

Sarepta Therapeutics Enters into Long-term Strategic Relationship with Aldevron for GMP-grade Plasmid in Support of Gene Therapy Development and Commercial Manufacturing Strategy

- -- Agreement provides Sarepta with committed capacity and dedicated manufacturing slots for GMP-grade plasmid production for its micro-dystrophin Duchenne muscular dystrophy (DMD) gene therapy program, as well as plasmid capacity for future gene therapy programs --
- -- Aldevron, a leading producer of plasmid DNA, proteins, mRNA and antibodies for the biotechnology industry, operates a 70,000 square foot facility in Fargo, North Dakota, the largest plasmid DNA GMP manufacturing facility in the world --

CAMBRIDGE, Mass., January 2, 2019 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases and Aldevron, the leading producer of custom nucleic acids, proteins, and antibodies for the biotechnology industry, announced today that they have entered into a long-term strategic relationship for the supply of plasmid DNA to fulfill Sarepta's needs for its gene therapy clinical trials and commercial supply. Under the terms of the agreement, Aldevron will provide GMP-grade plasmid for Sarepta's micro-dystrophin Duchenne muscular dystrophy (DMD) gene therapy program and Limb-girdle muscular dystrophy (LGMD) programs, as well as plasmid source material for future gene therapy programs, such as Charcot-Marie-Tooth, MPS IIIA, Pompe and other CNS diseases.

"One of our highest priorities is building a robust supply chain and scalable manufacturing that can accelerate and ensure robust patient access to our pipeline of promising gene therapies on an accelerated timeline," said Doug Ingram, Sarepta's president and chief executive officer. "Aldevron, one of the top plasmid DNA manufacturers in the world, is an important partner in fulfilling our strategic objectives. This agreement is anticipated to provide sufficient plasmid supply to support our ambitious development and commercial gene therapy objectives."

"Our greatest satisfaction comes in helping companies whose research is making an impact on the lives of patients and we are proud to partner with Sarepta, a company dedicated to extending and saving lives," said Michael Chambers, chief executive officer and co-founder of Aldevron. "Aldevron has made significant investments in people, processes and facilities to support the pre-clinical, clinical and commercial production of new, genetically-based therapies that have significant potential in



Founded in 1998, Aldevron supplies plasmid DNA and gene editing enzymes to biopharmaceutical researchers developing advanced gene-based medicines. The company employs a team of 270 across its three locations in Fargo, North Dakota, Madison, Wisconsin, and Freiburg, Germany. Its 70,000 square foot facility in Fargo is the largest plasmid DNA GMP manufacturing facility in the world.

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 5 Limb-girdle muscular dystrophy diseases (LGMD), Charcot-Marie-Tooth (CMT), MPS IIIA, Pompe 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.

About Aldevron

Founded in 1998, Aldevron supplies plasmid DNA and gene editing enzymes to biopharmaceutical researchers developing advanced gene-based medicines. Aldevron also provides recombinant biological products for veterinary and agriculture applications. Aldevron specializes in GMP manufacturing and is known for inventing the GMP-Source™ quality system. Company headquarters are in Fargo, N.D., with additional facilities in Madison, WI., and Freiburg, Germany. For more information, please visit www.aldevron.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 expected benefits and opportunities of the agreement between Sarepta and Aldevron; Sarepta's strategic objective to build a robust supply chain and scalable manufacturing that can accelerate and ensure robust patient access to Sarepta's pipeline of promising gene therapies on an accelerated timeline; the anticipation that the agreement with Aldevron will provide sufficient plasmid supply to support Sarepta's ambitious development and commercial gene therapy objectives;



Sarepta's product candidates having significant potential in transforming disease; 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 agreement with Aldevron may not be realized or may take longer to realize than expected; Sarepta's dependence on Aldevron to produce plasmid DNA to fulfill Sarepta's needs for its gene therapy clinical trials and commercial supply, including any inability on Sarepta's part to accurately anticipate product demand and timely secure manufacturing capacity to meet product demand, may impair the availability of products to successfully support various programs, including research and development and the potential commercialization of Sarepta's gene therapy product candidates; if Aldevron were to cease providing quality manufacturing and related services to Sarepta, and Sarepta is not able to engage appropriate replacements in a timely manner, Sarepta's ability to manufacture its gene therapy product candidates in sufficient quality and quantity would adversely affect Sarepta's various product research, development and commercialization efforts; if Aldevron fails to adhere to applicable cGMP and other applicable government regulations, or experiences manufacturing problems, Sarepta will suffer significant consequences, which could significantly delay or negatively impact the success of Sarepta's development efforts for its product candidates; Sarepta may not be able to successfully scale up manufacturing of its product candidates in sufficient quality and quantity or within sufficient timelines, or be able to secure ownership of intellectual property rights developed in this process, which could negatively impact the development of its product candidates; Sarepta's gene therapy programs 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; and even if Sarepta's gene therapy programs result in new commercialized products, Sarepta 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, 2017 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 2017 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.

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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