Sarepta Therapeutics Announces First Patient Dosed in Study of Eteplirsen in Non-Ambulant Patients with Duchenne Muscular Dystrophy

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 12, 2014-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of RNA-based therapeutics, today announced that it has initiated dosing in a clinical study of eteplirsen, the Company's lead exon-skipping therapeutic candidate for the treatment of Duchenne muscular dystrophy (DMD), in patients who are non-ambulant or who have advanced DMD and don't meet a minimum 6-minute walk test score at baseline.

The open-label study, 4658-204 (Study 204), will include approximately 20 patients treated with eteplirsen who have genotypes amenable to exon 51 skipping and who meet other study inclusion criteria. The study will be conducted at several sites in the United States and is designed to evaluate the safety of eteplirsen in DMD patients over 96 weeks of dosing. Patients enrolled in the study will receive once weekly intravenous infusions of 30mg/kg of eteplirsen, and data will be collected across a number of safety parameters and secondary efficacy endpoints.

“The initiation of this eteplirsen study represents an important milestone for patients, their families, and the DMD community,” said Edward Kaye, M.D., Sarepta’s Chief Medical Officer. “Expanding the DMD population to include patients who are older and non-ambulant demonstrates our strong commitment to develop eteplirsen for patients at all stages of DMD and will provide additional data to support our planned NDA filing.”

Fawn Leigh, M.D., of Mass General Hospital and a principal investigator in the study added, "Eteplirsen is a potential breakthrough treatment for patients with DMD. I am pleased to be able to offer this promising disease-modifying treatment to my patients and to potentially alter the course of this devastating disease.”

About Eteplirsen

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. 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 atwww.sarepta.com.

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 Sarepta’s Study 204, including the type and number of patients participating in the Study, dosing of eteplirsen, and expected number of sites; Sarepta’s strong

commitment to develop eteplirsen for all stages of DMD; the inclusion of data to support Sarepta's planned New Drug Application (NDA) submission for eteplirsen; and the potential of eteplirsen as a breakthrough and disease modifying treatment for all patients with DMD that may alter the course of the disease.

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 and we may not be able to successfully complete Study 204 for various reasons, including any negative or inconsistent safety and efficacy data; Study 204 data and results may not provide support for an eteplirsen NDA filing; we may not be able to obtain regulatory approvals required for commercialization of eteplirsen and those 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.

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.

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.

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