pursuit of our goal of bringing eteplirsen to patients amenable to exon 51 skipping, while maintaining our organizational focus on advancing our PMO technology to target other DMD subpopulations amenable to exon-skipping as quickly as possible."

The NDA submission includes a request for Priority Review. Previously, eteplirsen has been granted Orphan and Fast Track status by the FDA.

The rolling submission of the NDA began on May 20, 2015, after the completion of a pre-NDA meeting with the FDA held on May 19, 2015.

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 life threatening diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of drug-resistant bacteria and infectious, rare and other 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 percent 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 relating to Sarepta's plans to work with the FDA during the regulatory process, the Company's goal of bringing eteplirsen to patients amenable to exon 51 skipping and plans to advance its PMO technology to target other DMD subpopulations amenable to exon-skipping as quickly 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 FDA may disagree with our conclusion that we have completed our rolling eteplirsen NDA submission and may request that we provide additional data before considering the rolling submission complete; the FDA may determine that our NDA submission for eteplirsen, even if complete, does not qualify for filing or approval; the additional information and data we collect or have collected for the eteplirsen NDA submission may not be consistent with prior data or results; we may not be able to comply with all FDA requests in a timely manner or at all; and there may be delays in our projected timelines and our expectations may not be accurate with respect to a potential commercialization of eteplirsen for various reasons, including possible limitations of Company resources and regulatory or agency decisions, scale-up of manufacturing may not be successful, we may lack the funding necessary to commercialize eteplirsen or any of our product candidates, the results of the additional eteplirsen trials the Company conducts may not support an NDA filing or approval for eteplirsen and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual and Quarterly

Reports on Forms 10-K and 10-Q, respectively, filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta, 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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