Sarepta Therapeutics Enters Into Clinical Trial Agreement With the National Institutes of Health for Further Development of Influenza Drug

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Dec 21, 2012 (Marketwire via COMTEX) --Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced that the Company has entered into a Clinical Trial Agreement (CTA) with the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health (NIH), to conduct a Phase I study with AVI-7100, the Company's lead drug candidate with a novel mechanism of action and potentially broad-spectrum activity against influenza viruses, including Tamiflu-resistant virus strains.

"We are very excited and honored to work with the NIH, which has a long-standing commitment to fighting pandemic and seasonal influenza," said Chris Garabedian, President and CEO. "Our agreement signifies the importance of developing new treatment options for influenza with novel mechanisms of action given the limitations of currently available influenza antivirals. Our combined effort with the NIH will further increase our understanding of AVI-7100 and its underlying platform chemistry, which we are also applying to other infectious disease targets."

The agreement establishes a formal collaboration between NIAID and Sarepta to allow NIAID researchers to proceed with a Phase I, double-blind, placebo-controlled, dose-escalating study to assess the safety, tolerability and pharmacokinetics of single and multiple doses of an intravenous formulation of AVI-7100 in healthy volunteers. The trial is being conducted at the NIH Clinical Center in Bethesda, MD, under the direction of Richard Davey, M.D., of NIAID's Division of Intramural Research (ClinicalTrials.gov Identifier: NCT01747148). Per the terms of the agreement, Sarepta will provide AVI-7100 to NIAID. In return, Sarepta will have the right to use the data from this clinical study to support future development of AVI-7100.

About AVI-7100

AVI-7100 is Sarepta's lead therapeutic candidate for the treatment of influenza. AVI-7100 targets a well-conserved region of the influenza A virus, affording it the potential to act as a broad-spectrum treatment for multiple influenza strains, including Tamiflu-resistant flu strains. Seasonal influenza (H3N2) and the more recently emergent swine origin influenza virus (SOIV), H1N1, are both caused by the influenza A virus. AVI-7100 employs the Company's patented PMO*plus*® technology that selectively introduces positive charges to a phosphorodiamidate morpholino oligomer (PMO) backbone to improve selective interaction between the drug and its target. AVI-7100 was preclinically developed and identified as the lead candidate with support from the U.S. Department of Defense's Joint Project Manager Transformational Medical Technologies (JPM-TMT) under contract HDTRA1-09-C-0046. Preclinical studies funded under JPM-TMT contract HDTRA1-10-C-0079 demonstrated that AVI-7100 improved clinical symptoms and reduced viral titers in animal models infected with pandemic H1N1 or H3N2 viruses, and had statistically significant activity as compared to saline and Tamiflu controls.

About Sarepta's PMO*plus*® Chemistry

PMO*plus*® chemistry is an advanced generation of Sarepta's phosphorodiamidate morpholino oligomer, or PMO, technology pioneered by Sarepta. The PMO platform is designed to provide a stable chemistry backbone with superior drug-like characteristics for Sarepta's advanced RNA-based therapeutics. PMO*plus*® chemistry includes specific molecular charges positionally inserted into the PMO's inherent charge-neutral backbone. PMO*plus*® has potentially broad therapeutic applications and has thus far shown to be particularly effective in increasing the potency of PMO-based oligomers.

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. The Company'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.sareptatherapeutics.com.

Forward-Looking Statements and Information

In order to provide Sarepta's investors with an understanding of its current results and future prospects, this press release contains statements that are forward-looking. 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 about the development of Sarepta's product candidates, their efficacy, potency and utility in the treatment of rare and infectious diseases, their potential to treat a broad number of human diseases and Sarepta's studies.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: clinical trials may not demonstrate safety and efficacy of AVI-7100 or any of Sarepta's drug candidates and/or Sarepta's antisense-based technology platform; development of AVI-7100 may not result in funding; and any of Sarepta's drug candidates may fail in development, may not receive required regulatory approvals, or be delayed to a point where they do not become commercially viable. 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 Sarepta's reports filed with the Securities and Exchange Commission. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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