Press Release
16 April, 2018

Lysogene Reports First Quarter Financial Results and Provides Business Update

- Extended CNS pipeline with a collaboration agreement for the development of an AAV based gene therapy for Fragile X syndrome, the most common single gene cause of autism, a condition of high unmet medical need
- Completed all Investigational New Drug (IND)-enabling preclinical studies with positive results on efficacy and supporting toxicology for LYS-SAF302 in MPS IIIA. Pivotal Clinical trial now scheduled to start in the second half of 2018
- Progressed the preclinical studies for LYS-GM101 in GM1 Gangliosidosis
- Strengthened the management team with the appointment of Dr. Ralph Laufer (Teva, Merck Inc.) as Chief Scientific Officer

CAMBRIDGE, MA, U.S. and PARIS, France, – April 16, 2018, at 07:45am CEST – Lysogene (FR0013233475 – LYS), a leading biopharmaceutical company pioneering gene therapy technologies to treat central nervous system (CNS) diseases, today reports its cash position for the first quarter 2018 and provides a business update.

"We enter 2018 with significant momentum to achieve key clinical development and regulatory milestones across our portfolio of gene therapy products for neurological diseases," said Karen Aiach, Founder and Chief Executive Officer of Lysogene. *"This year, we expect to initiate our pivotal clinical trial in Mucopolysaccharidosis type IIIA (MPS IIIA) in the U.S. and Europe and pursue IND enabling studies for our GM1 Gangliosidosis (GM1) product. We are also delighted to have secured a collaboration agreement for the development of an AVV-based gene therapy for Fragile X syndrome, the most common genetic cause of autism, a condition of high unmet medical need."*

Cash position and revenues as of March 31, 2018

As of March 31, 2018, cash and cash equivalent amounted to €9.9 million[1] compared to €14.1 million on December 31, 2017. As expected, Lysogene did not generate revenues during the first quarter 2018.

[1] Unaudited and not subject to approval by the board of directors

Business Update

- Lysogene continues to strengthen its management team by attracting significant industry veterans with the appointment of Dr. Ralph Laufer as Chief Scientific Officer, effective May 2, 2018. Dr. Laufer is a highly experienced professional with a strong track record in drug discovery, preclinical development and CMC at Teva Pharmaceuticals and Merck Inc. He joins Lysogene's executive team, recently reinforced with Sophie Olivier as Chief Medical Officer (formerly at Wyeth), and Philippe Mendels-Flandre as Chief Operating Officer (formerly at Baxter/Baxalta).

- Lysogene has expanded its portfolio of programs, by entering into a collaboration to develop AAV-based gene therapies for the treatment of Fragile X syndrome, the most common inherited form of intellectual disability and autism spectrum disorder. Fragile X builds on Lysogene's existing expertise in CNS disorders and capitalizes on the company's clinical and manufacturing capabilities. Lysogene will provide more details on this new program in the near future.

LYS-SAF302, late stage clinical drug candidate for MPS IIIA progress and outlook

Lysogene continues to progress the preparation of its pivotal clinical program and recent developments include:

- Receipt of a positive opinion from the Paediatric Committee (PDCO) of the European Medicines Agency (EMA) for LYS-SAF302 Paediatric Investigation Plan (PIP) in January 2018. This important regulatory milestone confirmed the design of the proposed Phase 2-3 single arm clinical trial in children with MPS IIIA. Consequently, Lysogene is eligible for an additional 2-year marketing exclusivity extension in addition to the 10-year marketing exclusivity based on the EMA granted Orphan Drug Designation for LYS-SAF302.

- Completion of enrolment of Lysogene's International Pivotal Observational Study (SAMOS), serving as a comparator in the Phase 2-3 study, as agreed with the regulatory authorities in Europe and in the US. All patients in this study will complete their 12-month visit by 2Q 2018.

- Completion of all Investigational New Drug (IND)-enabling preclinical studies demonstrating positive results on efficacy and supporting toxicology.

- Start of the pivotal Phase 2-3 trial now planned for the second half of 2018, a change compared to initial expectations due to a manufacturing delay. Despite this delay, all plans to activate eight clinical sites in the U.S. and Europe for this trial are well underway and will ensure maximum patient access to this clinical trial and Lysogene's novel gene therapy treatment.

LYS-GM101, drug candidate for the treatment of GM1

- Lysogene's IND-enabling preclinical study program in GM1 Gangliosidosis is advancing as planned and we intend to report long-term results in the second half of 2018.

- Pre-IND meeting with the U.S. Food and Drug Administration is scheduled for the first half of 2018, as well as seeking Scientific Advice/Protocol Assistance from the EMA. These regulatory meetings will enable Lysogene to further define the optimal development plan for this critical, new, innovative therapy.

Next financial milestones:

- Shareholders' meeting in June, 2018
- Q2 2018 revenue and cash position expected on July 16, 2018 (after market close)

About Lysogene

Lysogene is a gene therapy company focused on the treatment of rare 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 in 2018, a phase 1-2 clinical trial in GM1 Gangliosidosis in 2019, while we are currently collaborating to define the clinical development path for the treatment of Fragile X syndrome. www.lysogene.com

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