



**Lysogene and Sarepta Therapeutics Announce Dosing of the First Patient in AAVance, a Phase 2/3 Clinical Trial Investigating LYS-SAF302, a Gene Therapy for the Treatment of MPS IIIA (Sanfilippo Syndrome Type A)**

-- Trial to assess efficacy on neurodevelopmental status of MPS IIIA patients --

CAMBRIDGE, Mass. and PARIS – Feb. 14, 2019, at 10:00pm CET – Lysogene (FR0013233475 – LYS), a pioneering biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, and Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, announced today that the first patient has been dosed in AAVance, a global Phase 2-3 clinical trial of LYS-SAF302, a gene therapy for the treatment of Mucopolysaccharidosis Type IIIA (MPS IIIA).

AAVance is a single-arm trial aimed at evaluating the effectiveness of a one-time delivery of a recombinant adeno-associated virus vector rh.10 carrying the N-sulfoglucosamine sulfohydrolase (SGSH) gene. MPS IIIA is caused by mutations in the SGSH gene, which is involved in producing an enzyme necessary for the breakdown and disposal of long chain sugar molecules. LYS-SAF302 is intended to deliver a functional copy of the SGSH gene and allow the brain to secrete the missing enzyme. The goal of the trial is to show improved or stabilized neurodevelopmental status of MPS IIIA patients. The trial will enroll 20 patients at eight sites in the U.S. and Europe. More information can be found on [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

“The first patient dosed in the AAVance trial is an important step in addressing this relentlessly progressing neurodegenerative disease. Our aim is to stabilize or improve the clinical status of patients with MPS IIIA by providing a permanent source of functional enzyme in the brain,” said Karen Aiach, Founder and Chief Executive Officer of Lysogene.

“MPS IIIA is a lethal neurological disease with debilitating symptoms for which there is currently no approved treatment,” said Principal Investigator Dr. Ronald Crystal, Chairman of the Department of Genetic Medicine and the Bruce Webster Professor of Internal Medicine at Weill Cornell Medicine, and a physician at NewYork-Presbyterian/Weill Cornell Medical Center, who is a paid consultant for Lysogene.

“It is a great motivation to know that the work we are doing here has the potential to make a life-changing difference to so many unfortunate children, and I am very much looking forward to further advancing this innovative therapy.”

“Every day is an opportunity to make progress in bringing transformative treatments to patients and today’s milestone is an important advancement toward that goal,” said Doug Ingram, President and Chief Executive Officer, Sarepta. “Sarepta is committed to working with Lysogene to advance this program with the greatest urgency on behalf of patients.”

In accordance with the Worldwide License and Collaboration Agreement signed between Lysogene and Sarepta, the dosing of the first patient with LYS-SAF302 in a Phase 2-3 clinical trial triggers milestone payments of USD18 million from Sarepta to Lysogene.

### **About MPS IIIA**

MPS IIIA is a rare inherited neurodegenerative lysosomal storage disorder affecting approximately 1 in 100,000 newborns. Inherited in an autosomal recessive pattern, it is 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. There are currently no treatment options for patients.

### **About LYS-SAF302**

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 central nervous system. 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. Sarepta holds exclusive commercial rights to LYS-SAF302 in the United States and markets outside Europe; and Lysogene maintains commercial exclusivity of LYS-SAF302 in Europe.

### **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](http://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 in partnership with Sarepta Therapeutics, Inc. is ongoing and a phase 1-2 clinical trial in GM1 Gangliosidosis is in preparation. Lysogene is also collaborating with an academic partner to define the strategy of development for the treatment of Fragile X syndrome, a genetic disease related to autism. [www.lysogene.com](http://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 design of the AAVance trial and the goal to show improved or stabilized neurodevelopmental status of MPS IIIA patients; the plan to enroll 20 patients at eight sites in the U.S. and Europe; Lysogene's goal to stabilize or improve the clinical status of patients with MPS IIIA by providing a permanent source of functional enzyme in the brain; the potential of LYS-SAF302 to make a life-changing difference for the children affected by MPS IIIA; Sarepta's commitment to work with Lysogene to advance LYS-SAF302 with the greatest urgency on behalf of patients; the number of patients suffering from MPS IIIA and the potential market opportunity of LYS-SAF302; 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 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, especially on the Company's progress of its phase 2-3 clinical trial. Although the Company believes its expectations 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," "objective", "project," "will," "can have," "likely," "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, uncertainties and other risks 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 Markets Authorities 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 has been prepared in both French and English. In the event of any differences between the two texts, the French language version shall supersede.

### **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](http://www.sarepta.com). We encourage investors and potential investors to consult our website regularly for important information about us.*

Source: Sarepta Therapeutics, Inc.

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