PRESS RELEASE

Lysogene Announces First Patient in the United States Dosed with LYS-GM101 Investigational Gene Therapy for the Treatment of GM1 Gangliosidosis

- **Second patient treated in the global adaptative-design clinical trial with LYS-GM101**
- **Ongoing enrollment of a total of 16 patients in the UK, US and France**

Paris, France — 30 August 2021 at 08:00 am CET — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today announced dosing of the first patient in the United States with LYS-GM101 investigational gene therapy at CHOC Hospital (CHOC) in a global adaptative-design clinical trial (NCT04273269) for the treatment of GM1 gangliosidosis.

This trial is an interventional, multi-center, single-arm, two-stage adaptive-design study evaluating the intracisternal delivery of a recombinant adeno-associated virus vector serotype rh.10 (AAVrh.10) carrying the human β-galactosidase gene (GBL1). The clinical trial includes a safety phase and a confirmatory efficacy phase. The trial will enroll 16 patients with a diagnosis of early or late infantile GM1 gangliosidosis at sites in the US and Europe. More information can be found on www.clinicaltrials.gov/ct2/show/NCT04273269.

GM1 gangliosidosis is a fatal autosomal recessive disease caused by mutations in the GLB1 gene leading to accumulation of GM1 ganglioside in neurons resulting in progressive neurodegeneration. No treatment has been approved so far for this disease.

"We are pleased with the rapid progress we are making with our teams in our effort to bring a new therapeutic solution to patients and families affected by GM1 gangliosidosis" said **Karen Aiach, Founder Chairman and Chief Executive Officer of Lysogene**. *"By promptly opening a new site in the US and dosing the second patient we demonstrate our quality and timely execution despite a challenging and persistent Covid-19 environment."*

"GM1 gangliosidosis is progressive, neurodegenerative disorder affecting children, for which no treatment is currently available. The many people in our gene therapy team are honored and delighted to be involved in this important clinical trial" said **Dr. Raymond Wang, metabolic disorders specialist and director of the Foundation of Caring Lysosomal Storage Disorder Program, CHOC.**

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.

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 (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 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 adaptive clinical trial in GM1 gangliosidosis is ongoing. 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 has also entered into an exclusive worldwide license agreement with SATT Conectus for a gene therapy candidate for the treatment of the 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 2020 universal registration document, registered with the French Markets Authorities on April 12, 2021, under number D.21-0296, 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