Sarepta Therapeutics Enters into Research and Option Agreement with Nationwide Children's Hospital for Microdystrophin Gene Therapy Program

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 10, 2017-- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial stage developer of innovative RNA-targeted therapeutics, today announced it has entered a research and option agreement with Nationwide Children's Hospital on their microdystrophin gene therapy program. Dr. Jerry Mendell, M.D. and Dr. Louise Rodino-Klapac, Ph.D., are the lead principal investigators of the program.

"Given the complexities of Duchenne muscular dystrophy, we know that it is going to require multiple treatment approaches," said Edward Kaye, Sarepta's chief executive officer. "With that goal in mind, we are excited to support clinical development for Nationwide's gene therapy program with the goal to help all boys with DMD."

The initial trial, expected to go into Phase 1/2a trial in late 2017, will be conducted at Nationwide Children's. Parent Project Muscular Dystrophy (PPMD) has committed 2.2 million dollars to the trial, with support from additional Duchenne foundations and families. Sarepta has committed to the trial through a separate research agreement with Nationwide Children's, and has an exclusive option to license the program. PPMD's grant provided incentive for Sarepta to help expand and accelerate this opportunity.

"We are thrilled Sarepta has entered into this research agreement with Nationwide. The additional resources will bolster Nationwide's ability to conduct an even more rigorous and robust trial," said Pat Furlong, founding president and chief executive officer of Parent Project Muscular Dystrophy.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of unique RNA-targeted therapeutics for the treatment

of rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates. For more information, please visit us at www.sarepta.com.

About The Research Institute at Nationwide Children's Hospital

Named to the Top 10 Honor Roll on *U.S. News & World Report's* 2016-17 list of "America's Best Children's Hospitals," Nationwide Children's Hospital is one of America's largest not-for-profit freestanding pediatric healthcare systems providing wellness, preventive, diagnostic, treatment and rehabilitative care for infants, children and adolescents, as well as adult patients with congenital disease. As home to the Department of Pediatrics of The Ohio State University College of Medicine, Nationwide Children's faculty train the next generation of pediatricians, scientists and pediatric specialists. The Research Institute at Nationwide Children's Hospital is one of the Top 10 National Institutes of Health-funded free-standing pediatric research facilities in the U.S., supporting basic, clinical, translational and health services research at Nationwide Children's. The Research Institute encompasses three research facilities totaling 525,000 square feet dedicated to research. More information is available at NationwideChildrens.org/Research.

Forward-Looking Statements

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 research and exclusive option agreement Sarepta has entered into with Nationwide Children's Hospital for their microdystrophin gene therapy program, that DMD is going to require multiple treatment approaches, Sarepta supporting Nationwide's gene therapy program, the goal to help all boys with DMD, Sarepta's resources bolstering Nationwide's ability to conduct an even more rigorous and robust trial, and the expected clinical progress of the program in 2017.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the expected benefits and opportunities related to the agreements may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; in particular the agreements may not result in any viable treatments suitable for commercialization due to a variety of reasons including any

inability of the parties to perform their commitments and obligations under the agreements, the results of research may not be consistent with past results or may not be positive or may otherwise fail to meet regulatory approval requirements for the safety and efficacy of product candidates and even if commercialization of any product under the agreements is achieved this may not result in any significant revenues to the parties.

Any of the foregoing risks could 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 2015 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2016 filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by Sarepta. 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 web site at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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