Press Release
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Lysogene gets PIP Green Light from European Medicines Agency on MPS IIIA Pivotal Study; Paving Way for Clinical Trial Application Approvals

- *Paediatric Investigational Plan (PIP) positive opinion from EMA for LYS-SAF302, AAV Gene Therapy in Mucopolysaccharidosis Type IIIA (MPS IIIA)*
- *Pivotal trial is a key component of agreed PIP*
- *Significant step toward European Union (EU) marketing authorization application*
- *Successful PIP compliance check extends market exclusivity to 12 years at EU market authorization*

PARIS, France, and CAMBRIDGE, MA, US – February 6th, 2018, at 5.45pm CET – Lysogene (FR0013233475 – LYS), a leading, biopharmaceutical company pioneering gene therapy technologies to treat central nervous system (CNS) diseases, today announced that the Paediatric Committee (PDCO) of the European Medicines Agency (EMA) adopted a positive opinion regarding the company's Paediatric Investigation Plan (PIP) for its lead product candidate LYS-SAF302 in patients with mucopolysaccharidosis type IIIA (MPS IIIA).

The EMA regulations require a positive PIP opinion prior to commercialization of new medicines.

Early PIP approval for LYS-SAF302 confirms the appropriate design of Lysogene's phase 3, single arm clinical trial in children with MPS IIIA.

Additionally, the positive PIP opinion makes Lysogene eligible for an additional two-year marketing exclusivity extension on top of the ten-year marketing exclusivity based on the EMA Orphan Drug Designation.

"*The positive PIP opinion by the EMA's PDCO is a significant milestone toward submission of our Marketing Authorization Application following completion of the phase 3 trial*" said Sean O'Bryan, VP and Head of Regulatory Affairs and Quality Assurance at Lysogene. "*This is a significant step forward toward bringing a potentially transformative treatment to patients in need*".

MPS IIIA leads to infantile-onset neurodegeneration and early death for which there is currently no approved drug therapy. CNS manifestations predominate, in particular intellectual disability, progressive

loss of acquired skills, behavioral and sleep disturbance. The reduced lifespan is related to severe progressive neurological deterioration and not to the involvement of other organ systems.

In a completed Phase 1-2 clinical trial in children with MPS IIIA, Lysogene's first generation gene therapy, LYS-SAF301, exhibited a favorable safety profile over 5 years, and encouraging efficacy data.

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 worldwide license with REGENXBIO for the use of the AAVrh.10 in MPS IIIA.

About Lysogene

Lysogene is a gene therapy company focused on orphan CNS diseases, and a leader in MPSIIIA. 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 first gene therapy trial for MPS IIIA. The company has included 23 patients in the first multi-national observational study in MPS IIIA, which will function as the non-concurrent control for the pivotal trial in H1 2018. Lysogene also plans a clinical trial for GM1 Gangliosidosis in 2019. Both programs have orphan drug designation from the EMA and the U.S Food and Drug Administration (FDA) and rare pediatric designation from the FDA.
For more information: www.lysogene.com.

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