Sarepta Therapeutics Initiates Dosing in Phase I Multiple Ascending Dose Study of Drug for Treatment of Marburg Virus

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CAMBRIDGE, MA -- (Marketwired) -- 05/07/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced that it has initiated dosing in a Phase 1 multiple ascending dose (MAD) clinical trial of AVI-7288, the Company's lead drug candidate for the treatment of Marburg virus infection. The Phase I MAD study is designed to characterize the safety, tolerability and pharmacokinetics of AVI-7288 after repeat dosing in healthy adult volunteers. The initiation of this study follows the successfully completed Phase 1 single ascending dose study, which showed AVI-7288 was well tolerated in healthy volunteers. Sarepta is developing AVI-7288 under a contract from the U.S. Department of Defense through the Joint Project Manager Transformational Medical Technologies (JPM-TMT) Project Management Office. AVI-7288 utilizes Sarepta's advanced and proprietary PMO*plus*® chemistry.

"The initiation of this safety study, which assesses the safety of our compound with repeated dosing, will help us understand the potential therapeutic window of our proprietary PMO*plus*® chemistry," said Chris Garabedian, President and CEO. "Together with our non-human primate efficacy studies, this safety study will help us determine the appropriate dose of this drug for the potential use as a medical counter measure against this lethal hemorrhagic fever virus."

The randomized, double-blind, placebo-controlled MAD study will be overseen by an independent Data and Safety Monitoring Board, who will review safety and clinical laboratory data after each dose cohort prior to enrolling the next highest dose cohort. Thirty-two volunteers will be enrolled in one of four cohorts made up of eight subjects each. The cohorts will include six subjects who receive the therapeutic, and two who will receive a placebo. In completed single ascending dose studies of Sarepta's Marburg and Ebola drug candidates, which both utilized the PMO*plus*® chemistry, no safety issues were identified from a total of 48 subjects receiving doses up to 9 mg/kg of either drug candidate.

Data from preclinical studies demonstrated that AVI-7288 provides post-exposure efficacy in infected non-human primates with survival rates between 83 and 100 percent when the drug is administered up to four days after exposure to the Marburg virus.

About Marburg Virus

Marburg hemorrhagic fever is a severe and potentially fatal disease in humans first recognized in 1967. It is caused by an RNA virus of the Filoviridae family and is understood to be endemic to Africa. The Marburg virus is classified as a Category A bioterrorism agent by the Centers for Disease Control and Prevention, or CDC, and is a material threat to national security and public health as determined by the Secretary of Homeland Security in 2006. Onset of the disease is often sudden, and the symptoms include fever, chills, nausea, vomiting, chest pain and diarrhea. Increasingly severe symptoms may also include massive hemorrhaging and multiple organ dysfunctions. There are currently no treatments for Marburg virus infection beyond supportive care.

About Sarepta's PMOplus® 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 JPM-TMT

JPM-TMT is a component of the U.S. Department of Defense's Joint Program Executive Office for Chemical and Biological Defense (JPEO-CBD). JPM-TMT aims to protect the Warfighter from emerging infectious diseases, genetically altered, and unknown biological threats. Through strategic investments and partnerships with innovative biotech firms, pharmaceutical corporations, other government agencies, and academic institutions, JPM-TMT facilitates the advanced development and acquisition of adaptable platform technologies, broad-spectrum medical countermeasures, and innovative systems to enhance our nation's biodefense response capability. For more information, visit www.jpmtmt.mil.

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, 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 any of Sarepta's drug candidates and/or Sarepta's antisense-based technology platform or methods of administration; development of AVI-7288 may not result in funding from JPM-TMT in the anticipated amounts or on a timely basis, if at all; and any of Sarepta's drug candidates may fail in development, may not receive required regulatory approvals, or may not become commercially viable. 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.

"Safe Harbor" Statement under the Private Securities Litigation Reform Act of 1995: The statements that are not historical facts contained in this release are forward-looking statements that involve risks and uncertainties, including, but not limited to, the results of research and development efforts, the results of preclinical and clinical testing, the effect of regulation by the FDA and other agencies, the impact of competitive products, product development, commercialization and technological difficulties, and other risks detailed in the company's Securities and Exchange Commission filings.

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