PRESS RELEASE

Lysogene Receives MHRA and Research Ethics Committee Approvals to Initiate the Gene Therapy Clinical Trial in the UK with LYS-GM101 for the Treatment of GM1 Gangliosidosis

- **LYS-GM101 is Lysogene's second CNS gene therapy program to enter clinic**
- **Trial enrollment expected to begin during the first half of 2021**

Paris, France — 11 January 2021 at 08:00am — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today announces that it has received MHRA and Research Ethics Committee approvals to initiate the gene therapy clinical trial in the United Kingdom with LYS-GM101 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.

This is the first regulatory authorization to initiate this 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 company expects to dose the first patient in the first half of 2021.

"This trial builds on Lysogene's considerable expertise for AAV gene therapy and clinical development. Since the most damaging effects of GM1 gangliosidosis occur in the brain and spinal cord, LYS-GM101 will be administered into a cerebrospinal fluid-filled space at the back of the head called the cisterna magna so that enough therapy reaches those tissues. I expect the study to provide us with data demonstrating the transformative potential of LYS-GM101" said **Karen Aiach, Founder Chairman and Chief Executive Officer of Lysogene.** *"LYS-GM101 is our second gene therapy program entering the clinical phase after LYS-SAF302 for MPS IIIA, followed by a discovery stage pipeline, including gene therapy candidates for Fragile X syndrome, Gaucher and Parkinson's disease."*

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 the 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 a safe and effective 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 and a phase 1/3 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.

About Manchester University NHS Foundation Trust

Manchester University NHS Foundation Trust is one of the largest NHS trusts in England and a leading provider of specialist healthcare services. Its nine hospitals are home to hundreds of world class clinicians and academic staff committed to finding patients the best care and treatments. More information is available at www.mft.nhs.uk.

Forward Looking Statement

This press release may contain certain forward-looking statements, especially on the Company's progress of its phase 2-3 clinical trial 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.

Contacts

Stéphane Durant des Aulnois
Chief Financial Officer
stephane.durant-des-aulnois@lysogene.com
+ 33 1 41 43 03 99