Sarepta Therapeutics Announces Presentations at the 2017 MDA Scientific Conference

3/17/17

CAMBRIDGE, Mass., March 17, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases, today provided an update to investors that it will present data from eteplirsen studies at the MDA Scientific Conference in Arlington, Virginia on March 19-22. Sarepta is scheduled to give an oral, as well as poster presentation, on the cardiac function of eteplirsen-treated patients; a poster presentation on non-ambulatory patients; and a poster presentation on the pulmonary function of eteplirsen-treated patients.

Details of Sarepta's presentations at the MDA Scientific Conference are as follows:

Oral Presentation and Poster:
Title: Effects of Long-Term Treatment with Eteplirsen on Cardiac Function: Left Ventricular Ejection Fraction in Eteplirsen-Treated Patients vs Disease Natural History
Date and Time: Wednesday, March 22, 10:45-11:00am ET

Posters:
Title: Effects of Long-Term Treatment with Eteplirsen on Cardiac Function: Left Ventricular Ejection Fraction in Eteplirsen-Treated Patients vs Disease Natural History

Title: Long-Term Treatment with Eteplirsen in Non-ambulatory Patients: A Case Study in Identical Twins

Title: Effects of Long-Term Treatment With Eteplirsen on Pulmonary Function in Patients With Duchenne Muscular Dystrophy: Findings of Two Phase 2 Clinical Trials

About EXONDYS 51™

EXONDYS 51 uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. EXONDYS 51 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally truncated dystrophin protein. Data from clinical studies of EXONDYS 51 in a small number of DMD patients have demonstrated a consistent safety and tolerability profile. The pivotal trials were not designed to evaluate long-term safety and a clinical benefit of EXONDYS 51 has not been established.

Important Safety Information

- Adverse reactions in DMD patients (N=8) treated with 30 or 50 mg/kg/week of EXONDYS 51 with incidence of at least 25% more than placebo (N=4) (Study 1) were: balance disorder (38%), vomiting (38%) and contact dermatitis (25%). The most common adverse reactions were balance disorder and vomiting. Because of the small numbers of patients, these represent crude frequencies that may not reflect the frequencies observed in practice. The 50 mg/kg once weekly dosing regimen of EXONDYS 51 is not recommended.

- In the 88 patients who received ≥30 mg/kg/week of EXONDYS 51 for up to 208 weeks in clinical studies, the following events were reported in ≥10% of patients and occurred more frequently than on the same dose in Study 1: vomiting, contusion, excoriation, arthralgia, rash, catheter site pain, and upper respiratory tract infection.

- There have been reports of transient erythema, facial flushing, and elevated temperature occurring on the day of EXONDYS 51 infusion.

Please see the EXONDYS 51 (eteplirsen) U.S. Full Prescribing Information at www.EXONDYS51.com.

About Sarepta Therapeutics
Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates. For more information, please visit us at www.sarepta.com.

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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