Sarepta Therapeutics Announces FDA Request For Dystrophin Data Prior To Making A Decision on Eteplirsen NDA

CAMBRIDGE, Mass.--(BUSINESS WIRE)--June 6, 2016-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced that the U.S. Food and Drug Administration (FDA) has requested that Sarepta provide dystrophin data, as measured by western blot, from biopsies already obtained from the ongoing confirmatory study of eteplirsen (PROMOVI), as part of its ongoing evaluation of the eteplirsen New Drug Application (NDA). The Company plans to submit data from thirteen patient biopsy samples, at baseline and Week 48, to the FDA over the coming weeks to facilitate a prompt decision on the NDA by the Agency.

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 rare, infectious and other diseases. For more information, please visit us at www.sarepta.com.

About Eteplirsen
Eteplirsen is designed to address the underlying cause of DMD by restoring the dystrophin messenger RNA (mRNA) reading frame, thus enabling the production of a shorter, functional form of the dystrophin protein. Eteplirsen uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. Approximately 13 percent of the DMD population is amenable to exon 51 skipping. Data from clinical studies of eteplirsen in DMD patients have demonstrated a consistent safety and tolerability profile and have also shown measurable dystrophin protein expression. Promoting the synthesis of a shorter dystrophin protein is intended to slow the decline of ambulation and mobility seen in DMD patients. There currently is no approved treatment in the United States for DMD and eteplirsen has not been approved by the FDA or any regulatory authority for the treatment of 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 3,500-5,000 males 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 "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. 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 FDA's request that the Company provide dystrophin data, as measured by western blot, from biopsies already obtained from the ongoing confirmatory study of eteplirsen (PROMOVI) as part of its ongoing evaluation of the eteplirsen NDA and the expected timelines for the Company submitting this additional data, including the Company's plans to submit data from thirteen patient biopsy samples, at baseline and Week 48, to the FDA over the coming weeks to facilitate a prompt decision on the NDA by the Agency, and the FDA's decision on the eteplirsen NDA.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: we may not be able to successfully gather all the data we currently plan to provide to the FDA or may not be able to otherwise comply with FDA requests, to the FDA's satisfaction, with respect to the additional dystrophin data requested by the FDA in a timely manner or at all; even if we provide the FDA with the additional dystrophin data requested, we do not know what the results will show and they could be inconsistent with prior data or result in an FDA finding that the data does not support approval of the eteplirsen NDA, on an expedited timeframe or at all, and the FDA may further delay its approval decision, make additional requests relating to the eteplirsen NDA, provide a complete response letter or otherwise decline to provide marketing approval for eteplirsen; we may not be able to comply with any other FDA requests relating to the eteplirsen NDA submission and the addendums we have submitted to the FDA or with respect to our ongoing or planned clinical trials, in a timely manner or at all; the FDA may further delay its decision on the eteplirsen NDA or may not provide marketing approval for eteplirsen; we may not be able to complete clinical trials required by the FDA for approval of our products or any submissions made in connection with our pipeline of product candidates; the results of our ongoing research and development efforts and clinical trials for our product candidates including eteplirsen and our technologies may not be positive or consistent with prior results or demonstrate a safe treatment benefit or support an NDA filing, positive advisory committee recommendation or marketing approval by the FDA or other regulatory authority; we may not be able to execute on our business plans including meeting our expected or planned regulatory milestones and timelines, clinical development plans and bringing our product candidates to market, including the planned commercialization of eteplirsen, for various reasons, including factors outside of the Company's control, including possible limitations of Company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner or at all, 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; 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, 2015 or Quarterly Report on Form 10-Q for the quarter ended March 31, 2016 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 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 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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