Sarepta Therapeutics Signs Long-term Strategic Investment and License Agreements with Lacerta Therapeutics, Gaining Rights to Multiple CNS-targeted Gene Therapy programs and Access to Important Gene Therapy Talent and Tools

-- Sarepta expands its presence in gene therapy and broadens its reach with license to up to three new CNS-targeted gene therapy programs, including exclusive rights to Lacerta's gene therapy candidate for Pompe Disease and options to two additional candidates --

-- Sarepta will make an equity investment of $30 million --

-- Lacerta is an AAV-based gene therapy company founded on technologies licensed from the University of Florida, ranked a top center of excellence in gene therapy research; its founders have led numerous clinical-stage gene therapy programs and made significant advances in and contributions to the gene therapy field --

CAMBRIDGE, Mass., August 8, 2018 (GLOBE NEWSWIRE) -- Sarepta Therapeutics, Inc. (NASDAQ: SRPT), a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases, announced today that it has made a strategic investment and entered into a license and option agreement with Lacerta Therapeutics, a gene therapy company using a constellation of proprietary adeno-associated virus (AAV) vector technologies to develop CNS-targeted treatments and lysosomal storage diseases. Sarepta expects to benefit from Lacerta's expertise in AAV-based CNS-targeted gene therapies, will gain access to Lacerta's capsid screening and proprietary OneBac manufacturing platform and process for the licensed products, and bolsters its pipeline to 11 gene therapy programs, with three CNS-focused programs from Lacerta.

"Today's investment with Lacerta bolsters Sarepta's position as a leader in precision genetic medicine and moves us forward on our mission is to deliver life-enhancing therapies to those living with underserved diseases and in so doing to become one of the most meaningful global genetic medicine companies in the coming few years," said Doug Ingram, Sarepta's president and chief executive officer.

Specifically, the Lacerta relationship provides Sarepta with the following:

- *Expansion of Sarepta's gene therapy pipeline to up to 11 unique candidates:* With this transaction, Sarepta has added up to three CNS gene therapy targets to its already significant pipeline of 20 programs in various stages of development, expanding the Company's presence in gene therapy and broadening its therapeutic focus into CNS-targeted therapies.

- *Access to world class talent:* Lacerta's founders, nine in all, whom are widely published (over 500 papers among them) in leading peer-reviewed journals, are highly regarded in gene therapy clinical research and have worked at leading centers across the United States, including University of Florida, Nationwide Children's Hospital, CHOP/University of Pennsylvania and Weill Medical College of Cornell.

Regarding Sarepta's license and option to three CNS gene therapy assets, Lacerta will manage the majority of pre-clinical development while Sarepta will lead clinical development and commercialization. Sarepta will owe development and sales-based milestones to Lacerta and pay single-digit royalties on net sales.

"Our co-founders have dedicated their careers to the development of AAV gene therapy platforms for the treatment of multiple diseases. Lacerta's mission is to advance these technologies to develop novel treatments for patients with CNS disorders," said Dr. Joseph Reddy, president and chief executive officer of Lacerta Therapeutics. "We are pleased to begin our collaboration with Sarepta Therapeutics, a gene therapy leader, as it represents a significant step in advancing Lacerta's treatments to the clinic," added Dr. Reddy.

About Sarepta Therapeutics

Sarepta Therapeutics is a commercial-stage biopharmaceutical company focused on the discovery and development of precision genetic medicine to treat rare neuromuscular diseases. The Company is primarily focused on rapidly advancing the development of its potentially disease-modifying DMD drug candidates. For more information, please visit www.sarepta.com.

About Lacerta Therapeutics

Lacerta Therapeutics is a clinical-stage gene therapy company using a constellation of proprietary adeno-associated virus (AAV) vector technologies to develop treatments for central nervous system and lysosomal storage diseases. We are advancing our clinical programs using proprietary capsid variants and a scalable vector manufacturing platform. Currently, Lacerta is focused on gene therapy solutions for

Pompe Disease, Sanfilippo Syndrome Type B, Aromatic L-amino acid decarboxylase deficiency, Neurodegenerative Proteinopathies, Spinocerebellar Ataxias and Glioblastoma. For more information, visit www.lacertatherapeutics.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 Sarepta's plan to invest $30 million in Lacerta; Sarepta's expectation to benefit from Lacerta's expertise in AAV-based CNS-targeted gene therapies; Sarepta gaining access to Lacerta's capsid screening and proprietary OneBac manufacturing platform and process for the licensed products; the investment with Lacerta bolstering Sarepta's position as a leader in precision genetic medicine and moving Sarepta forward on its mission to deliver life-enhancing therapies to those living with underserved diseases and in so doing to become one of the most meaningful global genetic medicine companies in the coming few years; the potential of the transaction to expand Sarepta's gene therapy pipeline to up to 11 unique candidates, including up to three CNS gene therapy targets; the transaction providing Sarepta with access to world class talent; Sarepta's plan to lead clinical development and commercialization of three CNS gene therapy assets; payments that Sarepta is expected to make under the agreements with Lacerta; Lacerta's mission to advance AAV gene therapy platforms and to develop novel treatments for patients with CNS disorders; and the collaboration with Sarepta representing a significant step for Lacerta in advancing Lacerta's treatments to the clinic.

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 transactions with Lacerta may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the collaboration with Lacerta 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 or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreements, including any inability by Sarepta to fulfill its financial commitments to Lacerta; and even if the agreement results 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 Securities and Exchange Commission (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.

Source: Sarepta Therapeutics, Inc.

Media and Investors:
Sarepta Therapeutics, Inc.
Ian Estepan, 617-274-4052
iestepan@sarepta.com
or
W2O Group
Rachel Hutman, 301-801-5540
rhutman@w2ogroup.com