Sarepta Therapeutics Signs Exclusive Global Collaboration with Duke University for Gene Editing CRISPR/Cas9 Technology to Develop New Treatments for Duchenne Muscular Dystrophy (DMD)

-- Exclusive license option grants Sarepta rights to Duke intellectual property for CRISPR/Cas9 --

CAMBRIDGE, Mass., October 31, 2017 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases, today announced that it has signed a research collaboration agreement with Duke University, granting the Company an option to an exclusive license to intellectual property and technology related to CRISPR/Cas9 technology developed in the laboratory of Charles A. Gersbach, Ph.D. The underlying premise of Dr. Gersbach's approach is to restore dystrophin expression by removing or "excising" exons from the dystrophin gene. This includes a strategy to excise exons potentially enabling treatment for a majority of the DMD patient population. Sarepta will collaborate with Dr. Gersbach's lab to advance the CRISPR platform and take the lead on clinical development.

"Gene editing has the potential to revolutionize the treatment of diseases with genetic mutations. We are particularly excited about the potential it holds for DMD patients," said Douglas Ingram, Sarepta's president and chief executive officer. "We will work closely with Dr. Gersbach, a pioneer in applying the CRISPR technology to treat Duchenne, to advance a program that builds upon the established body of research by Dr. Gersbach and his team. Today's agreement exemplifies our strategy of investing in and advancing a multi-faceted array of potential therapies for the largest number of individuals with DMD by leveraging our own research and development efforts, as well as forging external partnerships with the field's best and brightest minds."

"Although early, CRISPR technology represents hope for a large percentage of individuals with DMD. Excising certain exons has the potential to correct a majority of DMD mutations. Toward that goal, we've shown in mouse models that we can excise exons from the dystrophin gene, leading to restoration of a functional dystrophin protein and improvements in muscle strength," said Dr. Gersbach, an associate professor in Duke University's Department of Biomedical Engineering. "We are pleased to be partnering with

Sarepta, a leader in the development of DMD therapies. The Company's dedication to the patient community, and their goal to pursue a variety of scientific approaches, makes them an ideal partner for Duke and our team of researchers in pursuing our goal of translating the science into a treatment."

The financial terms of the agreement have not been disclosed.

About the Gersbach Laboratory – Genome Editing for Cell and Gene Therapy and DMD

The Gersbach Lab is dedicated to applying innovative methods in molecular and genetic engineering to advancing regenerative medicine, treating genetic disease, and enhancing our understanding of fundamental biological processes. In particular, the Gersbach Laboratory aims to develop new technologies to modify genome sequences, epigenomic regulation, and cellular gene networks in a precise and targeted manner. Examples of technologies used in Dr. Gersbach's research include genome and epigenome editing with CRISPR/Cas9 and other DNA-targeting systems, protein engineering, directed evolution, genetic reprogramming, and optogenetics.

The work of Dr. Gersbach and his team falls within the larger field of genome editing for cell and gene therapy that can be applied to diverse diseases and disorders (Nelson, Robinson-Hamm, and Gersbach, *Nature Reviews Neurology* 2017). A primary example of Dr. Gersbach's work in this area is developing genome editing methods such as CRISPR/Cas9 to correct mutations to the dystrophin gene that cause DMD. Dr. Gersbach and his team have used genome editing to correct mutations in patient cells and demonstrated restored dystrophin expression in patient cells in culture and after transplantation into skeletal muscle in mouse models (Ousterout et al., *Molecular Therapy* 2013; *Molecular Therapy* 2015; *Nature Communications* 2015). More recently, they have extended this work to correcting dystrophin mutations *in vivo* in mouse models of DMD (Nelson et al., *Science* 2016).

About Duke University's Pratt School of Engineering

Duke University's Pratt School of Engineering is a vibrant teaching and research institution dedicated to training the next generation of leaders and exploring the frontiers of engineering to develop solutions to societal challenges. Ranked among the top 20 engineering schools in the nation by *U.S. News & World Report*, the school includes highly interdisciplinary academic programs in biomedical engineering, civil and environmental engineering, electrical and computer engineering and mechanical engineering and materials science. With more than $70 million annually in research expenditures, Duke Engineering is home to major research centers focused on areas including biomolecular and tissue engineering, metamaterials,

photonics, environmental implications of nanotechnology, materials science, materials genomics and quantum computing, among others.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicines to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying Duchenne muscular dystrophy (DMD) drug candidates. For more information, please visit www.sarepta.com.

Forward-Looking Statements

This press release contains "forward-looking statements". 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 Sarepta's plan to collaborate with Dr. Gersbach's lab to advance the CRISPR platform, take the lead on clinical development and advance a program that builds upon the established body of research by Dr. Gersbach and his team; the potential of gene editing to revolutionize the treatment of diseases with genetic mutations and DMD in particular; the potential of excising certain exons to correct a majority of DMD mutations; Sarepta's strategy of investing in and advancing a multi-faceted array of potential therapies for the largest number of individuals with DMD by leveraging Sarepta's own research and development efforts, as well as forging external partnerships with the field's best and brightest minds; CRISPR technology representing hope for a large percentage of individuals with DMD; Sarepta's dedication to the patient community and goal to pursue a variety of scientific approaches; and Duke University and its team of researchers' goal to translate the science into a treatment.

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 research collaboration agreement with Duke University may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the collaboration with Duke University may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may 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 agreement, including any inability by Sarepta to fulfill its financial commitments to Duke University; and even if the agreement results in commercialized products, the parties may not achieve any significant revenues from the sale of such products; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2016 and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company'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 June 30, 2017 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 website at www.sarepta.com. We encourage investors and potential investors to consult our website regularly for important information about us.

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