



## *Sarepta and StrideBio Announce Multi-target Strategic Collaboration to Advance Novel Gene Therapies*

*-- Agreement leverages StrideBio's novel, industry-leading, structure-driven capsid engineering platform and expands Sarepta's early stage gene therapy pipeline --*

*-- Sarepta granted an exclusive license to four CNS targets, on which StrideBio will lead early research and development inclusive of IND-enabling capsid and construct development --*

*-- Sarepta granted an exclusive option for four additional neuromuscular and CNS targets --*

Cambridge, Mass., and Research Triangle Park, N.C., Nov. 14., 2019 – Sarepta Therapeutics, Inc. (NASDAQ: SRPT), the leader in precision genetic medicine for rare diseases, and StrideBio, Inc., a leading developer of novel adeno-associated viral (AAV) based gene therapies, today announced the signing of a collaboration and license agreement to develop *in vivo* AAV-based therapies for up to eight central nervous system (CNS) and neuromuscular targets. Pursuant to the agreement, Sarepta is granted an exclusive license on selected targets to leverage StrideBio's novel, structure-driven capsid technology, intended to enhance specific tropism to tissues of interest and evade neutralizing antibodies. The parties also plan to focus on strategies intended to address re-dosing challenges in patients who have received AAV-delivered gene therapy. StrideBio will conduct all investigational new drug (IND) enabling research, development and manufacturing for the first four CNS targets, which are MECP2 (Rett syndrome), SCN1A (Dravet syndrome), UBE3A (Angelman syndrome), and NPC1 (Niemann-Pick). Additionally, Sarepta will have an exclusive option to four additional targets based on StrideBio's capsid technology.

StrideBio possesses an innovative and proprietary platform that is enabled by a deep knowledge of AAV structure and a unique approach to engineering capsids with novel functions, and was co-founded by Mavis Agbandje-McKenna, Ph.D., and Aravind Asokan, Ph.D., leading scientists in the field of AAV biology and gene therapy. StrideBio has created a growing portfolio of novel AAV capsids evolved in non-human primates that show reduced seroprevalence and potential for improved tropism to targeted tissues.

“With our partnership with StrideBio, Sarepta continues to build on its leadership position in gene therapies to treat rare diseases. We are excited to work with StrideBio and access its innovative AAV platform for next-generation capsids,” said Doug Ingram, Sarepta's President and Chief Executive Officer. “Our partnership with StrideBio expands our research portfolio by up to eight new targets and, through our strategic partnering approach that has our collaborator lead all IND-enabling research and development, ensures that we gain access to new technology and targets while not distracting Sarepta from its near-term priorities.”



“We are very excited to initiate this multi-target collaboration with Sarepta, an established leader in the development and commercialization of genetic medicines,” stated Sapan Shah, Ph.D., Chief Executive Officer, StrideBio. “This partnership will provide significant resources and expertise to enable StrideBio’s continued rapid expansion of our research and manufacturing platform, as well as accelerate the development of AAV gene therapies for multiple rare disease targets. We are looking forward to working together with Sarepta to bring novel treatments to patients as quickly as possible.”

### **Terms of Agreement**

Under the terms of the agreement, StrideBio will be responsible for AAV capsid development, non-clinical development and manufacturing of preclinical candidates to be selected for advancement into clinical studies. The parties will also share early clinical development activities for selected targets, with Sarepta responsible for late stage development and commercialization. A total of four initial targets are specified under the collaboration. StrideBio will receive a \$48 million upfront payment in the form of cash and Sarepta stock, in addition to significant future development, regulatory and commercial milestones for the four programs. StrideBio will also receive royalties on worldwide net sales of any commercial products developed through the collaboration. Sarepta has also obtained an exclusive option to expand the collaboration to include up to an additional four targets with an upfront payment of up to \$42.5 million along with future downstream milestone payments, while StrideBio has an option to obtain co-development and co-commercial rights to one of the collaboration targets. In addition, Sarepta has made a commitment to invest in StrideBio’s next financing round. Further financial terms were not disclosed.

### **About Sarepta Therapeutics**

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in gene therapies for Limb-girdle muscular dystrophy diseases (LGMD), MPS IIIA and other CNS-related disorders, totaling over 20 therapies in various stages of development. The Company’s programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is fueled by an audacious but important mission: to profoundly improve and extend the lives of patients with rare genetic-based diseases. For more information, please visit [www.sarepta.com](http://www.sarepta.com).

### **About StrideBio, Inc.**

StrideBio, Inc is a gene therapy company focused on creating and developing novel adeno-associated viral (AAV) therapies for rare diseases. Our **STR**ucture **I**nspired **DE**sign approach holds the potential to generate unique AAV capsids with improved characteristics including potency, tissue tropism, and ability to evade pre-existing antibodies. This powerful new approach has broad application, enabling gene addition, gene silencing and gene editing



modalities for a wide range of diseases, including rare genetic diseases. StrideBio is headquartered in Research Triangle Park, NC. Current investors include Hatteras Venture Partners, Takeda Ventures, UCB Ventures and Alexandria Real Estate Equities, Inc. For more information, please visit [www.stridebio.com](http://www.stridebio.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 the potential benefits of the collaboration between Sarepta and StrideBio, including enhancing specific tropism to tissues of interest, evading neutralizing antibodies, granting Sarepta access to new technology and targets, providing significant resources and expertise to enable StrideBio's continued rapid expansion of its research and manufacturing platform, and accelerating the development of AAV gene therapies for multiple rare disease targets; the potential of StrideBio's portfolio to improve tropism to targeted tissues; the parties' plan to focus on strategies intended to address re-dosing challenges in patients who have received AAV-delivered gene therapy; the terms of the agreement between Sarepta and StrideBio, including expected and future payments, future development, regulatory and commercial milestones, future royalties, options and Sarepta's commitment to invest in StrideBio's next financing round; and Sarepta's mission to profoundly improve and extend the lives of patients with rare genetic-based diseases.*

*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 StrideBio 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, and 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, 2018 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 2018 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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