



## **Sarepta Therapeutics Enters into License Agreement with Nationwide Children’s Hospital for Galgt2 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 an exclusive license agreement with Nationwide Children’s Hospital, for their Galgt2 gene therapy program developed by researcher Dr. Paul Martin, Ph.D.

“We are taking a multi-front approach in the battle against Duchenne muscular dystrophy,” said Edward Kaye, Sarepta’s chief executive officer. “We find this therapeutic approach to be of particular interest as it has the potential to treat all patients suffering from the disease regardless their mutation.”

The experimental program, explores the potential surrogate gene therapy approach to Duchenne muscular dystrophy. As a “surrogate gene therapy approach”, the gene therapy looks to induce genes that make proteins that can perform a similar function as dystrophin, with the goal of producing a muscle cell that can function normally even when dystrophin is absent. It has the potential to be used broadly in several muscular dystrophies.

“We are pleased to have this opportunity to help advance our Galgt2 gene therapy program,” said Dr. Kevin Flanigan, the Principal Investigator leading the clinical trial. “Our goal is to have this program in the clinic during 2017 and begin to evaluate a therapy that has the potential to treat patients of all ages and disease severity.”

### **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](http://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](http://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 exclusive license agreement Sarepta has entered into with Nationwide Children's Hospital for their Galgt2 gene therapy program, the potential for this therapeutic approach to treat all patients suffering from DMD regardless of their mutation, age and disease severity, the expected clinical progress of the program in 2017, the potential for the gene therapy to be used in several muscular dystrophies, the gene therapy's mechanism of action and potential efficacy and Sarepta taking a multi-front approach in the battle against DMD.*

*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 exclusive license agreement and related agreements between the parties may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the partnership between Sarepta and Nationwide Children's hospital may*

*not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including that the results of additional 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 or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreements; and even if the agreements result in commercialized products, the parties may not achieve any significant revenues from the sale of such products.*

*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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

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