Press Release
5 September 2019

A Pivotal Year 2019 for Lysogene's MPS IIIA Phase 2-3 Clinical Trial

- ***Phase 2-3 AAVance study in MPS IIIA fully on track with seven children already safely treated with LYS-SAF302***
- ***Completion of robust two-year natural history study in MPS IIIA to serve as control arm for Phase 2-3***
- ***Launch of PROVide, parallel study using home-based smart phone video capture documenting Phase 2-3 treated children's progression on hallmarks of the disease***

FOR IMMEDIATE RELEASE

PARIS – September 5, 2019 at 06:00pm – Lysogene (FR0013233475 – LYS), a pioneering biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, today announced major advancements, for their Sanfilippo syndrome type A (MPS IIIA) clinical program.

"Having launched our Phase 2-3 clinical trial in MPS IIIA, enrolled half of the intended study population and completed our two-year natural history study to be used as the control group, so far 2019 has been a year of significant progress. MPS IIIA is a devastating disease in young children, for which there is no currently approved treatment option. Lysogene, our partner Sarepta and the patient and physician commitment and investment to deliver potentially transformative therapies to these patients is unparalleled," **said Karen Aiach, Founder, Chairman and Chief Executive Officer.**

First half 2019 MPS IIIA clinical developments

Enrollment momentum in ongoing Phase 2-3 AAVance study in MPS IIIA. The AAVance study is now underway with seven patients treated, an additional three patients enrolled and is on track to achieve full inclusion of 20 patients by H1 2020. Significant enthusiasm from MPS IIIA patient and physician communities continues.

Completion of two-year natural history study in MPS IIIA. Rigorously collected data using robust assessment tools for the primary cognitive endpoint, will be pooled with previously published data, and serve as the control arm for the ongoing pivotal Phase 2-3 trial.

Launch of PROVide, an innovative approach to record and measure treatment effects post gene therapy in the Phase 2-3 study. As a company whose origins come from within the MPS IIIA community, Lysogene has always put patients and families at the heart of their therapy development. In this effort, Lysogene has designed a study that includes qualitative interviews with caregivers about change after treatment and home-based smart phone video capture documenting the children's progression on hallmarks of the disease. This will be a unique and valuable adjunct to the Phase 2-3 clinical efficacy data package intended to support LYS-SAF302 registration, pricing and reimbursement.

Lysogene will continue to communicate on LYS-SAF302 clinical progress throughout the coming months.

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 phase 2-3 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. In accordance with the agreements signed between Lysogene and Sarepta Therapeutics, Inc., Sarepta Therapeutics, Inc. will hold exclusive commercial rights to LYS-SAF302 in the United States and markets outside Europe; and Lysogene will maintain commercial exclusivity of LYS-SAF302 in Europe. Lysogene is also collaborating with an academic partner to define the development strategy for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

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Lysogene's forward-looking statements

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