



## **Sarepta Therapeutics Announces Favorable Safety Results from Phase I Clinical Study of Influenza Drug Candidate**

CAMBRIDGE, Mass.—Sep. 30, 2014—Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-based therapeutics, today announced favorable safety results from the single ascending dose portion of a Phase I study of AVI-7100, the company's lead candidate for the treatment of influenza virus, in healthy volunteers. The clinical trial is being conducted at the National Institutes of Health (NIH) through a collaboration between the company and the NIH's National Institute of Allergy and Infectious Diseases. AVI-7100 uses Sarepta's advanced and proprietary PMOplus™ chemistry, which is also the basis of the company's clinical-stage Ebola and Marburg drug candidates.

The Phase I clinical study is a randomized, double-blind, placebo-controlled trial designed to characterize the safety, tolerability and pharmacokinetics of single and multiple doses of an intravenous formulation of AVI-7100 in healthy adult volunteers. In the completed single ascending dose portion, 40 subjects were enrolled in five cohorts (6 active: 2 placebo) up to the highest dose tested of 8 mg/kg AVI-7100. Results showed that AVI-7100 was well-tolerated with no reported serious or clinically significant adverse events. The pharmacokinetic analysis of AVI-7100 reveals a highly similar dose-dependent profile to that of Sarepta's Ebola and Marburg PMOplus™ drug candidates. An independent Data and Safety Monitoring Board reviewed safety results from the study and recommended the study continue as planned to the multiple dose portion of the study.

"We are very encouraged that our partnership with NIH is generating new favorable clinical safety data, adding to a growing body of evidence supporting the safety of Sarepta's PMO-based chemistry platform across a broad range of disease targets," said Chris Garabedian, president and chief executive officer of Sarepta Therapeutics. "The similar drug-like characteristics demonstrated across a broad spectrum of targets offers a versatility that will be especially critical in developing a capability to rapidly respond and adapt to real-world, global health threats – whether that is an Ebola outbreak, an influenza pandemic, or a never-before-seen emerging infectious disease."

AVI-7100 utilizes a novel mechanism of action to target a well-conserved region of the influenza A virus, affording it the potential to act as a broad-spectrum treatment for multiple influenza strains, including Tamiflu-resistant flu strains. Seasonal influenza (H3N2) and 2009 H1N1 are both caused by the influenza A virus. Preclinical studies funded by the U.S. Department of Defense demonstrated that AVI-7100 improved clinical symptoms and reduced viral titers in animal models infected with pandemic H1N1 or H3N2 viruses, and had statistically significant activity as compared to saline and Tamiflu controls.

### **About Sarepta's PMOplus® Chemistry**

PMOplus® 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. PMOplus® chemistry includes specific molecular charges positionally inserted into the PMO's inherent

charge-neutral backbone. PMOplus® has potentially broad therapeutic applications and has thus far shown to be particularly effective in increasing the potency of PMO-based oligomers.

## **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 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](http://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 about the development of AVI-7100; continuing to the multi-dose portion of the AVI-7100 study; Sarepta's partnership with the National Institutes of Health generating new favorable clinical safety data and adding to a growing body of evidence supporting the safety of Sarepta's PMO-based chemistry platform across a broad range of disease targets; similar drug-like characteristics demonstrated across a broad spectrum of targets offering a versatility that will be especially critical in developing a capability to rapidly respond and adapt to real-world, global health threats; and the potential of AVI-7100 to act as a broad spectrum treatment for multiple influenza strains.*

*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 AVI-7100 or any of Sarepta's drug candidates and/or Sarepta's PMO-based chemistry platform; AVI-7100 and any of Sarepta's drug candidates, including those using Sarepta's PMO-based chemistry 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, 2014 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.*

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