Sarepta Therapeutics Announces FDA Advisory Committee Meeting to Review Eteplirsen as a Treatment for Duchenne Muscular Dystrophy Amenable to Exon 51 Skipping

Advisory Committee Meeting Scheduled for January 22, 2016

CAMBRIDGE, Mass.--(BUSINESS WIRE)--December 18, 2015-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced that the Peripheral and Central Nervous System Drugs Advisory Committee of the U.S. Food and Drug Administration (FDA) will review Sarepta's New Drug Application (NDA) for eteplirsen, on January 22, 2016. The Prescription Drug User Fee Act (PDUFA) action date for completion of FDA review of the eteplirsen NDA is February 26, 2016.

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

"Duchenne muscular dystrophy is a devastating disease with no currently approved treatment available to the patients and families it affects." said Edward Kaye, M.D., Sarepta's interim chief executive officer and chief medical officer. "We look forward to discussing the efficacy and safety data included in our NDA submission for eteplirsen with the Advisory Committee, with the ultimate goal of bringing a treatment to patients with Duchenne amenable to skipping exon 51."

The FDA has granted eteplirsen Priority Review status, which is designated for drugs which 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.

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 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 Advisory Committee date to review the NDA for eteplirsen and the applicable PDUFA date, the potential market for eteplirsen, Sarepta's planned discussions with the Advisory Committee and the FDA on efficacy and safety data included in the NDA submission for eteplirsen and Sarepta's ultimate goal of bringing treatment to more patients with Duchenne amenable to skipping exon 51. 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 or cancel the Advisory Committee meeting; 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 the eteplirsen NDA and plans for commercializing eteplirsen and developing 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, scale-up of manufacturing may not be successful, and 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