# Sarepta Therapeutics Announces Progress on the MyoAAV Program and Exclusive Licensing Agreement with The Broad Institute for MyoAAV Next-generation Capsids for Rare Genetic Diseases

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- ***Following internal corroboration of published results on the MyoAAV platform, Sarepta secures exclusive license for Duchenne muscular dystrophy, plus four additional neuromuscular and cardiac indications***

- ***The MyoAAV platform is a potential breakthrough in genetic medicine delivery, with early research showing significantly greater gene expression at lower doses compared to natural serotype capsids***

CAMBRIDGE, Mass., Aug. 08, 2022 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced that following progress on its sponsored research agreement on the MyoAAV program, it has executed a license agreement with the Broad Institute of MIT and Harvard (Broad Institute) for MyoAAV in Duchenne muscular dystrophy and certain other neuromuscular and cardiac indications. The announcement follows confirmation by Sarepta's internal research and manufacturing teams of earlier published research from Broad Institute, which was conducted under a research agreement that began in 2020.

MyoAAV is a new group of adeno-associated viruses (AAV) that use a modified outer protein shell of AAV, known as the capsid, to deliver genetic therapies with greater efficiency and at lower doses. Data published in the journal *Cell* in 2021 found that, in mouse models of Duchenne muscular dystrophy and X-linked myotubular myopathy, MyoAAV demonstrated more efficient delivery of gene therapy and gene editing payloads, resulting in complete restoration of muscle function and improved survival. In preclinical data from non-human primates, compared to natural AAV serotypes, MyoAAV:

- Delivered 25-50 times greater gene expression in multiple skeletal muscles and 10-15 times greater gene expression in cardiac muscle;
- Demonstrated reduced delivery to the liver by 50 percent and showed lower accumulation in the liver;
- Can be used at up to a log lower dose than traditional AAV vectors, due to increased efficiency.

"Research published by Broad Institute, and so far corroborated by Sarepta's own internal research, reinforces the potential of MyoAAV as a breakthrough next-generation approach in genetic medicine delivery," said Doug Ingram, president and chief executive officer, Sarepta. "The significantly improved efficiency of MyoAAV may unlock the ability to effectively deliver genetic medicine at as much as a log lower doses when compared to current AAVs, which could substantially reduce viral load and cost of goods in the future. As one of the leaders in the use of AAV-mediated genetic medicine to treat rare disease, we intend to push the science forward, and our license for MyoAAV is a quintessential example of that effort."

"At Sarepta, we are committed to the future of gene therapy and gene editing, and excited about the promise and potential of MyoAAV as a revolutionary approach in the pursuit of novel treatments for rare genetic disease," said Louise Rodino-Klapac, executive vice president, head of research and development and chief scientific officer, Sarepta. "The MyoAAV platform is noteworthy for its broad applicability across multiple disease states and will further advance the science of genetic medicine allowing for swift advancement of treatments into the clinic across a variety of conditions, including larger rare cardiac and neuromuscular indications."

Under the terms of the agreement, Sarepta will receive worldwide commercial license grants for five neuromuscular and cardiac indications, including Duchenne muscular dystrophy, plus exclusive options on additional targets. In addition to an upfront payment, Broad Institute is entitled to future royalties and milestone payments, details of which were not disclosed.

***About Sarepta Therapeutics***
Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on Twitter, LinkedIn, Instagram and Facebook.

***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.*

***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 parties' obligations and responsibilities under the agreement; Sarepta's right to receive worldwide commercial license grants for five neuromuscular and cardiac indications, plus exclusive options on additional targets; the potential benefits of the collaboration between Sarepta and Broad Institute; and the potential benefits of MyoAAV, including being a breakthrough next-generation approach in genetic medicine, and its significantly improved efficiency unlocking the ability to effectively deliver genetic medicine at as much as a log lower doses when compared to current AAVs, which could substantially reduce viral load and cost of goods in the future.*

*These forward-looking statements involve risks and uncertainties, many of which are beyond our control. Known risk factors include, among others: the expected benefits and opportunities related to the agreement 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 agreement 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; success in preclinical trials does not ensure that later clinical trials will be successful; 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; even if Sarepta's programs result in new commercialized products, Sarepta may not achieve the expected 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, 2021, 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 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, except as required by law.*

Source: Sarepta Therapeutics, Inc.

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