Sarepta Therapeutics to Present at the 22nd International Annual Congress of the World Muscle Society

CAMBRIDGE, Mass., September 28, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases, will present a total of six posters at the 22nd International Annual Congress of the World Muscle Society (WMS), including a late-breaking poster presentation titled*, SRP-4053 Induces Exon Skipping Leading to Sarcolemmal Dystrophin Expression in Duchenne Muscular Dystrophy Patients Amenable to Exon 53 Skipping,* on Saturday, October 7, 2017 from 12:30-13:30 CEST by Francesco Muntoni, M.D., principal investigator for the 4053-101 study. The 4053-101 study was supported by the European Community FP7-HEALTH-2012-Grant 305370, SKIP-NMD, and by Sarepta Therapeutics. The International Congress of the World Muscle Society will take place October 3-7, 2017 at the Palais du Grand Large in Saint Malo, France.

Dr. Muntoni is a Pediatric Neurologist, Great Ormond Street Hospital for Children NHS Foundation Trust and the UCL Great Ormond Street Institute of Child Health. He also serves as Director of the Dubowitz Neuromuscular Center, a leading clinical and research institution for children affected by neuromuscular disorders, and Deputy Director for the MRC Neuromuscular Translational Research Centre at University College London.

Details of Sarepta's other four poster presentations and its joint poster with Catabasis Pharmaceuticals, Inc. are as follows:

Title: Optimization and Implementation of Best Practices for Collection and Preparation of Muscle Biopsies for Analysis During Clinical Trials of Neuromuscular Disease Therapeutics

Date and Time: Thursday, October 5, 2017; 17:00-18:30 CEST

Title: Edasalonexent (CAT-1004), an NF-kB Inhibitor, Enhances Myotube Formation In Vitro, and Increases Exon-Skipped Sarcolemmal Dystrophin in Muscle of Mdx Mice

Date and Time: Thursday, October 5, 2017; 17:00-18:30 CEST

Title: Development of Novel Observer-Reported Outcome Assessments in Clinical Trials of Patients with Duchenne Muscular Dystrophy

Date and Time: Thursday, October 5, 2017; 17:00-18:30 CEST

Title: Effects of Long-Term Treatment with Eteplirsen on Cardiac Function

Date and Time: Wednesday, October 4, 2017; 14:30-16:00 CEST

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

Date and Time: Thursday, October 5, 2017; 17:00-18:30 CEST

About Eteplirsen

Eteplirsen uses Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. Eteplirsen 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 eteplirsen 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 eteplirsen has not been established.

Important Safety Information About Eteplirsen

Adverse reactions in DMD patients (N=8) treated with eteplirsen 30 or 50 mg/kg/week by intravenous (IV) infusion with an incidence of at least 25% more than placebo (N=4) (Study 1, 24 weeks) were (eteplirsen, placebo): balance disorder (38%, 0%), vomiting (38%, 0%) and contact dermatitis (25%, 0%). 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 eteplirsen is not recommended.

In the 88 patients who received ≥30 mg/kg/week of eteplirsen 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 eteplirsen infusion.

For further information, please see the full Prescribing Information.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

Forward-Looking Statements

This press release contains "forward-looking statements". 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 Sarepta and Professor Muntoni presenting at the 22nd International Annual Congress of the World Muscle Society and SRP-4053 inducing exon skipping that leads to Sarcolemmal Dystrophin Expression in DMD patients amenable to Exon 53 skipping.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: Sarepta and/or Professor Muntoni may not be able to present at the 22nd International Annual Congress of the World Muscle Society due to reasons outside of their control; the results of Sarepta's ongoing research and development efforts and clinical trials for its product candidates, including SRP-4053, may not be positive or consistent with prior results or demonstrate a safe treatment benefit; there may be delays in Sarepta's projected development or regulatory timelines for its product candidates for various reasons, some of which may be outside of Sarepta's control, including regulatory, court or agency decisions, 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 most recent Annual Report on Form 10-K for the year ended December 31, 2016 and Sarepta's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta which you are encouraged to review.

Any of the foregoing risks could 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 Sarepta's 2016 Annual Report on Form 10-K and most recent Quarterly Report

on Form 10-Q for the quarter ended June 30, 2017 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. 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 web-site at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Source: Sarepta Therapeutics, Inc.

Media and Investors:
Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com
or
W2O Group
Brian Reid, 212-257-6725
breid@w2ogroup.com