**Sarepta Therapeutics Receives Rare Pediatric Disease Designation From FDA for Eteplirsen for the Potential Treatment of Duchenne Muscular Dystrophy**

CAMBRIDGE, Mass.--(BUSINESS WIRE)--August, 21, 2015-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation for eteplirsen, a potential treatment for patients with Duchenne Muscular Dystrophy (DMD) who are amenable to skipping exon 51. The Rare Pediatric Disease Designation supplements the Orphan Drug Designation and Fast Track Status previously granted by the FDA for eteplirsen.

"We are pleased that the FDA's Office of Orphan Products Development has granted eteplirsen with a Rare Pediatric Disease Designation," said Edward M. Kaye, Sarepta's interim chief executive officer and chief medical officer. We appreciate that FDA has created the Rare Pediatric Disease Priority Review Voucher program to foster development of treatments for rare pediatric diseases, a core focus for the Company, and we hope it leads to expedited treatments for children who desperately need them."

**About Rare Pediatric Disease Designation**

The FDA defines a "rare pediatric disease" as a disease that affects fewer than 200,000 individuals in the U.S. primarily aged from birth to 18 years. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application (NDA) or biologics license application (BLA) for a rare pediatric disease may be eligible for a voucher which can be redeemed to obtain priority review for a subsequent marketing application for a different product. The Priority Review Voucher may be sold or transferred an unlimited number of times.

**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 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 eteplirsen receiving rare pediatric disease designation from the FDA and the hope that the FDA Rare Pediatric Disease Priority Review Voucher program leads to expedited treatments for children who desperately need them.*

*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: we may never receive a priority review voucher as a result of the rare pediatric disease designation for eteplirsen or, if we do receive a voucher, we may not be able to use it to benefit any of our product candidates or transfer it for various reasons, such as the expiration or termination of the priority review voucher program or failure to obtain regulatory approval of eteplirsen; the FDA may determine that our NDA submission for eteplirsen is incomplete, does not qualify for filing or approval; an advisory committee 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 submission, clinical studies, our planned meetings and discussions with the FDA, and plans for commercializing eteplirsen and our 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.*

Source: Sarepta Therapeutics, Inc.

Media and Investors:

Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com

OR

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