Sarepta and Lysogene Announce Exclusive License Agreement for LYS-SAF302, a Late-stage Gene Therapy for the Treatment of MPS IIIA, and Grant of Option Rights to an Additional CNS Gene Therapy Candidate

-- Expands Sarepta's portfolio to as many as 14 gene therapy programs --

-- Mucopolysaccharidosis type IIIA (MPS IIIA), also called Sanfilippo syndrome type A, is a rare, severe and fatal inherited neurodegenerative lysosomal storage disorder --

-- The pivotal gene therapy study is scheduled to start by year-end 2018; trial to assess the efficacy of LYS-SAF302 in improving or stabilizing the neurodevelopmental status of MPS IIIA patients --

-- Sarepta receives full commercial rights to LYS-SAF302 in the U.S. and other markets outside of Europe, while Lysogene retains full commercial rights in Europe --

-- Lysogene will receive $15 million (€13m) on closing, with potential future LYS-SAF302 development, regulatory, and commercial milestone payments totaling approximately $125 million (€108 million) and sales-based royalties; and Sarepta will make an equity investment in Lysogene of $2.5 million (€2.2 million) at a 30% premium --

CAMBRIDGE, Mass. and PARIS, France, October 15, 2018 (GLOBE NEWSWIRE) – Sarepta Therapeutics, Inc. (NASDAQ:SRPT), a leader in precision genetic medicine for rare diseases, announced today it has signed a license agreement with Lysogene (FR0013233475 – LYS), a pioneering biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, for the development of a gene therapy, LYS-SAF302, to treat Mucopolysaccharidosis type IIIA (MPS IIIA). Under the terms of today's agreement, Sarepta will also have certain option rights to an additional CNS-targeted gene therapy candidate.

Under the terms of the license, Lysogene shall be responsible for completion of the pivotal trial, which is set to commence in the fourth quarter of 2018. Sarepta shall have exclusive commercial rights to LYS-SAF302 in the United States and all territories outside of Europe, and Lysogene will retain exclusive commercial rights to LYS-SAF302 in Europe. Sarepta will be responsible for global manufacturing of LYS-SAF302 and will supply Lysogene for its territory.

In 2018, Sarepta will make committed cash payments to Lysogene totaling $26 million (€22 million) plus the purchase of Lysogene equity for $2.5 million (€2.2 million). In 2019, Sarepta will pay Lysogene up to an additional $19 million (€16 million).

Payments from Sarepta to Lysogene for all items, if all milestones are met, would total approximately $125 million (€108 million) plus royalties. The financial terms of the agreement significantly extend Lysogene's cash runway, enabling the company to continue the development of its other assets.

"We stand together today with the MPS community and Lysogene in service of a common goal of developing what could be a transformative therapy for this cruel disease," stated Doug Ingram, Sarepta's president and chief executive officer. "As with our other therapies targeted to serious, life-altering genetic diseases, we share with Lysogene a sense of urgency and a deep commitment to see this program through to fruition. Toward that goal, Sarepta will leverage its expertise in rare disease therapies and gene therapy to bring LYS-SAF302 to the MPS community."

"This partnership with Sarepta, an innovative global leader in genetic diseases, is a major step forward for Lysogene in our commitment to bring a therapy to market to treat MPS IIIA patients," said Karen Aiach, Lysogene's chief executive officer and founder. "We believe that Sarepta is the ideal partner for LYS-SAF302 in the U.S. and other countries outside of Europe. We are proud of this important validation of our efforts to date and look forward to working together closely with Sarepta."

As part of this partnership, Sarepta is subscribing to an equity investment of $2.5 million (€2.2 million) at a 30% premium to the 5-day volume-weighted average share price, through the issuance of 950,606 ordinary shares, with the same rights as existing shares, under a capital increase with cancellation of preemptive rights under article L.225-138 of the French Commercial Code and the 22d resolution of Lysogene's Extraordinary General Meeting in June 2018. The issuance of these shares will result in dilution of 7.1% for existing Lysogene shareholders. The issuance of these shares does not require the publication of a prospectus submitted to the visa of the Autorité des Marchés Financiers in accordance with article 211-3 of the AMF General Regulations. Lysogene intends to apply the funds raised for general corporate purposes including the further development of LYS-GM101 for GM1 gangliosidosis.

Torreya acted as exclusive financial advisor to Lysogene.

About MPS IIIA and LYS-SAF302

MPS IIIA is a rare inherited neurodegenerative lysosomal storage disorder characterized by intractable behavioral problems and developmental regression resulting in early death. It is caused by mutations

in the SGSH gene, which encodes an enzyme called Heparan-N-sulfamidase necessary for heparan sulfate (HS) recycling in cells. The disrupted lysosomal degradation and resulting storage of HS and glycolipids such as gangliosides leads to severe neurodegeneration. MPS IIIA affects about 1 in 100,000 newborns and is inherited in an autosomal recessive pattern. There are currently no treatment options for patients. The pivotal gene therapy trial is scheduled to start by year-end 2018; trial to assess the efficacy of LYS-SAF302 in improving or stabilizing the neurodevelopmental status of MPS IIIA patients.

LYS-SAF302 is an AAV-mediated gene therapy, the goal of which is to replace the faulty SGSH gene with a healthy copy of the gene. LYS-SAF302 employs the AAVrh10 virus, chosen for its ability to target the CNS.

Proof-of-concept was established in MPS IIIA pre-clinical models demonstrating strong expression, broad distribution, and the ability of the compound to correct lysosomal storage defects by producing the missing enzyme. Safety data from an IND-enabling toxicity and a biodistribution GLP study showed that, at any dose level evaluated, LYS-SAF302 was not associated with unexpected mortality, change in clinical signs, body weight, behavior or macroscopic findings in the brain.

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 Limb-girdle muscular dystrophy (LGMD), Charcot-Marie-Tooth (CMT) and CNS-related disorders, reaching a total of over 20 therapies in various stages of development. The Company's programs span across several therapeutic modalities, including RNA, gene therapy and gene editing. Sarepta is poised to be the most meaningful precision genetic medicine company in the world and make a profound difference in the lives of patients suffering from rare neuromuscular diseases and other rare diseases. For more information, please visit www.sarepta.com.

About Lysogene

Lysogene is a gene therapy company focused on the treatment of orphan diseases of the central nervous system (CNS). The company has built a unique capability to enable a safe and effective delivery of gene therapies to the CNS to treat lysosomal diseases and other genetic disorders of the CNS. A pivotal clinical trial in MPS IIIA is expected to start by year end on 2018 and a phase 1-2 clinical trial in GM1 Gangliosidosis is in preparation, while we are currently collaborating with a major partner to define the strategy of development for the treatment of Fragile X syndrome, a genetic disease related to autism. For more information, please visit www.lysogene.com.

Sarepta 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 license agreement Sarepta has entered into with Lysogene for the development of a gene therapy, LYS-SAF302, to treat Mucopolysaccharidosis type IIIA (MPS IIIA), including the responsibilities of the parties, the expected purchase of Lysogene equity and Lysogene's expected use of proceeds, and the expected cash and milestone payments and royalties; the goal of the pivotal gene therapy trial and the plan to dose the first patient in the trial in the fourth quarter of 2018; the possibility of Sarepta having exclusive rights to LYS-SAF302 in the United States and all territories outside of Europe; the potential of LYS-SAF302 to be a transformative therapy for MPS; Sarepta's plan to leverage its expertise in rare disease therapies and gene therapy to bring LYS-SAF302 to the MPS community; the goal of LYS-SAF302 to replace the faulty SGSH gene with a healthy copy of the gene; and Sarepta being poised to be the most meaningful precision genetic medicine company in the world and make a profound difference in the lives of patients suffering from rare neuromuscular diseases and other rare 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 Lysogene 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, 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 even if commercialization of any product under the agreement is achieved, this may not result in any significant revenues to the parties; if the actual number of patients suffering from MPS IIIA is smaller than estimated, Sarepta's revenue and ability to achieve profitability may be adversely affected; 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.

Lysogene Forward-Looking Statements

This press release may contain certain forward-looking statements, notably on the ability of the Company to conclude a strategic transaction or a potential partnership for one or several of its product candidates. Although the Company believes that these forward-looking statements are based on reasonable assumptions, all statements other than statements of historical fact included in this press release about future events are subject to (i) change without notice, (ii) factors beyond the Company's control and (iii) the financial capabilities of the Company. These statements may include, without limitation, any statements preceded by, followed by or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can have," "likely", "reasonably", "should," "would," "could" and other words and terms of similar meaning or the negative thereof. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that could cause the Company's actual results, performance or achievements to be materially different from the expected results, performance or achievements expressed or implied by such forward-looking statements. A further list and description of these risks, contingencies and uncertainties can be found in the Company's regulatory filings with the French Autorité des Marchés Financiers, including in the 2017 registration document (Document de référence), registered with the French Autorité des Marchés Financiers on June 4, 2018, under number R. 18-047, and future filings and reports by the Company. Furthermore, these forward-looking statements are only as of the date of this press release. Readers are cautioned not to place undue reliance on these forward-looking statements. Except as required by law, the Company assumes no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future. If the Company updates one or more forward-looking statements, no inference should be drawn that it will or will not make additional updates with respect to those or other forward-looking statements.

This press release is for information purposes only, and the information contained herein does not constitute either an offer to sell, or the solicitation of an offer to purchase or subscribe securities of the Company in any jurisdiction, in particular in France. Similarly, it does not give and should not be treated as giving investment advice. It has no connection with the investment objectives, financial situation or specific needs of any recipient. It should not be regarded by recipients as a substitute for exercise of their own judgement. All opinions expressed herein are subject to change without notice. The distribution of this document may be restricted by law in certain jurisdictions. Persons into whose possession this document comes are required to inform themselves about and to observe any such restrictions.

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