Sarepta Issues Statement on Advisory Committee Outcome for Use of Eteplirsen in the Treatment of Duchenne Muscular Dystrophy

CAMBRIDGE, Mass., April 25, 2016 – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-based therapeutics, today announced that the U.S. Food and Drug Administration's (FDA) Peripheral and Central Nervous System Advisory Committee (PCNSC) met to review the new drug application (NDA) for eteplirsen as a treatment for Duchenne muscular dystrophy amenable to exon 51 skipping. The advisory committee voted 6-7 against the finding of substantial evidence from adequate and well controlled studies that show that eteplirsen induces production of dystrophin to a level that is reasonably likely to predict clinical benefit (FDA Question #2). The advisory committee voted 3 – 7, with three abstentions, against finding substantial evidence based on the clinical results of the single historically controlled study (Study 201/202) that eteplirsen is effective for treatment of DMD (FDA Question #7).

"We would like to thank the hundreds of patients and families who participated in the discussion today, underscoring the critical unmet need of people living with Duchenne." said Edward Kaye, M.D., Sarepta's interim chief executive officer and chief medical officer. "We appreciated the opportunity to present our data to the advisory committee panel and will continue to work with FDA as they complete their review of the eteplirsen NDA. Today more than ever, we remain committed to our mission of bringing a treatment to the Duchenne community."

DMD is a rare degenerative neuromuscular disorder causing severe progressive muscle loss and premature death. The FDA is not bound by the Advisory Committee's recommendation but takes its advice into consideration when reviewing New Drug and Biologic License Applications in general. The Prescription Drug User Fee Act (PDUFA) action date for completion of FDA review of eteplirsen is May 26, 2016.

In three additional voting questions, the panel voted 5 to 7, with one abstention, against whether decisions to administer the 6-minute walk test (vs. conclusions that the patient could no longer walk) were sufficiently objective and free of bias and subjective decision-making by patients, their caregivers, and/or health care professionals to allow for a valid comparison between patients in Study201/202 and an external control group (FDA Question #4). The panel voted on the impact of the North Star Ambulatory Assessment with one panel member voting that it strengthened the persuasiveness of the findings in Study 201/202, with five voting that it weakened the persuasiveness, and seven voting that it had no effect (FDA Question #5). The panel also voted on the impact of the other tests of physical performance (e.g., rise time, 10-meter run/walk) on the persuasiveness of the findings in Study 201/202, with the result of one panel member voting that they strengthened the persuasiveness, two voting that they weakened the persuasiveness, and ten voting that they had no effect (FDA Question # 6).

The PCNSC Advisory Committee recommendation was based on a review of results from the Phase IIb clinical program for eteplirsen (studies 201 and 202), long-term outcomes (through 168 weeks) from the Study 202 open-label extension study, as well as 4-year clinical effectiveness data based on a comparison of patients in Study 201/202 to a historical control group that was designated as a major amendment to the NDA in January 2016.

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

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.

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 morpholine 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 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.

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 Company's plans to continue to work with the FDA as they complete their review of the eteplirsen NDA; the Company's commitment to its mission of bringing treatment to the Duchenne Community; eteplirsen's potential for approval as the first medicine to treat the underlying cause of DMD; that the FDA is not bound by the advisory committee recommendation but takes its advice in consideration when reviewing applications; 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 not provide eteplirsen with marketing approval by the applicable PDUFA date or at all; we may not be able to comply with all FDA requests, including with respect to our 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; 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 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 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 Reports on Form 10-Q 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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