**Press Release**
21 December 2017

# Lysogene Holds First Clinical Advisory Board Meeting in Mucopolysaccharidosis Type IIIA (MPS IIIA) and GM1 Gangliosidosis

- *Advisory Board comprised of world-renowned international gene therapy and lysosomal disease experts*
- *Discussed design of pivotal trial in MPS IIIA and clinical trial development plans in GM1*
- *Provided valuable insight concerning expectations of therapy and the urgent need to address the neurological component of MPS IIIA*
- *Clinicians support the feasibility of Lysogene's direct-to-CNS trial design and recruitment strategy in MPS IIIA*

**FOR IMMEDIATE RELEASE**

**PARIS, France, and CAMBRIDGE, MA, US – December 21, 2017, at 05:45pm CET –** Lysogene (FR0013233475 – LYS), a leading, biopharmaceutical company pioneering gene therapy technologies to treat central nervous system (CNS) diseases, today announced the first meeting of the Clinical Advisory Board (CAB), held on December 8, 2017 in New York (US).

The CAB is comprised of world-renowned international gene therapy and lysosomal disease experts and is chaired by Professor Roberto Giugliani (University of Rio Grande do Sul, Porto Alegre, Brazil) and Professor Frits Wijburg (Academisch Medisch Centrum (AMC), the Netherlands). The CAB was established to provide strategic advice to Lysogene on its clinical development programs and commercialization strategy for its orphan gene therapy candidates to treat CNS diseases, beginning with LYS-SAF302 for Mucopolysaccharidosis Type IIIA (MPS IIIA) and LYS-GM101 for GM1 Gangliosidosis (GM1) patients, for which there is currently no treatment.

Lysogene has obtained Orphan Drug Designation from the European Medicines Agency (EMA) and U.S. Food and Drug Administration (FDA) and Rare Pediatric Designation by the FDA for both programs.

At the meeting, the CAB confirmed that "Lysogene's Pivotal clinical trial [in MPS IIIA] appears to be in a unique and advantaged position because it entails a method of administration that is known to be more effective [in the CNS] than study medication administered intravenously. Additionally, Lysogene has selected clinical endpoints and assessment tools that have been agreed amongst experts for measuring clinically relevant outcomes."

CAB members were impressed with the quality of Lysogene's Natural History Study data in MPS IIIA, which will be used as the comparator for the LYS-SAF302 Pivotal trial as discussed with the FDA and EMA. The group agreed that the data confirm that the disease predominantly involves the CNS. Children with MPS IIIA suffer neurological symptoms, including developmental delay, difficulty sleeping, hyperactivity, and issues with motor function. Symptoms and severity may vary between children, and there is a need to compare treated children with matched controls using the Bayley Scales of Infant and Toddler Development third edition (BSID-III), the only tool to have international consensus and validation as appropriate[1]. The CAB proposed significant support in Lysogene's patient recruitment plan.

Leading international gene therapy and lysosomal disease U.S. and European centers will participate in the pivotal study of LYS-SAF302.

LYS-SAF302 is a rAAV vector serotype rh.10 carrying the gene coding for SGSH. This *in vivo* gene therapy offers the possibility of a one-time treatment by inserting a healthy copy of the SGSH gene and allowing the body to start making the missing enzyme, therefore slowing or halting disease progression. Lysogene's gene therapy is delivered directly to the CNS during a neurosurgical procedure. By delivering the missing SGSH gene, Lysogene believes MPS IIIA patients will be provided a permanent source of functional enzyme in the brain that reverses phenotypic abnormalities of CNS cells.

Lysogene holds an exclusive license for the use of the AAVrh.10 in MPS IIIA.

**About Lysogene**

Lysogene (www.lysogene.com) is a global biopharma leader in orphan CNS disease research and development. Lysogene has generated five non-cumulative years of clinical safety data to show the efficiency of a direct delivery route to the CNS with its initial gene therapy trial for MPS IIIA. Lysogene has recently completed the enrollment for the first multi-national observational study in MPS IIIA which will function as the non-concurrent control for the first pivotal trial for MPS IIIA in H1 2018. Lysogene also plans a clinical trial for GM1 Gangliosidosis in 2019.

Lysogene is listed on the Euronext regulated market in Paris (ISIN code: FR0013233475). For more information: www.lysogene.com.

[1] Van der Lee et al (2017) Cognitive endpoints for therapy development for neuronopathic Mucopolysaccharidoses: Results of a consensus procedure. Mol Genet Metab

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