Sarepta Therapeutics Announces Eteplirsen Demonstrates Continued Stability on Walking Test through 120 Weeks in Phase IIb Study in Duchenne Muscular Dystrophy

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Company to present today at the 32nd Annual J.P. Morgan Healthcare Conference

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 15, 2014-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced data through Week 120 from Study 202, a Phase IIb open-label extension study of eteplirsen in patients with Duchenne muscular dystrophy (DMD). Results through more than two years showed a continued stabilization of walking ability in eteplirsen-treated patients evaluable on the 6-minute walk test (6MWT). As previously reported, Study 202 met its primary endpoint of increased novel dystrophin as assessed by muscle biopsy at Week 48 and is now in the long-term extension phase in which patients continue to be followed for safety and clinical outcomes.

At 120 weeks, patients in the 30 mg/kg and 50 mg/kg eteplirsen cohorts who were able to perform the 6MWT (modified Intent-to-Treat or mITT population; n=6) experienced a decline of 13.9 meters, or less than 5 percent, from baseline in walking ability. A statistically significant treatment benefit of 64.9 meters ($p \leq 0.006$) was observed for the mITT population compared with the placebo/delayed-treatment cohort (n=4), which initiated treatment at Week 25 following 24 weeks of placebo. After experiencing a substantial decline earlier in the study, the placebo/delayed-treatment cohort also demonstrated stabilization in walking ability for more than 1.5 years, from Week 36 through 120, the period from which meaningful levels of dystrophin were likely produced, with a decline of 9.5 meters over this timeframe. These analyses were based on the maximum 6MWT score when the test was performed on two consecutive days.

“We now have more than two years of data with eteplirsen on the 6-minute walk test, the most accepted clinical outcome measure in Duchenne muscular dystrophy, which demonstrates walking stability that we believe would not be expected based on the natural history of this disease over the same time period,” said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. “We also now have over two years of safety data with eteplirsen with no treatment-related serious adverse events which is important when considering the need for lifelong treatment of this disease.”

Through 120 weeks, eteplirsen was well tolerated and there were no reported clinically significant treatment-related adverse events and no treatment-related serious adverse events. In addition, there were no treatment-related hospitalizations or discontinuations.

Summary of Additional 6MWT Analyses

Patients performed two 6MWT evaluations on consecutive days at time points coinciding with a muscle biopsy procedure at baseline and Weeks 12, 24 and 48. Two 6MWT evaluations were also performed at Week 120, and will be performed at all future functional assessment visits. All other evaluations were a single 6MWT. The pre-specified primary analysis included the maximum distance walked at those clinic visits where repeated tests were taken. Other analyses of the repeated 6MWT results assessed mean, minimum, and Day 1 (first measure) scores. Results from these additional 6MWT analyses confirm the treatment effect observed in the primary analysis.

Summary of 6MWT: Week 120 Treatment Results*

Analysis of Repeated 6MWT Values†	Baseline 6MWT (meters)	Adjusted Mean 6MWT Change from Baseline (meters) at 120 Weeks	Estimated Treatment Benefit (Eteplirsen Minus Placebo/delayed-Tx)	P-Value
Maximum Score Eteplirsen (n=6)	399.7	-13.9	64.9	0.006
Maximum Score Placebo/delayed-Tx (n=4)	394.5	-78.8		

Mean Score Eteplirsen (n=6)	388.6	-9.8	58.0	0.016
Mean Score Placebo/delayed-Tx (n=4)	380.3	-67.8		
Minimum Score Eteplirsen (n=6)	377.5	-5.7	51.0	0.042
Minimum Score Placebo/delayed-Tx (n=4)	366.0	-56.7		
Day 1 Score Eteplirsen (n=6)	379.7	+3.6	59.4	0.021
Day 1 Score Placebo/delayed-Tx (n=4)	371.5	-55.8		

** All 6MWT analyses are based on a Mixed Model Repeated Measures test.*

† All 6MWT analyses include the mITT population

‡ The pre-specified primary analysis of the 6MWT results was based on the maximum score.

Mr. Garabedian will present these data today at the 32nd Annual J.P. Morgan Healthcare Conference at 4:30 p.m. PST (7:30 p.m. EST) in San Francisco, California. The presentation will be webcast live under the investor relations section of Sarepta's website at www.sarepta.com and will be archived there for 90 days. Please connect to Sarepta's website several minutes prior to the start of the broadcast to ensure adequate time for any software download that may be necessary.

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

Study 201 was a randomized, double-blind, placebo-controlled clinical study conducted 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 the Statistical Methodology and the Modified Intent-to-Treat (mITT) Population

The Mixed Model Repeated Measures (MMRM) test was used for all statistical analyses of the 6MWT results. Baseline 6MWT scores and duration since DMD diagnosis were included as covariates.

The mITT population used in the 6MWT analyses consisted of 10 of the 12 enrolled patients, including 4 patients in the 50 mg/kg cohort, 2 patients in the 30 mg/kg cohort and 4 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 since they were no longer evaluable for the 6MWT. All other data were analyzed for all 12 patients.

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 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 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.

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, the potential for the creation of ongoing novel dystrophin and its ability to lead to significant clinical benefit, including as measured by the 6MWT and exploratory measures, over a longer course of treatment.

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, including as measured by the 6MWT and exploratory measures; 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 during projected time frames or at all due to delays or other reasons; and those identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the full year ended December 31, 2012 and as updated by our 2013 third quarter 10-Q, and filed with the Securities and Exchange Commission (SEC).

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.

Source: Sarepta Therapeutics, Inc.

Sarepta Investor Contact:
Erin Cox, 857-242-3714
ecox@sarepta.com
or
Sarepta Media Contact:
Jim Baker, 857-242-3710
jbaker@sarepta.com