Sarepta Therapeutics Announces Publication of Eteplirsen Clinical Study Results in the Annals of Neurology

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Results Show a Significant Increase in Dystrophin Production and a Stabilization of Walking Ability in Duchenne Muscular Dystrophy Patients

CAMBRIDGE, MA -- (Marketwired) -- 08/08/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced the first peer-reviewed publication of the 48-week results from the Phase IIb clinical study of eteplirsen in the Annals of Neurology. Eteplirsen is an investigational medicine in development for the treatment of patients with Duchenne muscular dystrophy (DMD) who have a genotype amenable to skipping of exon 51.

Published study results showed that once-weekly treatment with eteplirsen resulted in a statistically significant increase from baseline in novel dystrophin, the protein that is lacking in patients with DMD. In addition, eteplirsen-treated patients evaluable on the 6-minute walk test (6MWT) demonstrated stabilization in walking ability compared to a placebo/delayed-treatment cohort. Eteplirsen was well tolerated in the study with no clinically significant treatment-related adverse events. These data will form the basis of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for eteplirsen planned for the first half of 2014.

"These unprecedented data for eteplirsen in DMD patients with genetic mutations correctable by skipping exon 51 represent a significant milestone in the scientific community's efforts to address the tremendous need for treatments for this devastating and deadly disease in children," said Jerry Mendell, M.D., director of the Centers for Gene Therapy and Muscular Dystrophy at Nationwide Children's Hospital, principal investigator of the Phase IIb study and lead author of the publication. "For the first time in this disease, we have a potentially disease-modifying treatment that has demonstrated strong evidence of a relationship between a biochemical effect on dystrophin production and a clinically meaningful outcome on the 6-minute walk test. In addition, we believe this technology can potentially be applied to target additional genetic mutations in DMD in the future."

DMD is a rare degenerative neuromuscular disorder that causes progressive muscle loss, leading to severe disability and premature death. It 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 leads to loss of ambulation, limitations in activities of daily living, and serious cardiac and respiratory dysfunction. The condition is universally fatal, and patients typically die from complications of the disease in their twenties.

"The peer-reviewed publication of these data is an important achievement as we continue to advance eteplirsen through late-stage clinical development with the goal of bringing to patients and their treating physicians a safe and effective therapy that addresses the underlying cause of this devastating disease," said Edward Kaye, M.D., senior vice president and chief medical officer of Sarepta Therapeutics and a co-author of the publication. "The results published today provide the foundation of efficacy and safety data that we continue to build upon with our ongoing study of eteplirsen in DMD patients, with consistent results now seen through 84 weeks of treatment. We would like to thank all of the patients, families, physicians and healthcare providers who have diligently worked on the successful execution of this important clinical study of eteplirsen."

Summary of the Phase IIb Study and Key Results Through Week 48

The safety and efficacy of eteplirsen were evaluated in a 24-week randomized, double-blind, placebo-controlled study (Study 201). The study enrolled twelve boys aged seven to 13 years with a confirmed genotype amenable to treatment with an exon-51 skipping drug. These patients were randomized to one of three treatment arms including placebo (n=4), eteplirsen 30 mg/kg (n=4) and eteplirsen 50 mg/kg (n=4), and received eteplirsen or placebo weekly by intravenous infusion. After 24 weeks, all placebo-treated patients initiated weekly eteplirsen treatment at 30 mg/kg (n=2) or 50 mg/kg (n=2). After Week 28, all patients were rolled over into a long-term open-label extension study (Study 202), which continues to follow patients on clinical and safety measures with data reported through 84 weeks.

Dystrophin Production: The primary efficacy endpoint in Study 201 and Study 202 was the change from baseline in the percent of dystrophin-positive fibers present in muscle biopsies. Pre-treatment muscle biopsies were collected from all patients in the study. To evaluate the effect of eteplirsen dose and treatment duration on dystrophin production, a second biopsy was collected at week 12 from the four patients in the 50 mg/kg cohort and two placebo-treated patients, and at Week 24 from the

four patients in the 30 mg/kg cohort and two placebo-treated patients. A third biopsy was collected in all patients at Week 48.

After 48 weeks of treatment, eteplirsen administered at either 30 mg/kg or 50 mg/kg for 48 weeks (n=8) resulted in a statistically significant increase ($p \leq 0.001$) in dystrophin-positive fibers to 47.3 percent of normal. The placebo/delayed-treatment cohort, which had received 24 weeks of eteplirsen at either 30 mg/kg or 50 mg/kg following 24 weeks of placebo (n=4), also showed a statistically significant increase in dystrophin positive fibers to 37.7 percent of normal ($p \leq 0.008$).

Results from the study suggested that at least 12 weeks of treatment was needed to observe increases in dystrophin production in muscle biopsies. In addition, there was no meaningful difference in dystrophin production between the 30 mg/kg and 50 mg/kg dose arms at 48 weeks.

Walking Ability: A key secondary endpoint and the study's principal clinical outcome measure was the 6-minute walk test, a standard and well-accepted measure of walking ability and clinical function in DMD.

After 48 weeks, patients in the 30 mg/kg and 50 mg/kg dose cohorts who were able to perform the 6MWT (modified Intent-to-Treat or mITT population; n=6) showed a statistically significant treatment benefit of 67.3 meters ($p \leq 0.001$) when compared to the placebo/delayed-treatment cohort (n=4). This difference exceeds the 28 to 44 meter treatment effect reported in clinical studies that have served as a basis for the FDA approval of treatments for other neuromuscular disorders.

The mITT population used in the 6MWT analyses consisted of 10 of the 12 enrolled patients, including four patients in the 50 mg/kg cohort, two patients in the 30 mg/kg cohort and four patients in the placebo/delayed-treatment cohort. Two patients in the 30 mg/kg cohort showed rapid disease progression upon enrollment and lost ambulation by week 24, and thus were excluded.

Safety: Through 48 weeks, eteplirsen was well tolerated and there were no clinically significant treatment-related adverse events, no serious adverse events, hospitalizations or discontinuations.

About the Phase IIb Eteplirsen Program (Studies 201 and 202)

Study 201 was a randomized, double-blind, placebo-controlled clinical study initiated at Nationwide Children's Hospital in Columbus, Ohio. Twelve boys aged 7 to 13 years with a confirmed genotype amenable to treatment with an exon-51 skipping drug were randomized to one of three cohorts: 30 mg/kg (n=4), 50 mg/kg (n=4), and placebo/delayed treatment (n=4). Eteplirsen and placebo were administered weekly by intravenous infusion.

At Week 25, all patients rolled over to Study 202, a long-term open-label extension study, and placebo-treated patients initiated eteplirsen treatment at 30 mg/kg (n=2) or 50 mg/kg (n=2).

The primary efficacy endpoint in Study 201 and Study 202 was the increase in novel dystrophin as assessed by muscle biopsy at Weeks 12 and 24 and at Week 48, respectively. The primary clinical endpoint was the 6MWT, a well-accepted measure of ambulation and clinical function in DMD. Long-term follow up in Study 202 continues to evaluate safety and clinical outcomes including the 6MWT.

About the 6-Minute Walk Test (6MWT)

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 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 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 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 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 patients typically die from complications of the disease in their twenties.

About Sarepta's Proprietary Exon-Skipping Platform Technology

Eteplirsen is Sarepta's lead drug candidate and 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 stabilize or significantly slow the disease process and prolong and improve the quality of life for patients with DMD.

Eteplirsen has been granted orphan drug designation by the FDA and European Medicines Agency.

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.sarepta.com.

Forward-Looking Statements and Information

This press release contains forward-looking statements. These forward-looking statements generally can be identified by use of words such as "believes or belief," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," "advance" and similar expressions. These forward-looking statements include statements about the development of eteplirsen and its efficacy, potency and utility as a potential treatment for DMD and the potential for the creation of novel dystrophin to lead to significant clinical benefit over a longer course of treatment, the sufficiency of the Week 48 data as a basis for an NDA filing with the FDA and our ability to provide the FDA with the additional information and data that the FDA deems necessary for their regulatory determinations regarding eteplirsen.

Each forward-looking statement contained in this press release is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statement. Applicable risks and uncertainties include, among others: subsequent clinical trials may fail to demonstrate the safety and efficacy of eteplirsen or replicate results; treatment of patients with DMD using eteplirsen over a longer duration may not lead to significant clinical benefit; any of Sarepta's drug candidates, including eteplirsen, may fail in development, may not receive required regulatory approvals (including Subpart H accelerated approval), or may not become commercially viable due to delays or other reasons; and those identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the quarter ended June 30, 2013 and other information Sarepta files with the Securities and Exchange Commission.

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. 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.

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