PRESS RELEASE

Lysogene Receives FDA Clearance of Investigational New Drug Application to Initiate the Gene Therapy Clinical Trial in the US with LYS-GM101 for the Treatment of GM1 Gangliosidosis

- **Second country to authorize trial initiation after recent UK MHRA approval, further country approvals pending**
- **Trial enrollment expected to begin during the first half of 2021**

Paris, France — 12 February 2021 at 08:00 am CET — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today announces that the U.S. Food and Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for LYS-GM101, the company's gene therapy candidate for the treatment of GM1 gangliosidosis, a serious, pediatric, life threatening disease. LYS-GM101 builds on Lysogene's extensive experience in direct to CNS adeno-associated viral vector (AAV)-based gene therapy clinical development.

The IND clearance follows the recent clinical trial authorization granted by the MHRA in the United Kingdom. Lysogene intends to initiate its global, multi-center, single-arm, two-stage, adaptive-design clinical trial of LYS-GM101 in patients with a diagnosis of early or late infantile GM1 gangliosidosis. The clinical trial will include a safety phase and a confirmatory efficacy phase. The company intends to dose a total of 16 patients, with dosage of the first patient expected in the first half of 2021.

"We are very pleased to receive this IND clearance for LYS-GM101 which completes the MHRA approval received a few weeks ago. It represents a major milestone that marks our second CNS gene-therapy program to enter into a global clinical trial" said **Karen Aiach, Founder Chairman and Chief Executive Officer of Lysogene.** *"This IND clearance once again demonstrates our quality and timely execution, and our strong determination to bring new therapeutic solutions for diseases that currently have no treatment."*

Christine Waggoner, President and Co-Founder of Cure GM1 Foundation added: *"Children with GM1 gangliosidosis represent a clear unmet medical need and we are thrilled to see a new therapeutic option entering the clinic, as it brings tremendous hope to families and the entire GM1 gangliosidosis community."*

LYS-GM101 ('adeno-associated viral vector serotype rh.10 expressing beta-galactosidase') received orphan drug designation for the treatment of GM1 gangliosidosis in the European Union and in the US in 2017, as well as Rare Pediatric Disease designation in the US in 2016.

Leading international gene therapy and Lysosomal Storage Disease centers plan to participate in the clinical trial (NCT04273269).

Lysogene is also funding a GM1 gangliosidosis natural history study being conducted by Casimir Trials to collect prospective and/or retrospective videos of children doing certain everyday tasks and behaviors in infantile and juvenile GM1 gangliosidosis (NCT04310163).

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 delivery of gene therapies to the CNS to treat lysosomal diseases and other genetic disorders of the CNS. A phase 2/3 clinical trial in MPS IIIA in partnership with Sarepta Therapeutics, Inc. is ongoing. An adaptative clinical trial in GM1 gangliosidosis is in preparation. In accordance with the agreements signed between Lysogene and Sarepta Therapeutics, Inc., Sarepta Therapeutics, Inc. will hold exclusive commercial rights to LYS-SAF302 in the United States and markets outside Europe; and Lysogene will maintain commercial exclusivity of LYS-SAF302 in Europe. Lysogene is also collaborating with an academic partner to define the strategy of development for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

Forward Looking Statement

This press release may contain certain forward-looking statements, especially on the Company's progress of its clinical trials and cash runway. 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, (iii) clinical trial results, (iv) increased manufacturing costs and (v) potential claims on its products. 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," "objective", "project," "will," "can have," "likely," "should," "would," "could" and other words and terms of similar meaning or the negative thereof. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that could cause the Company's actual results, performance or achievements to be materially different from the expected results, performance or achievements expressed or implied by such forward-looking statements. A further list and description of these risks, uncertainties and other risks can be found in the Company's regulatory filings with the French Autorité des Marchés Financiers, including in the 2019 universal registration document, registered with the French Markets Authorities on April 30, 2020, under number D.20-0427, and future filings and reports by the Company. Furthermore, these forward-looking statements are only as of the date of this press release. Readers are cautioned not to place undue reliance on these forward-looking statements. Except as required by law, the Company assumes no obligation to update these forward-looking statements publicly, or to update the reasons actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future. If the Company updates one or more forward-looking statements, no inference should be drawn that it will or will not make additional updates with respect to those or other forward-looking statements.

This press release has been prepared in both French and English. In the event of any differences between the two texts, the French language version shall supersede.

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