Sarepta Therapeutics' Lead Therapeutic Drugs for Ebola and Marburg Viruses Receive FDA Fast Track Designation

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Sep 18, 2012 (Marketwire via COMTEX) --Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track status for the development of its lead infectious disease drug candidates, AVI-7288 and AVI-7537, for the treatment of Marburg virus and Ebola virus, respectively. Sarepta has been developing these platform-based therapeutics 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).

The FDA's Fast Track program is designed to facilitate the development of, and expedite the review of, new drugs that have shown the potential to address an unmet medical need in a serious or life-threatening disease. Fast Track designated drugs qualify for Priority Review, an expedited review process available to drugs that offer major advances in treatment or provide a treatment where no adequate therapy exists.

"This is a significant milestone for Sarepta," said Chris Garabedian, President and CEO. "The FDA recognizes the significant unmet medical need for these highly lethal hemorrhagic fever viruses and the necessity to move the development of these drugs forward as fast as possible to ensure effective medical countermeasures for the Warfighter. We believe Sarepta has the only drug candidates for Marburg and Ebola with Fast Track designation and expect this designation will provide an expedited approval process to help DoD achieve its mission of protecting the nation from biothreats."

Sarepta continues to evaluate the safety and efficacy of AVI-7288 for the treatment of Marburg virus following FDA's Animal Rule and next plans to initiate a Phase I multiple ascending dose study to characterize the safety, tolerability and pharmacokinetics of AVI-7288 after repeat dosing in healthy adult volunteers. Currently, the JPM-TMT's Ebola programs, including development of AVI-7537, remain under a temporary stop-work order due to funding constraints.

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 was determined to pose a material threat to national security and public health 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 Ebola Virus

The hemorrhagic fever caused by the Ebola virus is severe and often fatal in humans. The disease was first recognized in 1976 and is one of two members of a family of RNA viruses called Filoviridae. The disease is generally understood to be endemic to parts of Africa. Onset of illness from Ebola virus is abrupt with symptoms that include fever, headache, muscle ache, vomiting and stomach pain. Internal and external bleeding may also be observed in some patients. There are currently no treatments for Ebola virus infection beyond supportive care and the mortality rate is very high.

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 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 http://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 http://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; 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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