# Lysogene Receives Rare Pediatric Disease Designation from FDA for LYS-GM101 *for Treatment of GM1 Gangliosidosis*

**FOR IMMEDIATE RELEASE**

**PARIS, France, and CAMBRIDGE, Mass., USA—January 18th, 2017—**Lysogene, a leading, clinical-stage biotechnology company specializing in gene therapy for rare central nervous system diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to LYS-GM101, the Company's drug candidate in development for patients with GM1 Gangliosidosis (GM1), a severe neurodegenerative disease. The designation of Rare Pediatric Disease status makes Lysogene eligible for a Rare Pediatric Disease Priority Review Voucher upon approval of LYS-GM101 by the FDA.

*"This Rare Pediatric Disease Designation for LYS-GM101, is Lysogene's second designation after the LYS-SAF302 designation for the treatment of MPS IIIA. It is an important validation of Lysogene's work in the rare disease space and is indicative of our commitment to achieving a meaningful impact on the lives of the patients affected by GM1 and their families*," said Karen Aiach, Founder and Chief Executive Officer of Lysogene. "*We look forward to continuing to advance this product candidate in our upcoming phase I/II clinical trial (LYS-GM101).*"

LYS-GM101 has the potential to replace the defective gene in the cells of GM1 patients, which will allow for the production of the functional enzyme and prevent the progressive nature of the neurological damage caused by GM1.

**About the Rare Pediatric Disease Designation**

The FDA defines a "rare pediatric disease" as a disease that affects fewer than 200,000 individuals in the U.S. primarily aged from birth to 18 years. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor with Rare Pediatric Disease Designation who receives an approval of a new drug application (NDA) or biologics license application (BLA) is eligible for a voucher that can be redeemed to obtain priority review for any subsequent marketing application. The Priority Review Voucher may also be sold or transferred an unlimited number of times.

**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 unique platform and network, with lead products in Mucopolysaccharidosis Type IIIA and GM1 Gangliosidosis, to become a global leader in orphan CNS diseases.

For more information, visit www.lysogene.com.

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