Sarepta Therapeutics Announces Additional Long-Term Efficacy and Safety Data from Pivotal Phase IIb Program of Eteplirsen for Treatment of Duchenne Muscular Dystrophy

-- Eteplirsen provided a statistically significant 6 minute walk test advantage of 151 meters at three years compared to an external control --

-- Fourth muscle biopsy results confirm increased dystrophin production in nearly all eteplirsen-treated patients and exon skipping in 100 percent of patients --

-- Eteplirsen safety profile remains consistent with prior results --

Cambridge, Mass.--October 1, 2015--Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-targeted therapeutics, today announced additional clinical efficacy and safety data from the Company's Phase IIb program of eteplirsen in patients with Duchenne muscular dystrophy (DMD). The data demonstrated that eteplirsen provided a statistically significant advantage of 151 meters in the ability of study participants to walk at three years, compared with external controls. Further, the fourth biopsy data confirmed the mechanism of action of eteplirsen, demonstrating exon skipping in all patients and dystrophin production in nearly all patients. Safety data remained consistent with prior results.

Eteplirsen, Sarepta's lead drug candidate, is designed to target the underlying cause of DMD by enabling the production of a functional dystrophin protein in patients with mutations amenable to exon 51 skipping. Approximately 13 percent of people with DMD are estimated to have a mutation targeted by eteplirsen/exon 51 skipping.

"We are encouraged by the positive clinical outcomes, such as the statistically significant difference in the 6MWT in eteplirsen-treated patients compared to a control, especially since we see them accompanied by data that continues to demonstrate exon skipping and dystrophin production in most patients," said Edward Kaye, M.D., Sarepta's interim chief executive officer and chief medical officer. "We are committed to bringing eteplirsen and our other investigational exon skipping therapies to patients with DMD and will continue to work with all stakeholders to advance these programs as quickly as possible so we can better address the unmet need for treatments in the DMD community."

Results of Sarepta's Phase IIb program were included in the New Drug Application (NDA) that Sarepta submitted to the U.S. Food and Drug Administration (FDA) for eteplirsen for the treatment of DMD amenable to exon 51 skipping. The primary clinical endpoint in the NDA was the comparison of the 6MWT ITT analysis of the eteplirsen-treated group compared to an external control with similar inclusion criteria. The FDA granted eteplirsen Priority Review status and assigned a Prescription Drug User Fee Act (PDUFA) action date of February 26, 2016. Previously, the FDA granted Rare Pediatric Disease Designation to eteplirsen, as well Orphan Drug Designation and Fast Track Status.

New Long-Term Efficacy Data

- Patients who were treated with eteplirsen experienced a statistically significant 151 meter difference in the 6-minute walk test (6MWT) at three years compared with external DMD controls. The 6MWT is a well-accepted measure of ambulation and clinical function in patients with DMD. ($p<0.01$).
- Eteplirsen-treated patients had a lower rate of loss of ambulation than external DMD controls over three years.
- Eteplirsen-treated patients experienced a slower rate of decline through Week 192 than external DMD controls.
- Pulmonary function remained relatively stable through approximately four years in eteplirsen-treated patients.

New results from a fourth biopsy performed on 11 patients demonstrated that exon skipping occurred in 100 percent of patients after 180 weeks of treatment, confirming the mechanism of action of eteplirsen. In addition, biochemical evidence from three quantification methods, analysis of dystrophin positive fibers, dystrophin intensity and Western Blot testing, confirmed that dystrophin was present in most patients following eteplirsen treatment.

Fourth Biopsy Results

- Confirmed exon skipping in 100% of patients
- Percent dystrophin-positive fibers increased ($p<0.001$) in comparison to untreated controls
- Dystrophin intensity increased ($p<0.001$) in comparison to untreated controls
- Western Blot confirmed presence of dystrophin protein in 9 of 11 (82%) of eteplirsen-treated patients at Week 180 vs 1 of 9 (11%) in the DMD control biopsies

New Long-Term Safety Data

New results from Sarepta's safety database, which includes approximately 100 patients exposed to eteplirsen, showed that the eteplirsen safety profile remained consistent with prior results. Common

adverse drug reactions included flushing, erythema, and mild temperature elevation. No pulmonary embolisms, hospitalizations, injection site reactions or thrombocytopenia have been observed.

Webcast & Conference Call

Sarepta will provide a corporate update and report on recent data from the Phase IIb study of eteplirsen for Duchenne muscular dystrophy via a live webcast and conference call on October 1, 2015 at 7:00 AM EST. The update will be followed by a panel discussion with Duchenne muscular dystrophy experts Anne Connolly, MD; Eugenio Mercuri, MD, PhD; Jerry Mendell, MD; Perry Shieh, MD, PhD; and Steve Wilton, PhD, BSc.

The presentation will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

The conference call may be accessed by dialing 877-727-3245 for US domestic callers and 530-379-4673 for international callers. The passcode for the call is 48471076. Please specify to the operator that you would like to join the "Sarepta Corporate Update and Report on Recent Data."

About the 6-Minute Walk Test (6MWT)

The 6MWT was developed as an integrated assessment of cardiac, respiratory, circulatory, and muscular capacity for use in clinical trials of various cardiac and pulmonary conditions. In recent years, the 6MWT has been adapted to evaluate functional capacity in neuromuscular diseases and has served as the basis for regulatory approval of a number of drugs for rare diseases, with mean changes in the 6MWT ranging from 28 to 44 meters. Additionally, published data from longitudinal natural history studies assessing dystrophinopathy, a disease continuum comprised of DMD and Becker muscular dystrophy, support the utility of the 6MWT as a clinically meaningful endpoint in DMD. These data show that boys with DMD experience a significant decline in walking ability compared to healthy boys over one year, suggesting that slowing the loss of walking ability is a major treatment goal.

About Duchenne Muscular Dystrophy

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.

About Eteplirsen

Eteplirsen is designed to address the underlying cause of DMD by enabling the production of a functional dystrophin protein. Eteplirsen uses Sarepta's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene. This enables the repair of specific genetic mutations that affect approximately 13 percent of people with DMD. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from messenger RNA (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. Eteplirsen has not been approved by the FDA or any regulatory authority for the treatment of DMD.

Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable safety and tolerability profile and restoration of dystrophin protein expression.

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.

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 safety and efficacy of eteplirsen, analysis of eteplirsen and external control data and their implications, eteplirsen's potential as a treatment for Duchenne Muscular Dystrophy and its potential market size and Sarepta's commitment to bringing eteplirsen and its other exon skipping investigational therapies to patients with DMD and plans to continue working with all stakeholders to advance these programs as quickly as possible. 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 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 (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 and most recent Quarterly Report on Form 10-Q for the quarter ended June 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.

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