Sarepta Therapeutics and Dyno Therapeutics Announce Agreement to Develop Next-Generation Gene Therapy Vectors for Muscle Diseases

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-- Agreement leverages Sarepta's leadership in gene therapy for neuromuscular and cardiovascular diseases and Dyno's CapsidMap artificial intelligence platform to design AAV vectors --

CAMBRIDGE, Mass., May 11, 2020 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, and Dyno Therapeutics, Inc., a biotech company applying artificial intelligence (AI) to gene therapy, today announced an agreement to develop next-generation Adeno-Associated Virus (AAV) vectors for muscle diseases, using Dyno's CapsidMap™ platform.

AI and machine learning technologies have the potential to deliver enhanced vectors for gene therapies. Dyno's proprietary CapsidMap platform opens up new ways to identify novel capsids – the cell-targeting protein shell of viral vectors – that could offer improved muscle targeting and immune-evading properties, in addition to advantages in packaging and manufacturing.

"Sarepta's world-leading gene therapy engine is founded on three pillars: developing a broad portfolio of programs to treat rare diseases; our first-in-class manufacturing expertise; and investment in advancing and further improving the science of gene therapy to help patients in need of more options. To that end, our agreement with Dyno provides us with another valuable tool to develop next-generation capsids for gene therapies to treat rare diseases," said Doug Ingram, Sarepta's President and Chief Executive Officer. "By leveraging Dyno's AI platform and Sarepta's deep expertise in gene therapy development, our goal is to advance next-generation treatments with improved muscle-targeting capabilities."

Under the terms of the agreement, Dyno will be responsible for the design and discovery of novel AAV capsids with improved functional properties for gene therapy and Sarepta will be responsible for conducting preclinical, clinical and commercialization activities for gene therapy product candidates using the novel capsids. If successful, Dyno could receive over $40 million in upfront, option and license payments during the research phase of the collaboration. Additionally, if Sarepta develops and commercializes multiple candidates for multiple muscle diseases, Dyno will be eligible for additional significant future milestone payments. Dyno will also receive royalties on worldwide net sales of any commercial products developed through the collaboration.

"This agreement is a major step forward in our plan to realize the potential of Dyno's AI platform for gene therapies to improve patient health. We are excited to work with Sarepta to create gene therapies with improved properties to address a range of muscle-related diseases," stated Dyno's CEO and co-founder Eric D. Kelsic, Ph.D. "The success of the gene therapies developed through this collaboration with Sarepta will rely on AI-powered vectors that allow gene therapies to be safely and precisely targeted to the muscle tissue."

About CapsidMap™ for Designing AAV Gene Therapies
By designing capsids that confer improved functional properties to Adeno-Associated Virus (AAV) vectors, Dyno's proprietary CapsidMap™ platform overcomes the limitations of today's gene therapies on the market and in development. Today's treatments are primarily confined to a small number of naturally occurring AAV vectors that are limited by delivery, immunity, packaging size, and manufacturing challenges. CapsidMap uses artificial intelligence (AI) technology for the design of novel capsids, the cell-targeting protein shell of viral vectors. The CapsidMap platform applies leading-edge DNA library synthesis and next-generation DNA sequencing to measure *in vivo* gene delivery properties in high throughput. At the core of CapsidMap are advanced search algorithms leveraging machine learning and Dyno's massive quantities of experimental data, that together build a comprehensive map of sequence space and thereby accelerate the discovery and optimization of synthetic AAV capsids.

Dyno's technology platform builds on certain intellectual property developed in the lab of George Church, Ph.D., who is Robert Winthrop Professor of Genetics at Harvard Medical School (HMS), a Core Faculty member at Harvard's Wyss Institute for Biologically Inspired Engineering, and a co-founder of Dyno. Several of the technical breakthroughs that enabled Dyno's approach to optimize synthetic AAV capsid engineering were described in a November 2019 publication in the journal *Science,* based on work conducted by Dyno founders and members of the Church Lab at HMS and the Wyss Institute. Dyno has an exclusive option to enter into a license agreement with Harvard University for this technology.

About Dyno Therapeutics
Dyno Therapeutics is a pioneer in applying artificial intelligence (AI) and quantitative high-throughput *in vivo* experimentation to gene therapy. The company's proprietary CapsidMap™ platform is designed to rapidly discover and systematically optimize superior Adeno-Associated Virus (AAV) capsid vectors with delivery properties that significantly improve upon current approaches to gene therapy and expand the range of diseases treatable with gene therapies. Dyno was founded in 2018 by experienced biotech entrepreneurs and leading scientists in the fields of gene therapy and machine learning. The company is located in Cambridge, Massachusetts. Visit www.dynotx.com for additional information.

About Sarepta Therapeutics
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 Therapeutics 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 potential

of artificial intelligence and machine learning technologies to deliver enhanced vectors for gene therapies; the potential of the CapsidMap™ platform to offer improved muscle targeting and immune-evading properties, in addition to advantages in packaging and manufacturing; the agreement between Sarepta and Dyno Therapeutics providing a valuable tool to develop next-generation capsids for gene therapies to treat rare disease; the parties' goal to advance next-generation treatments with improved muscle-targeting capabilities; the parties' responsibilities under the agreement and potential payments to Dyno Therapeutics; and the potential of AI-powered vectors to allow gene therapies to be safely and precisely targeted to the muscle tissue.

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 Dyno Therapeutics 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 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; 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; 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 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 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 2019 Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q filed with the 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.

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