Sarepta Therapeutics Receives Notification of PDUFA Extension for Eteplirsen

-PDUFA goal date extended by standard extension period of three months to May 26, 2016

CAMBRIDGE, Mass.--(BUSINESS WIRE)--February 8, 2016--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced that the U.S. Food and Drug Administration (FDA) will require additional time to complete its review of the New Drug Application (NDA) for eteplirsen, for the treatment of Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. In a notice received from the FDA, the Prescription Drug User Fee Act (PDUFA) date for eteplirsen has been extended to May 26, 2016. The rescheduled date for the Peripheral and Central Nervous System Advisory Committee meeting has not yet been determined.

The FDA notified Sarepta that its January 8, 2016 submission of 4-year clinical effectiveness data, which included additional six minute walk test (6MWT) and loss of ambulation data compared to a historical control, has been designated as a major amendment to the NDA. The FDA stated that the PDUFA goal date has been extended by three months to allow for a full review of the submission. As described in the Sarepta Advisory Committee Briefing Document Addendum, the principal basis for establishing the effectiveness of eteplirsen is a comparison of patients in Study 201/202 to a historical control group.

“While our primary goal is to bring treatment to patients with Duchenne as quickly as possible, we appreciate the efforts of the FDA to conduct a complete review of all of the data supporting our NDA and we remain committed to working closely with them throughout the remainder of the regulatory process”, said Edward Kaye, Sarepta’s interim chief executive officer and chief medical officer.

The FDA has previously granted eteplirsen Priority Review status, which is designated for drugs that provide a treatment where no adequate therapy exists. The FDA also granted Rare Pediatric Disease Designation to eteplirsen, as well Orphan Drug Designation and Fast Track Status.

It is estimated that Duchenne muscular dystrophy affects approximately one in every 3,500 – 5,000 boys born worldwide, with 13 percent of people with the disease having mutations addressable by eteplirsen/exon 51 skipping.

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 boys 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 rescheduled PDUFA goal date for an FDA decision on our NDA for eteplirsen to allow for a full review by the FDA of the additional 6MWT and loss of ambulation data submitted by the Company on January 8, 2016 in the Sarepta Advisory Committee Briefing Document Amendment which describes the Company's principal basis for establishing the effectiveness of eteplirsen is a comparison of patients in Study 201/202 to a historical control group; Sarepta's primary goal of bringing treatment to patients with Duchenne as quickly as possible and its commitment to continue working closely with the FDA throughout the remainder of the regulatory process.; and the potential market size for eteplirsen, Forward-looking statements also include those regarding Sarepta's future business developments and actions and the timing of the same.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the FDA may further delay rescheduling or cancel the Advisory Committee meeting or may further delay the PDUFA date for our NDA for eteplirsen; any rescheduled Advisory Committee meeting may not result in decisions or recommendations that result in the approval of eteplirsen by the FDA; the results of our ongoing research and development efforts and clinical trials for eteplirsen and our other product candidates may not be positive or consistent with prior results or demonstrate a safe treatment benefit; there may be delays in Sarepta's projected regulatory and development timelines relating to additional research, clinical trials and commercialization plans of eteplirsen and the development of Sarepta's other product candidates for various reasons including possible limitations of Sarepta's financial and other resources; Sarepta may not be able to successfully complete its planned commercialization of eteplirsen or continue developing its product candidates as planned for a variety of reasons including due to regulatory, court or agency decisions, such as decisions by the USPTO with respect to patents that cover Sarepta's product candidates, or manufacturing issues; any or all of Sarepta's product candidates may fail in development or may not receive required regulatory approvals for commercialization; and those risks identified under the heading "Risk Factors" in Sarepta's 2014 Annual Report on Form 10-K or and most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 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 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.

Source: Sarepta Therapeutics, Inc.

Media and Investors:
Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com

Or

W2O Group
Ryan Flinn, 415-946-1059
Mobile: 510-207-7616
rflinn@w2ogroup.com