Sarepta Therapeutics Announces First Patient Dosed in European Phase I/II Study of SRP-4053 in Duchenne Muscular Dystrophy Patients

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 14, 2015--Sarepta Therapeutics Inc. (NASDAQ: SRPT), a developer of RNA-based therapeutics, today announced that it has initiated dosing of SRP-4053 in its first human trial, a Phase I/II study in Duchenne muscular dystrophy (DMD). This multiple-dose study will assess the safety, tolerability, efficacy, and pharmacokinetics of SRP-4053 in DMD patients with genotypes amenable to exon-53 skipping.

Professor Francesco Muntoni, the Chief Investigator and SKIP-NMD Project Coordinator from UCL Institute of Child Health & Great Ormond Street Hospital in London said, "It is certainly pleasing to see that the long-term collaboration with our European partners from London, Paris, Newcastle, and Rome – and with Sarepta in the U.S- – has come to fruition with the dosing of the first patient recruited into the dose escalation part of the study. This project, which started two years ago, enters now into the in-patient phase in which we aim to demonstrate safety of this new antisense oligonucleotide, before proceeding to the subsequent maintenance phase in which exploratory efficacy will also be determined using a variety of techniques, including novel and innovative outcome measures."

The study will be conducted at four sites in Europe under a consortium agreement between Sarepta and various European hospitals, institutions, and scientists established to conduct the study and which is funded in part by the European Union's Seventh Programme for research, technological development and demonstration under grant agreement No. 305370.

"We are excited to add a second exon-skipping drug to our clinical development pipeline," said Edward Kaye, M.D., Sarepta's Chief Medical Officer. "With the support of the SKIP-NMD partners, this marks a significant milestone in the expansion of our DMD program and a major step toward our goal of providing treatments to those children with Duchenne who may benefit from our exon-skipping technology."

"Recruitment of patients into this and other clinical trials is made possible through collaboration with the French network of clinicians who have identified and followed up in a natural history with all patients theoretically treatable by skipping exon 53," Laurent Servais, M.D., of the Institut de Myologie added. "We are happy that the first patient has received this new drug, to induce skipping of exon 53, here in Paris."

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. Sarepta'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.

About Skip-NMD

SKIP-NMD is an EU FP7 funded collaborative grant involving 10 partners from Europe and the U.S., whose aim is to restore dystrophin production in a subset of DMD boys. This will be achieved by developing a drug which 'skips' the mutations causing DMD, so as to restore dystrophin protein expression. The SKIP-NMD partners include University College London, Great Ormond Street Hospital for Children, Royal Holloway-University of London, Charles River Laboratories, Università Cattolica del Sacro Cuore, Institute of Myology, Sysnav, Consultants for Research in Imaging and Spectroscopy, Newcastle University, and Sarepta Therapeutics.

Forward-Looking Statement

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 study of SRP-4053, including what will be assessed in the study, the location and number of sites at which the study will be conducted, the determinations of the safety and efficacy of SRP-4053 and its potential as a therapeutic; the expansion of Sarepta's pipeline and its goal of providing treatments to those children with Duchenne who may benefit from exon-skipping technology.

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 study timelines or Sarepta, with its consortium partners, may not be able to successfully complete the study at planned sites for various reasons, including the possibility that the data does not support safety or efficacy of SRP-4053; SRP-4053 or any of Sarepta's product candidates could fail in development or may never receive regulatory approvals required for commercialization as therapeutics for Duchenne patients with mutations amenable to exon-skipping, and those risks identified under the heading "Risk Factors" in Sarepta's Quarterly Report on Form 10-Q for the quarter ended September 30, 2014 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 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.

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