Sarepta Therapeutics Announces New England Journal of Medicine Publication of Phase I Clinical Data of Marburg Drug Candidate, AVI-7288, Supporting Safety of the PMO*plus*® platform

CAMBRIDGE, Mass.--(BUSINESS WIRE)--July 23, 2015-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a developer of innovative RNA-targeted therapeutics, today announced the publication of results from a multiple ascending dose study to determine the safety of AVI-7288, a PMO*plus*® antisense oligonucleotide, in healthy adult volunteers, in the July 23, 2015 issue of The New England Journal of Medicine. The results of the study, conducted in collaboration with the U.S. Army Medical Research Institute of Infectious Diseases (USAMRIID), demonstrated no clinical or toxicologic safety concerns with AVI-7288, an investigational treatment for Marburg Virus (MARV) infection. These results add to the continued documentation of safety data for Sarepta's PMO-based technology.

AVI-7288 utilizes Sarepta's advanced and proprietary PMO*plus*® technology. AVI-7288 is designed to bind to viral messenger RNA encoding Marburg Virus nucleoprotein to inhibit nucleoprotein synthesis and prevent viral replication and assembly. This mechanism of AVI-7288 is fundamentally distinct from other RNA-based anti-infective therapies that utilize a gene editing or degradation pathway. Additionally, this approach highlights the flexibility and precision of the PMO-based platform.

The Phase I clinical study was a randomized, double-blind, placebo-controlled trial designed to characterize the safety, tolerability and pharmacokinetics of AVI-7288 after daily repeat dosing. Over 14 days, 40 healthy human volunteers (8 per dose group) were dosed with up to 16 mg/kg/day, representing the highest continuous dosing of any PMO*plus*® or any other antisense oligonucleotide. This dosing also exceeded the predicted human efficacious dose for AVI-7288 estimated by three different models based upon non-human primate studies demonstrating up to 100% animal survival, including in a delayed time to treat setting.

In healthy human volunteers, no significant safety concerns or dose-dependent adverse side effects of AVI-7288 were reported with respect to any safety end point evaluated, nor were any gross abnormalities in renal function or biomarkers of renal dysfunction observed. The maximum dose of AVI-7288 that could be administered without raising significant safety concerns was not reached.

“These data add to the growing body of evidence underpinning the safety profile of Sarepta’s PMO-based chemistry platform and its potential in treating a variety of diseases.” stated Michael Wong, MD, Senior Medical Director, Infectious Diseases. “We look forward to continuing to build upon these data and further demonstrating the versatility and utility of this novel precision medicine approach in tackling some of the most challenging infectious disease threats today including pandemic influenza and antimicrobial resistance.”

“Results described in this manuscript provide further confirmation that PMO-based antisense therapeutics can protect against a highly pathogenic virus in nonhuman primate disease models and, importantly, that the dose regimen that we predict to be efficacious in humans is not likely to compromise patient safety during treatment,” said Sina Bavari, Ph.D., USAMRIID Science Director.

This work was conducted under contract with the Department of Defense Medical Countermeasures Systems/Joint Product Management Office of BioDefense Therapeutics (BD-Tx).

Works Cited

N Engl J Med 2015;373;339-48; doi:10.1056/NEJMoa1410345
AVI-7288 for Marburg virus in nonhuman primates and humans.
Alison E. Heald, Jay S. Charleston, Patrick L. Iversen, Travis K. Warren, Jay B. Saoud, Mohamed Al-Ibrahim, Jay Wells, Kelly L. Warfield, Dana L. Swenson, Lisa S. Welch, Peter Sazani, Michael Wong, Diane Berry, Edward M. Kaye, and Sina Bavari.

About Sarepta's PMO*plus*® Chemistry Platform

PMO*plus*® chemistry platform is an advanced generation of Sarepta's phosphorodiamidate morpholino oligomer, or PMO, technology pioneered by Sarepta. The PMO-based platform is designed to provide a stable chemistry backbone with drug-like characteristics for Sarepta's advanced RNA-targeted therapeutics. The PMO*plus*® chemistry platform provides positionally specific positive molecular charges into the inherently charge-neutral PMO.

About Sarepta Therapeutics

Sarepta Therapeutics is a biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment of rare, infectious and other life threatening diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates, including its lead DMD product candidate, eteplirsen, designed to skip exon 51. Sarepta is also developing therapeutics for the treatment of drug-resistant bacteria and infectious, rare and other human diseases. 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 safety data and profile being collected on the use of PMO-based technology, the mechanism and design of AVI-7288 and how they are distinct from other RNA-based anti-infective therapies, the flexibility and precision of Sarepta's PMO-based platform and its potential in treating a variety of diseases, Sarepta's plans to add to the existing body of data and demonstrate the versatility and utility of this novel precision medicine approach in treating some of the most challenging infectious diseases today (including influenza and antimicrobial resistance), and the potential safety, efficacy and dosing of PMO-

based therapeutics.

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, efficacy or dosing of AVI-7288, any of Sarepta's drug candidates and/or Sarepta's PMO-based chemistry platform, AVI-7288 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 March 31, 2015 and Annual Report on Form 10-K for the year ended December 31, 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.

Internet Posting of Information

We routinely post information that may be important to investors in the 'For Investors' section of our website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

Source: Sarepta Therapeutics

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