Sarepta Therapeutics Announces First Patient Dosed in Phase III Clinical Trial of SRP-4045 and SRP-4053 for the Treatment of Duchenne Muscular Dystrophy Amenable to Exon 45 or 53 Skipping

CAMBRIDGE, Mass.--(BUSINESS WIRE)—September 28, 2016--Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a developer of innovative RNA-targeted therapeutics, today announced the first patient dosed in the phase III clinical trial of SRP-4045 and SRP-4053 in patients with Duchenne muscular dystrophy amenable to exon 45 or 53 skipping.

“We are excited to announce the first patient dosed in our ESSENCE trial of SRP-4045 and SRP-4053, for DMD patients amenable to Exon 45 and 53 skipping “ said Edward Kaye, Sarepta’s chief executive officer. “It is our goal to treat as many patients amenable to exon skipping as possible, and therefore is important to advance our clinical pipeline candidates. We have made great effort to develop a meaningful clinical trial, using our learnings from our previous clinical programs and observations of the natural history of Duchenne.”

The Phase III study, ESSENCE, is a double-blind, placebo-controlled, multi-center study to evaluate the efficacy and safety of SRP-4045 and SRP-4053. Eligible patients with out-of-frame deletion mutations amenable to exon 45 or 53 skipping will be randomized to receive once weekly intravenous (IV) infusions of 30 mg/kg SRP-4045 or 30 mg/kg SRP-4053 respectively (combined-active group, 66 patients) or placebo (33 patients) for up to 96 weeks (the placebo-controlled period of the trial). This will be followed by an open label extension period in which all patients will receive open-label active treatment for up to 96 weeks.

The study will enroll approximately 99 patients aged 7 to 13 years, inclusive, with a minimum target of 45 patients amenable to exon 45 skipping and 45 patients amenable to exon 53 skipping.

Twice as many patients will receive active treatment as will receive placebo. Approximately 66 patients will be randomized to receive active treatment with either SRP-4045 or SRP-4053 (depending on deletion mutation), and 33 patients will be randomized to receive placebo.

More information can be found at www.Sarepta.com or www.clinicaltrials.gov, identifier: NCT02500381

About Duchenne Muscular Dystrophy (DMD)
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 3,500-5,000 males 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 SRP-4045 and SRP-4053
SRP-4045 and SRP-4053 are designed to address the underlying cause of DMD by restoring the messenger RNA (mRNA) reading frame, thus enabling the production of a shorter form of the dystrophin protein. SRP-4045 and SRP-4053 use Sarepta's proprietary phosphorodiamidate morpholino oligomer (PMO) chemistry and exon-skipping technology to skip exons 45 and 53, respectively, of the dystrophin gene. Promoting the synthesis of a shorter dystrophin protein is intended to slow the decline of ambulation and mobility seen in DMD patients. There currently is no approved treatment in the United States for DMD and SRP-4045 and SRP-4053 have not been approved by the FDA or any regulatory authority for the treatment of DMD.

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, including EXONDYS 51, designed to skip exon 51 and approved under the accelerated approval pathway. For more information, please visit us at www.sarepta.com.

Forward Looking Statements
This press release contains forward-looking statements. These forward-looking statements generally can be identified by the 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 ESSENCE study for product candidates SRP-4045 and SRP-4053 in DMD patients amenable to exon 45 and exon 53 exon skipping, respectively, advancing Sarepta's pipeline with clinical studies such as ESSENCE, Sarepta's goal of treating as many patients amenable to exon-skipping as possible, ESSENCE being a meaningful clinical trial, and the ESSENCE study design.

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: there may be delays in the ESSENCE study timelines, Sarepta may not be able to successfully complete the ESSENCE study for various reasons, including the possibility that the data does not support safety or efficacy of SRP-4045 SRP-4053; SRP-4045 and SRP-4053 could fail in development or may never receive regulatory approvals required for commercialization as therapeutics for DMD patients with mutations amenable to skipping of exon 45 and 53, respectively, and those risks identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the quarter ended June 30, 2016 and Sarepta's Annual Report on Form 10-K for the year ended December 31, 2015 filed with the Securities and Exchange Commission (SEC), and Sarepta's other filings with the SEC.

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.

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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