Press Release
5 September, 2018

Lysogene Announces FDA approval of IND Application to Initiate Phase 2-3 Clinical Trial in MPS IIIA

- **Novel, one-time, investigational gene therapy treatment for MPS IIIA focused toward preventing neurocognitive decline**
- **LYS-SAF302 product manufacture released to support the demands of phase 2-3 clinical trial**
- **Implementation of the clinical trial depends on the strengthening of the Company's financial capabilities, which is currently being pursued**

CAMBRIDGE, MA and PARIS, France, – September 5, 2018, at 08.30am CEST – Lysogene (FR0013233475 – LYS), a pioneering biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, announced today that the U.S. Food and Drug Administration (FDA) has granted an Investigational New Drug (IND) clearance to proceed in the U.S. with its international, Phase 2-3 (AAVance) clinical trial of LYS-SAF302 for the treatment of Mucopolysaccharidosis Type IIIA (MPS IIIA).

Lysogene has also submitted Clinical Trial Applications (CTA) in Europe.

"The IND clearance of the Phase 2-3 study of LYS-SAF302 in MPS IIIA represents a major milestone for the clinical trial planned in four leading U.S. sites and four European sites," said Karen Aiach, Founder and Chief Executive Officer. "The same design, implementation and operational conduct of the study has also been approved by the European Medicines Agency Pediatric Committee (PDCO), thus allowing a robust, rigorous and consistent multi-national approach".

"Following standing interactions with the FDA, we are pleased to announce full alignment with the Agency on the non-clinical, clinical, regulatory and manufacturing dimensions of our Phase 2-3 study in the MPS IIIA," added Sophie Olivier, M.D., Chief Medical Officer. "Also, the past months, were important from a manufacturing perspective resulting in the successful production of LYS-SAF302 and product release to support the demands of the phase 2-3 clinical trial".

LYS-SAF302 Pediatric Investigation Plan (PIP) received approval from the PDCO earlier this year. Additionally, LYS-SAF302 has received Orphan Drug Designation from the FDA and EMA as well as Rare Pediatric Disease Designation from the FDA. Leading international gene therapy and MPS centers plan to participate in the clinical trial (clinicaltrials.gov NCT03612869).

As previously announced, the company is currently looking for funding opportunities in order to extend its cash runway and launch the MPS IIIA clinical trial, notably through a strategic partnership or transaction, or potential sale of one or several products.

Lysogene's approach

MPS IIIA is a lethal neurological disease with debilitating symptoms for which there is currently no approved treatment. CNS manifestations predominate, in particular intellectual disability, progressive loss of acquired skills, severe sleep and behavior disorder.

LYS-SAF302 is an 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.

LYS-SAF302 is Lysogene's fully optimized second generation gene therapy program for MPS IIIA. The data build upon its completed five-year Phase 1-2 clinical study with LYS-SAF301. Furthermore, the selection of an optimally efficacious dose and of a specific cannula to deliver LYS-SAF302 directly to the CNS, where it is most needed, reinforce Lysogene's belief that LYS-SAF302 could potentially show substantial improved efficacy. Lysogene holds an exclusive worldwide license from REGENXBIO for the use of AAVrh.10 in MPS IIIA.

About Lysogene

Lysogene is a gene therapy company focused on the treatment of orphan diseases of the central nervous system (CNS). The company has built a unique capability to enable a safe and effective delivery of gene therapies to the CNS to treat lysosomal diseases and other genetic disorders of the CNS. A pivotal clinical trial in MPS IIIA and a phase 1-2 clinical trial in GM1 Gangliosidosis are in preparation, while we are currently collaborating with a major partner to define the strategy of development for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

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Forward looking statement

This press release may contain certain forward-looking statements. Although the Company believes its expectations are based on reasonable assumptions, all statements other than statements of historical fact included in this press release about future events are subject to (i) change without notice, (ii) factors beyond the Company's control and (iii) the financial capabilities of the Company. These statements may include, without limitation, any statements preceded by, followed by or including words such as "target," "believe," "expect," "aim," "intend," "may," "anticipate," "estimate," "plan," "project," "will," "can have," "likely," "should," "would," "could" and other words

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