**Sarepta Therapeutics Announces EMA Validation of Eteplirsen Authorization Application for Treatment of Duchenne Muscular Dystrophy Amenable to Exon Skipping 51**

CAMBRIDGE, Mass.--(BUSINESS WIRE)--December 19, 2016--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage developer of innovative RNA-targeted therapeutics, today announced that the European Medicines Agency (EMA) validated the previously submitted Marketing Authorization application (MAA) for eteplirsen to treat Duchenne muscular dystrophy amenable to exon 51 skipping. Sarepta is seeking conditional approval of eteplirsen in the EU through the centralized procedure. Validation of the MAA confirms that the submission is accepted and starts the formal review process by the EMA's Committee for Human Medicinal Products (CHMP). The standard review period is 210 days (plus additional time for applicant to respond to questions from the agency).

"Around the world, there are many patients living with DMD who do not have access to a medicine that treats the underlying cause of the disease," said Edward Kaye, Sarepta's chief executive officer. "The validation of the MAA is the next step toward our goal of providing potential therapies to more patients with Duchenne in Europe."

**About Eteplirsen**

Eteplirsen uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. Eteplirsen is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. Data from clinical studies of eteplirsen in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of eteplirsen has not been established.

**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 Sarepta Therapeutics**

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates. For more information, please visit us at www.sarepta.com.

**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 regarding Sarepta seeking conditional approval of eteplirsen in the EU, the validation starting the formal review process by the EMA’s CHMP, the standard 210 day review period, and that the validation of the MAA is the next step toward Sarepta’s goal of providing potential therapies to more patients with Duchenne in Europe.

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: the EMA may delay its decision beyond the standard MAA review or may determine that our MAA submission for eteplirsen does not qualify for approval; 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; the estimates regarding the market size for eteplirsen or any of our product candidates may not be correct; we may not be able to achieve or there may be delays in our projected regulatory and development timelines for eteplirsen including satisfying EMA requests related to our eteplirsen MAA filing, our clinical studies, our planned meetings and discussions with regulatory authorities, our plans for commercializing eteplirsen in the E.U., the development of our other product candidates for various reasons including possible limitations of Company financial and other resources and regulatory, court or agency decisions, such as decisions with respect to patents that cover our product candidates; factors that would negatively impact our manufacturing efforts; and those risks identified under the heading "Risk Factors" in Sarepta's 2015 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 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.

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“Safe Harbor” Statement under the Private Securities Litigation Reform Act of 1995: The statements that are not historical facts contained in this release are forward–looking statements that involve risks and uncertainties, including, but not limited to, the results of research and development efforts, the results of preclinical and clinical testing, the effect of regulation by the FDA and other agencies, the impact of competitive products, product development, commercialization and technological difficulties, and other risks detailed in the company’s Securities and Exchange Commission filings.