



# Lysogene Receives Orphan Drug Designation from FDA for LYS-GM101 *for treatment of GM1 Gangliosidosis*

## **FOR IMMEDIATE RELEASE**

**PARIS, France - February 2<sup>nd</sup>, 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 orphan drug designation to LYS-GM101, the company's gene therapy drug candidate for treatment of GM1 Gangliosidosis ("GM1").

*"FDA Orphan Drug Designation for LYS-GM101 is an important regulatory milestone, which will facilitate clinical development of LYS-GM101. It is good news for patients that we can further study this therapy as this is a severe neurodegenerative disease with unmet medical need,"* stated Karen Aiach, Founder and Chief Executive Officer of Lysogene. *"We look forward to starting our upcoming phase I/II clinical trial (LYS-GM101) by the end of the first half of 2018, in line with the timetable previously announced."*

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**

Orphan Drug Designation is granted by the FDA to novel therapeutics for diseases or conditions affecting fewer than 200,000 patients in the U.S. or greater than 200,000 patients if there is no reasonable expectation that the production cost of the drug will be covered by its sales. The designation allows the drug developer to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of the drug, as well as, in some cases, tax credits for clinical research costs, the ability to apply for annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

## **About GM1**

GM1 is an extremely severe, autosomal recessive disease caused by a mutation in the GLB1 gene encoding for the lysosomal acid beta-galactosidase (βgal) 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, to the best of the Company's knowledge, no disease modifying treatment available.

## **About Lysogene**

Lysogene is a clinical stage biotechnology company pioneering the basic research and clinical development of gene therapies that use vectors derived from adeno-associated viruses to treat rare and fatal central nervous system disorders in children, for which, to the best of the company's knowledge, there is currently no treatment. Since 2009, Lysogene has established a solid platform and extensive network, along with innovative products in MPS IIIA and GM1 Gangliosidosis, in order to become a global leader in gene therapies for rare and fatal central nervous system diseases.

For more information, visit [www.lysogene.com](http://www.lysogene.com).

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