Sarepta Therapeutics Announces Publication of Ebola and Marburg Phase I Clinical Study Results in *Antimicrobial Agents and Chemotherapy*

Results Show PMO*plus*® Drug Candidates to be Well-Tolerated with no Clinically Significant Effects on any Evaluated Safety Parameters Observed

CAMBRIDGE, Mass., Oct 16, 2014 (BUSINESS WIRE) –Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced the publication of results from two single ascending-dose studies that demonstrated no clinical or toxicologic safety concerns with the company's drug candidates for the treatment of Ebola and Marburg virus, respectively. The study results are to be published in the November issue of the American Society for Microbiology's journal, *Antimicrobial Agents and Chemotherapy* and are available online at dx.doi.org/10.1128/AAC.03442-14.

AVI-6002 for the treatment of Ebola is a combination therapy of two phosphorodiamidate morpholino oligomers (PMOs AVI-7537 and AVI-7539), which target the viral matrix proteins VP24 and VP35, respectively. AVI-6003 for the treatment of Marburg is a combination therapy of two PMOs, (AVI-7287 and AVI-7288), which target the viral proteins VP24 and NP, respectively. These drug candidates use Sarepta's advanced and proprietary PMO*plus*® chemistry, which is also the basis of the company's clinical-stage influenza drug candidate, AVI-7100. Results from previous viral challenge studies of AVI-6002 and AVI-6003 in non-human primates demonstrated prevention of disease development and death following exposure to Ebola or Marburg virus. Subsequent animal studies demonstrated that for each combination therapy, only one oligomer contributed to efficacy, and therefore, the lead drug candidates for Ebola and Marburg have since become the single compounds AVI-7537 and AVI-7288.

"We believe these promising early clinical safety results, coupled with the strong safety and efficacy data generated from animal studies for all four PMO compounds, reinforce the use of our PMO*plus*® chemistry platform to pursue potential treatments for deadly infectious diseases such as Ebola and Marburg," said Michael Wong, senior medical director, infectious diseases at Sarepta Therapeutics. "We are particularly encouraged to see results such as these in the healthy human volunteers to what we have learned to be the effective agents, AVI-7537 and AVI-7288. These compounds have protected up to 80-100 percent of the non-human primates to Ebola and Marburg virus challenge infections, respectively."

The two Phase I clinical studies were randomized, double-blind, placebo-controlled trials designed to characterize the safety, tolerability and pharmacokinetics of single doses of intravenous formulations of AVI-6002 or AVI-6003 in healthy adult volunteers. In each study, 30 subjects were enrolled in six cohorts receiving up to 9 mg/kg of the combination drug candidates (4 active:1 placebo per cohort) for a total of

60 subjects. Results showed the compounds to be well-tolerated with no dose limiting level demonstrated. No clinically significant or dose-dependent effects were observed at any of the safety endpoints evaluated. The safety and pharmacokinetics of the four PMO*plus*® compounds comprising the two combination therapies were similar, regardless of the target RNA sequence.

A previously reported Phase I MAD study of AVI-7288 for the treatment of Marburg found no clinically significant or dose-dependent effects on any of the safety endpoints evaluated when tested at up to 16 mg/kg/day for 14 days in healthy adult volunteers. The results of these clinical studies add to a growing body of evidence supporting the safety of Sarepta's PMO-based chemistry platform across a broad range of disease targets.

This work was conducted under contract with the Joint Product Management Office of BioDefense Therapeutics (BD-Tx).

Works Cited

Antimicrob. Agents Chemother. November 2014 58:6639-6647; published ahead of print 25 August 2014 , doi:10.1128/AAC.03442-14
Safety and Pharmacokinetic Profiles of Phosphorodiamidate Morpholino Oligomers with Activity against Ebola Virus and Marburg Virus: Results of Two Single-Ascending-Dose Studies
Alison E. Heald, Patrick L. Iversen, Jay B. Saoud, Peter Sazani, Jay S. Charleston, Tim Axtelle, Michael Wong, William B. Smith, Apinya Vutikullird, and Edward Kaye

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 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.

About Sarepta Therapeutics

Sarepta 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 and follow-on drug candidates, 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

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

regarding the use of our PMOplus™ chemistry platform to pursue potential treatments for deadly infectious diseases such as Ebola and Marburg and the growing body of evidence supporting the safety of PMOs across a broad range of disease targets..

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 continue to be consistent with prior results supporting the safety and efficacy AVI-6002, AVI-6003 or any of Sarepta's drug candidates and/or Sarepta's PMO-based chemistry platform; AVI-6002, AVI-6003 and any of Sarepta's drug candidates, including those using Sarepta's PMO-based chemistry may not be further developed by Sarepta for various reasons some of which may be outside of Sarepta's control, 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 Quarterly Report on Form 10-Q for the Quarter ended June 30, 3014 filed with the Securities and Exchange Commission (SEC) and Sarepta's other filings 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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