

Sarepta Therapeutics Announces Publication of Positive Long-Term Safety and Efficacy Data for Eteplirsen in the *Annals of Neurology*

Study evaluated disease progression during three years of eteplirsen treatment in patients with Duchenne muscular dystrophy amenable to exon 51 skipping

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 17, 2015 -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-based therapeutics, today announced that the *Annals of Neurology* published online positive efficacy and safety results from a Phase IIb long-term open-label extension study of eteplirsen in patients with Duchenne muscular dystrophy (DMD) amenable to exon 51 skipping. The study found that at three years of treatment, patients experienced a slower rate of disease progression when compared to untreated matched historical controls and the investigational drug continued to be well-tolerated.

This analysis used historical data from the Italian Telethon Network and the Leuven Neuromuscular Reference Group for comparative analysis of 6MWT performance at baseline and Months 12, 24, and 36. Patients were matched to the eteplirsen group based on age, corticosteroid use and genotype.

At 36 months, eteplirsen-treated patients demonstrated a statistically significant difference of 151 meters in six minute walk test (6MWT), compared to the external cohort. The eteplirsen-treated patients experienced a lower incidence of loss of ambulation (16.7%) compared to natural history control patients (46.2%).

“As a clinician with extensive experience treating patients with Duchenne muscular dystrophy, it is apparent based on these data, that the boys treated with eteplirsen in this study are showing more signs of stability than would normally be expected at this stage of the disease. Remaining ambulatory is critical to greater independence over a longer period of time and a 151 meter difference indicates a marked slowing of disease progression,” said Jerry Mendell, M.D. director of the Center for Gene Therapy and Muscular Dystrophy at Nationwide Children’s Hospital, Columbus, Ohio., and lead author of the publication. “The safety and tolerability demonstrated by eteplirsen is also important, as exon skipping therapies will require lifelong dosing to be effective. For over three years, it continues to be one of the safest drugs I have used in my career.”

“Over the last 10 years, there has been an effort to harmonize standards of care, which has resulted in published guidelines that have allowed our center to generate and publish more reliable natural history data for Duchenne muscular dystrophy,” said Eugenio Mercuri, Professor of Pediatric Neurology, Università Cattolica del Sacro Cuore, Rome, Italy. “The observations of the eteplirsen-treated boys in comparison to this matched natural history group shows an encouraging difference from what one would normally expect from the natural history of the disease.”

During the follow-up period of the Phase IIb study, the most common adverse events (AE) at 36 months included flushing, erythema, and mild temperature elevation. No pulmonary embolisms, hospitalizations, injection site reactions or thrombocytopenia have been observed.

“We are pleased to have these results, which indicate that eteplirsen-treated patients have a slower disease progression at three years of treatment, published in such a prestigious peer-reviewed journal,” said Edward Kaye, M.D., Sarepta’s interim chief executive officer and chief medical officer. “The use of natural history studies will become even more important for the development of drugs for rare exons, where there is currently no suitable regulatory pathway due to limited patient populations.”

Mendell JR, Goemans N, Lowes LP, Alfano LN, Berry K, Shao J, et al. Longitudinal effect of eteplirsen vs. historical control on ambulation in DMD. *Annals of Neurology*. 2015: Accepted Article, ahead of print. DOI: [10.1002/ana.24555](https://doi.org/10.1002/ana.24555).

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)

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 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 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 untreated matched historical controls data, the implications of this data and analysis including the significance of the 151 meter 6MWT difference for patient independence and slowing of disease progression, the possibility of eteplirsen being effective as a life-long therapy and the importance of natural history studies for the development of drugs for rare exons. 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. 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 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 or milestones and an advisory committee or the FDA may disagree with Sarepta's analysis of data relating to eteplirsen and matched natural history data; Sarepta may not succeed in executing its plans for commercializing eteplirsen and developing Sarepta's other product candidates for various reasons including possible limitations of Sarepta's financial and other resources, the impact of regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office 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; and those risks identified under the heading "Risk Factors" in Sarepta's 2014 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended September 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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