



As Anticipated, Sarepta Receives Negative CHMP Opinion for EXONDYS® (eteplirsen) to Treat Patients with Duchenne Muscular Dystrophy in Europe

-- Sarepta will seek re-examination of the opinion and request that a Scientific Advisory Group (SAG) be convened --

CAMBRIDGE, Mass., June 1, 2018 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), has adopted a negative opinion for EXONDYS® (eteplirsen), as expected. EXONDYS is designed to treat patients with Duchenne muscular dystrophy (DMD) amenable to skipping exon 51 of the dystrophin gene.

As previously reported on Sarepta’s May 5, 2018 earnings call, a negative opinion was anticipated following the oral explanation trend vote. Sarepta will request a re-examination of the opinion, which will result in the assignment of a new rapporteur and co-rapporteur. The Company will also request a Scientific Advisory Group (SAG) on DMD be called so that neuromuscular specialists, experienced with working with treatments for these patients, can provide expert guidance and insight into, among other things, the validity of the external controls used and the importance of certain functional endpoints, including for instance, the relevance of meaningful slowing pulmonary decline in patients with this difficult to treat disease. The re-examination process is expected to be completed by year-end 2018.

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 male births worldwide.

About EXONDYS® (eteplirsen)

EXONDYS® (eteplirsen) is being developed to treat individuals who have a specific type of Duchenne muscular dystrophy (DMD), comprising 13 percent of the overall population who have one of the mutations amenable to exon 51 skipping. If approved, it would be the first injectable therapy in Europe

designed to treat the underlying cause of DMD — the lack of a protein called dystrophin, which is critical for muscles to function properly.

EXONDYS utilizes Sarepta's precision RNA splicing technology to correct specific genetic mutations and produce a shortened, but functional, form of the dystrophin protein.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

Forward-Looking Statements

This press release contains "forward-looking statements." 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 Sarepta's plan to seek a re-examination of the opinion and request that a SAG be convened; and the expectation that the re-examination process will be completed by year-end 2018.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: we may not be granted a re-examination of our MAA for eteplirsen, a SAG may not be convened, and even if a re-examination and a related SAG are granted, the CHMP may render a negative opinion and we may not be able to obtain regulatory approval for eteplirsen from the European Medicines Agency; we may not be able to execute on our business plans, including meeting our expectations with respect to EXONDYS 51 sales, meeting our expected or planned regulatory milestones and timelines, research and clinical development plans, and bringing our product candidates to market, 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, and regulatory, court or agency decisions, such as decisions by the CHMP on eteplirsen or 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, 2017 and 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. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta’s 2017 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the SEC, as well as other SEC filings made by Sarepta. 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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