Press Release
February 21st 2017

Lysogene Receives Orphan Drug Designation from EMA for LYS-GM101 for Treatment of GM1 Gangliosidosis

FOR IMMEDIATE RELEASE

PARIS, France, and CAMBRIDGE Mass. USA —February 21st, 2017—Lysogene, (the **"Company"** FR0013233475 – LYS) a leading, clinical-stage biotechnology company specializing in gene therapy for rare central nervous system diseases, today announces that the European Medicines Agency (EMA) has granted orphan drug designation to LYS-GM101, the Company's gene therapy drug candidate for treatment of GM1 Gangliosidosis (**GM1**).

The U.S. Food and Drug Administration also granted an orphan drug designation and a rare pediatric disease designation to LYS-GM101 earlier this year.

"The EMA Orphan Drug Designation for LYS-GM101 is a key regulatory milestone further validating the medical plausibility of our approach," stated Karen Aiach, Founder and Chief Executive Officer of Lysogene. *"This designation will further facilitate and accelerate clinical development of our treatment.* It *is good news for patients suffering from this severe neurodegenerative disease and we look forward to studying this therapy further as we approach our upcoming Phase I/II clinical trial (LYS-GM101) in 2018."*

LYS-GM101 is designed to replace a defective gene in the cells of GM1 patients, in order to allow for production of the functional enzyme and to prevent the progressive nature of the neurological damage caused by GM1 in humans.

About the Orphan Drug Designation (ODD)

An ODD by the EMA allows a pharmaceutical company to benefit from incentives from the EU to develop a medicine for a rare disease. Applications for ODD are examined by the Committee for Orphan Medicinal Products (**COMP**), which adopts an opinion that is forwarded to the European Commission (**EC**). The EC then decides whether to grant an orphan designation for the medicine in question within 30 days of receipt of the COMP opinion. Pharmaceutical companies that obtain ODD benefit from a number of incentives, including protocol assistance, a type of scientific advice specific for designated orphan medicines, and market exclusivity once the medicine is on the market. Fee reductions are also available, depending on the status of the sponsor and the type of service required

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About GM1

GM1 is an extremely severe, autosomal recessive disease caused by a mutation in the GLB1 gene encoding for the lysosomal acid beta-balactosidase (ßgal) enzyme. The resulting enzymatic deficiency leads to accumulation of GM1-ganglioside in cells. Clinical presentation is mainly neurological with rapidly progressive impairment (motor, cognitive and behavioral) leading to premature death, mostly in early childhood. It is a devastating disease for patients and families. There is currently no disease modifying treatment available.

About Lysogene

Lysogene is a clinical stage biotechnology company pioneering the basic research and clinical development of AAV gene therapy for CNS disorders with a high unmet medical need. Since 2009, Lysogene has established a solid platform and network, with lead products in Mucopolysaccharidosis Type IIIA and GM1 Gangliosidosis, to become a global leader in orphan CNS diseases. Lysogene has also obtained ODD by the EMA and FDA and rare pediatric designation by the FDA for its MPS IIIA program.

Lysogene is listed on the Euronext regulated market in Paris (ISIN code: FR0013233475)

For more information, visit www.lysogene.com.

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