



Sarepta Therapeutics and University of Florida Announce Collaboration to Accelerate the Discovery and Development of Therapies for Rare Genetic Diseases

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CAMBRIDGE, Mass. and GAINESVILLE, Fla., Aug. 11, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, and the University of Florida today announced a strategic collaboration to enable cutting-edge research for novel genetic medicines. Through the agreement, Sarepta will fund multiple research programs at the University, and will have an exclusive option to further develop any new therapeutic compounds that result from the funded research programs.

"We have developed a productive incubator approach to our pipeline development, partnering with the best and brightest in genetic medicine, including leading academic researchers like those at the University of Florida, to discover and translate into meaningful therapies genetic medicine for rare diseases," said Sarepta President and CEO Doug Ingram. "We are excited to partner with and support UF research that has the potential to profoundly improve and extend the lives of patients with rare genetic-based diseases."

Through the collaboration, currently unique to UF, funding has been allocated for four innovative projects. These projects include exploratory research in novel gene therapy vectors, next-generation capsids and gene editing technologies as well as work in new therapeutic areas in degenerative genetic diseases. The goal is to foster early relationships with experts and accelerate the scientific advancements that lead to the development of transformational precision genetic medicines for patients in need.

"Our researchers intend to find solutions for diseases that have no cure or limited therapeutic options. Their goal is to move these solutions from their labs to patients who need them – to see their discoveries change lives. Because Sarepta has a focus and expertise in disease areas that coincide with the work of some of our scientists, it's a match and collaboration that make sense and, we hope, will save lives," said Jim O'Connell, assistant vice president of UF Innovate, the technology commercialization arm of the university. "Sarepta has a bold vision for transforming genetic disease because the company, ultimately, serves patients. That end goal drives its willingness and ability to translate research into a medical reality. We want to be part of that."

University of Florida is a gene therapy powerhouse. UF researchers were the first to discover the life cycle of the adeno-associated virus (AAV), the smallest human virus. Using AAV as a benign delivery vehicle to carry therapeutics to a target, UF was first to reverse blindness in dogs with genetic disease, and UF researchers were integral in the first gene therapy approved by the FDA to treat an inherited genetic disease that can cause blindness. Today, UF is developing technologies in manufacturing, capsid design and therapies to address neuromuscular, cardiovascular, inflammatory, metabolic, pulmonary, skeletal, ophthalmic, and other disorders.

About Sarepta

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on [Twitter](#), [LinkedIn](#), [Instagram](#) and [Facebook](#).

Sarepta 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 the ability of the collaboration between Sarepta and UF to engage in cutting-edge research for novel genetic medicines; Sarepta's commitment to fund multiple research programs at UF; Sarepta's option to further develop any new therapeutic compounds that result from the funded research programs; Sarepta's incubator approach to discover and translate into meaningful therapies genetic medicine for rare diseases; the collaboration's potential to profoundly improve and extend the lives of patients with rare genetic-based diseases; the collaboration's ability to foster early relationships with experts to accelerate the scientific advancements that lead to the development of transformational precision genetic medicines; and Sarepta's vision to transform genetic disease and translate research into a medical reality.

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 collaboration between Sarepta and UF 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 collaboration may not result in the discovery of any new therapeutic compounds or 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 agreement; Sarepta may not be able to execute on its business plans and goals, including meeting its expected or planned regulatory milestones and timelines, clinical development plans, and bringing its product candidates to market, due to a variety of reasons, many of which may be outside of Sarepta's control, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved for in a timely manner, regulatory, court or agency decisions, such as decisions by the United States Patent and Trademark Office with respect to patents that cover Sarepta's product candidates and the COVID-19 pandemic; 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, 2019, 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 Sarepta which you are encouraged to review.

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 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.

Contacts:

Sarepta Therapeutics

Investors: Ian Estepan, 617-274-4052, iestepan@sarepta.com

Media: Tracy Sorrentino, 617-301-8566, tsorrentino@sarepta.com

UF Innovate:

Sara Dagen, 352-294-0998, saradagen@ufl.edu