



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

CAMBRIDGE, Mass. --(BUSINESS WIRE)-- Nov. 18, 2014 – Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of RNA-based therapeutics today announced that it has initiated dosing in a confirmatory study of eteplirsen, the Company's lead exon-skipping therapeutic candidate for the treatment of Duchenne muscular dystrophy (DMD), in ambulatory patients who meet specific criteria on their baseline 6-minute walk test score.

This open-label study, 4658-301 (PROMOVI), designed to evaluate the efficacy and safety of eteplirsen in DMD patients over 48 weeks of dosing, will be conducted at approximately 39 sites across the United States and include up to 160 patients. The study will enroll 60-80 boys, aged 7-16 years with genotypes amenable to exon 51 skipping who will be treated with eteplirsen, while an additional 60-80 patients with genotypes not amenable to exon 51 skipping will serve as a concurrent control group. Patients enrolled in the treatment group of the study will receive once weekly intravenous infusions of 30 mg/kg of eteplirsen. Data will be collected across a number of efficacy and safety parameters, including dystrophin assessments and will be powered to show a benefit on the 6-minute walk test compared with the untreated DMD control group.

"This confirmatory study with eteplirsen in ambulatory patients is an important step in the process of confirming the promising clinical results we achieved in our previous studies 201/202," said Edward Kaye, M.D., Sarepta's Chief Medical Officer. "By evaluating a larger population of boys and examining both functional and biochemical endpoints, we expect to further support the evidence of eteplirsen's potential benefit to patients."

Susan Apkon, M.D., Principal Investigator at Seattle Children's Hospital added, "The commencement of this study is a significant achievement for the DMD community. Eteplirsen shows great potential as an important therapeutic, and I am delighted to be a part of this larger

confirmatory study to further understand the impact that eteplirsen may have on families dealing with this terrible 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 at www.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 301, including the length of the study, type and number of patients expected to participate in the Study, dosing of eteplirsen, and expected

number of sites; the confirmation of prior clinical results and the ability to further develop evidence of eteplirsen benefit to patients; and the promise and potential of eteplirsen as a therapeutic.

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 301 for various reasons, including any negative or inconsistent safety and efficacy data; 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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