Sarepta's Marburg Drug Shows High Survival Rates After Intramuscular Delivery in Non-Human Primates

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CAMBRIDGE, MA -- (MARKETWIRE) -- 03/04/13 -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced positive results from a non-human primate study of AVI-7288, the Company's lead drug candidate for the treatment of Marburg virus infection. The data showed that intramuscular administration of AVI-7288 resulted in survival rates up to 100 percent in treated subjects, similar to efficacy observed in previous studies that evaluated the drug when administered by intravenous injection. Marburg hemorrhagic fever is a severe and highly lethal disease with no effective treatments, and it has been classified as a Category A bioterrorism agent by the Centers for Disease Control and Prevention (CDC).

"These data reinforce the strong efficacy of AVI-7288, while showing that the drug can be delivered via a convenient intramuscular injection," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "This alternative delivery method to the intravenous route has the potential to greatly enhance the practical utility of AVI-7288 in a mass casualty situation and also serves as a model for delivery of our rapidly adaptable platform for other therapeutic applications."

Sarepta is developing AVI-7288 under a U.S. Department of Defense (DoD) contract managed by the Joint Project Manager Transformational Medical Technologies (JPM-TMT) Project Management Office, a component of the Joint Program Executive Office for Chemical and Biological Defense (JPEO-CBD). Under the contract, Sarepta initiated the study in non-human primates to evaluate the tolerability, pharmacokinetics and efficacy of AVI-7288 through intramuscular administration. The study included four cohorts in which subjects received daily treatments of AVI-7288 ranging from 7.5 to 30 mg/kg or a placebo after exposure to the virus.

In the study, intramuscular injections of AVI-7288 were well tolerated. Efficacy results showed a high degree of survival between 83 and 100 percent in each of the three treatment groups. No subjects survived in the placebo-treated control group.

Under a separate contract with JPM-TMT, Sarepta is developing an intravenous formulation of AVI-7288, which has demonstrated similar survival rates even when the drug is administered up to four days after exposure to the Marburg virus.

The work is a collaborative effort between Sarepta and scientists at the U.S. Army Medical Research Institute of Infectious Diseases (USAMRIID), the DoD's leading medical research laboratory for biological defense, which has the DoD's only maximum containment, or Biosafety Level 4, capability.

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 USAMRIID

U.S. Army Medical Research Institute of Infectious Diseases (USAMRIID), located at Fort Detrick, Maryland, is the lead medical research laboratory for the U.S. Department of Defense's Biological Defense Research Program, and plays a key role in national defense and in infectious disease research. The Institute conducts basic and applied research on biological threats resulting in medical solutions (such as vaccines, drugs and diagnostics) to protect the Warfighter. While USAMRIID's primary mission is focused on the military, its research often has applications that benefit society as a whole. USAMRIID is a subordinate laboratory of the U.S. Army Medical Research and Materiel Command. For more information, visit www.usamriid.army.mil.

About Sarepta Therapeutics

Sarepta Therapeutics -- formerly AVI BioPharma -- 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 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 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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