Sarepta Therapeutics Announces Positive Safety Results from Phase I Clinical Study of Marburg Drug Candidate

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 10, 2014-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced positive safety results from a Phase I multiple ascending dose study of AVI-7288 in healthy volunteers. AVI-7288, which uses Sarepta's advanced and proprietary PMO*plus*™ chemistry, is the company's lead drug candidate for the treatment of Marburg virus infection. Sarepta has been developing AVI-7288 under a Department of Defense contract managed by the Medical Countermeasure Systems BioDefense Therapeutics (MCS-BDTX) Joint Product Management Office.

The Phase I clinical study was a randomized, double-blind, placebo-controlled trial that enrolled 40 healthy adult volunteers, and was designed to characterize the safety, tolerability and pharmacokinetics of AVI-7288 after daily repeat dosing. In each of five cohorts, six subjects received AVI-7288 and two subjects received placebo, daily for 14 days. Results showed that AVI-7288 was well tolerated through the highest dose tested, 16 mg/kg per day, which is higher than the anticipated therapeutic dose, with no reported serious or clinically significant adverse events. An independent Data and Safety Monitoring Board reviewed blinded safety results from the study and recommended continued clinical development of AVI-7288.

"We are very encouraged by the AVI-7288 study results announced today," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "These safety data, combined with previously reported efficacy results showing up to 100 percent survival in infected animals, differentiates AVI-7288 as the most advanced medical countermeasure in development for the treatment of Marburg infection."

"The new data for AVI-7288 add to a growing body of evidence supporting the safety and activity of Sarepta's PMO-based chemistries," said Art Krieg, M.D., senior vice president and chief scientific officer of Sarepta Therapeutics. "Our proprietary RNA technologies offer a versatile drug development platform with broad potential utility across a spectrum of therapeutic areas."

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 (CDC), and was determined to be a material threat to national security and public health by the Secretary of the U.S. Department 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 PMO*plus*™ Chemistry

PMO*plus*™ is an advanced and proprietary chemistry based on the 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. The 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 BD-Tx and JPM-MCS

The Joint Product Management Office of BioDefense Therapeutics (BDTX) is a component of the Medical Countermeasure Systems Joint Project Management Office (JPM-MCS) within the U.S. Department of Defense's Joint Program Executive Office for Chemical and Biological Defense. JPM-MCS aims to provide U.S. military forces and the nation with safe, effective, and innovative medical solutions to counter chemical, biological, radiological, and nuclear threats. JPM-MCS facilitates the advanced development and acquisition of medical countermeasures and systems to enhance our nation's biodefense response capability. For more information, visit www.jpeocbd.osd.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.sarepta.com.

Forward-Looking Statements and Information

This press release contains "forward-looking statements" within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. 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, government decisions under existing contracts with the Company relating to Sarepta product candidates, the potential of Sarepta's PMO chemistries and RNA technologies and 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; the government may not exercise its additional options under the JPM-MCS contract or may not fund the development of AVI-7288 in anticipated amounts or on a timely basis, if at all; AVI-7288 and any of Sarepta's drug candidates may fail in development, may not receive required regulatory approvals, or may not become commercially viable; and those additional risks identified under the heading "Risk Factors" in Sarepta's Annual Report on Form 10-K for the full year ended December 31, 2012 and as updated by our 2013 third quarter 10-Q, and filed with the Securities and Exchange Commission (SEC). For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review Sarepta's reports filed 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.

Source: Sarepta Therapeutics

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