Sarepta Therapeutics to Present Additional 48-Week Data From the Phase IIb Study of Eteplirsen for the Treatment of Duchenne Muscular Dystrophy at the 17th Annual International World Muscle Society

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Oct 12, 2012 (Marketwire via COMTEX) --Sarepta Therapeutics (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, announced today that data from a Phase IIb study evaluating eteplirsen, an investigational treatment for boys with Duchenne muscular dystrophy (DMD), will be presented Saturday, October 13th at the World Muscle Society in Perth, Australia. Principal investigator, Jerry R. Mendell, M.D. of Nationwide Children's Hospital, will present the data in an oral presentation of the abstract titled, "Results at 48 Weeks of a Phase IIb Extension Study of the Exon-Skipping Drug Eteplirsen in Patients with Duchenne muscular dystrophy (DMD)." Dr. Mendell will present tomorrow from 2:30 to 4:00 p.m. WST UTC +8 hours/2:30 to 4:00 a.m. EDT.

The presentation will describe new and previously reported efficacy and safety data from the Phase IIb study examining 48 weeks of treatment with eteplirsen in boys with DMD. Results from the Phase IIb extension study confirmed that treatment with Sarepta's lead exon-skipping compound, eteplirsen, met the primary efficacy endpoint, increase in novel dystrophin, and achieved a significant clinical benefit on the primary clinical outcome, the 6-minute walk test (6MWT) over the placebo/delayed treatment cohort.

Additional data to be presented includes:

- Individual patient data on the primary endpoint of change in dystrophin-positive fibers from baseline;
- Additional biochemical findings including RT-PCR and western blot images from selected patients;
- Additional information on the two patients in the 30 mg/kg cohort who showed a rapidly progressive decline on the 6-minute walk test and were excluded from the analysis; and
- A summary of treatment-emergent adverse events comparing eteplirsen-treated patients versus placebo, which demonstrated that eteplirsen was well-tolerated through 48 weeks of treatment. No treatment-related adverse events, serious adverse events, or treatment discontinuations related to eteplirsen were observed. In addition, no treatment related changes were detected on any safety laboratory parameters, including several biomarkers for renal function.

Dr. Mendell's presentation will be posted on the Sarepta website in the "Events & Presentations" section after the session is completed.

About Study 201 and Study 202 (Phase IIb Eteplirsen Study)

Study 4658-US-201 was conducted at Nationwide Children's Hospital in Columbus, Ohio. Twelve boys meeting the inclusion criteria being between 7 and 13 years of age with appropriate deletions of the dystrophin gene that confirm eligibility for treatment with an exon-51 skipping drug, received double-blind IV infusions of placebo (n=4), 30 mg/kg of eteplirsen (n=4), or 50 mg/kg of eteplirsen once weekly for 24 weeks (n=4). Muscle biopsies for evaluation of dystrophin were obtained at baseline for all subjects, and after 12 weeks for patients in the 50 mg/kg cohort and after 24 weeks for patients in the 30 mg/kg cohort. Two placebo patients were randomized to the 30 mg/kg cohort and two placebo patients were randomized to the 50 mg/kg cohort. This study design allowed Sarepta to investigate the relationship of dose and duration of eteplirsen treatment on the production of dystrophin over the course of the 24-week study.

Study 4658-US-202 is the extension study to 201 and continues to assess the long-term safety and efficacy of open-label eteplirsen. The four placebo patients were rolled over to open-label eteplirsen at week 24, with six patients on 30 mgs/kg, and six patients on 50 mgs/kg. Third biopsies occurred at 48 weeks in the original study 201 treated patients, and at 24 weeks, the same time point, in the original placebo patients. 6MWT was performed at 32 weeks, 36 weeks, 48 weeks and will continue to be performed every 12 weeks going forward.

About Dystrophin

Dystrophin, a large structural protein, is critical to the stability of myofiber membranes in skeletal, diaphragmatic and cardiac muscle, protecting muscle fibers from contraction-induced damage. Loss of functional dystrophin destabilizes the dystroglycan protein complex, impairing its localization to the muscle membrane, and compromising the integrity of the membrane structure. The absence of functional dystrophin results in muscle membrane breakdown with muscle fibers being replaced by adipose and fibrotic tissue.

About the 6-Minute Walk Test

The 6-minute walk test (6MWT) was developed as an integrated assessment of cardiac, respiratory, circulatory, and muscular capacity (American Thoracic Society 2002) 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 (Rubin 2002, Wraith 2004, Muenzer 2006). 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 (McDonald C, et al, Muscle & Nerve, December 2010) 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 the Statistical Methodology

The Mixed Model Repeated Measures (MMRM) test was used for all statistical analyses of the 6MWT results, including for all subgroups. Analysis of Covariance (ANCOVA) for ranked data was used when the assumptions of normality of the dependent variable (the change in 6MWT distance from baseline) were violated. The inclusion of the two patients with extreme scores due to rapid progression in the ITT population (n=12) resulted in a violation of the normality assumptions of the Change from Baseline in 6MWT data, and thus required the use of ANCOVA for ranked data. The exclusion of these two patients from the mITT population (n=10) resulted in the 6MWT data becoming normally distributed and the MMRM statistics exhibiting much improved residuals and fit statistics as compared to the ITT population. As such, the estimated mean values and their associated p-values for the mITT population were slightly different from those for the ITT population.

About Duchenne Muscular Dystrophy and Eteplirsen

Duchenne muscular dystrophy (DMD) is an X-linked rare, degenerative neuromuscular disorder causing severe, progressive muscle loss and a premature death. One of the most common fatal genetic disorders, DMD affects approximately one in every 3,500 boys 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 eventually spreads to the arms, neck and other areas. Eventually, this progresses to complete paralysis and increasing difficulty in breathing due to respiratory muscle dysfunction requiring ventilatory support, as well as cardiac muscle dysfunction leading to heart failure. The condition is terminal, and death usually occurs before the age of 30.

Eteplirsen is Sarepta's lead drug candidate that is designed to address the underlying cause of DMD by enabling the production of a functional dystrophin protein. Data from clinical studies of eteplirsen in DMD patients have demonstrated a broadly favorable safety and tolerability profile and restoration of dystrophin protein expression. Eteplirsen uses Sarepta's novel phosphorodiamidate morpholino oligomer (PMO)-based chemistry and proprietary exon-skipping technology to skip exon 51 of the dystrophin gene enabling the repair of specific genetic mutations that affect approximately 13 percent of the total DMD population. By skipping exon 51, eteplirsen may restore the gene's ability to make a shorter, but still functional, form of dystrophin from messenger RNA, or mRNA. Promoting the synthesis of a truncated dystrophin protein is intended to improve, stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD.

Sarepta is also developing other PMO-based exon-skipping drug candidates intended to treat additional patients with DMD.

About Sarepta Therapeutics

Sarepta Therapeutics is focused on developing first-in-class RNA-based therapeutics to improve and save the lives of people affected by serious and life-threatening rare and infectious diseases. The Company's diverse pipeline includes its lead program eteplirsen, for Duchenne muscular dystrophy, as well as potential treatments for some of the world's most lethal infectious diseases. Sarepta aims to build a leading, independent biotech company dedicated to translating its RNA-based science into

transformational therapeutics for patients who face significant unmet medical needs. For more information, please visit us at www.sareptatherapeutics.com.

Forward-Looking Statements and Information

In order to provide Sarepta's investors with an understanding of its current results and future prospects, 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," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements about the development of eteplirsen and its efficacy, potency and utility in the treatment of DMD and the potential for the creation of novel dystrophin to lead to significant clinical benefit over a longer course of treatment.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: clinical trials may not demonstrate the safety and efficacy of eteplirsen and/or Sarepta's antisense-based technology platform; treatment of patients with DMD using eteplirsen over a longer duration may not lead to significant clinical benefit; and any of Sarepta's drug candidates, including eteplirsen, may fail in development, may not receive required regulatory approvals, or be delayed to a point where they do not become commercially viable.

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 Securities and Exchange Commission. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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