PRESS RELEASE

Lysogene Announces FDA Fast Track Designation for LYS-GM101 Gene Therapy for the Treatment of GM1 Gangliosidosis

Paris, France — 08 July 2021 at 06:00 pm CET — Lysogene (FR0013233475 – LYS), a gene therapy platform Company targeting central nervous system (CNS) diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation to its LYS-GM101 program, which is being studied in the recently initiated global adaptative-design clinical trial for the treatment of GM1 gangliosidosis. 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.

Karen Aiach, Founder, Chairman and Chief Executive Officer of Lysogene commented: *"We are pleased that FDA has granted Fast Track designation to LYS-GM101, as it underscores its potential to improve neurocognitive deficits in children with GM1 gangliosidosis, a lethal neurological disease for which there is currently no treatment. We have dosed the first patient last month and recruitment is on track, with completion of the first cohort expected by first quarter next year".*

The Fast Track program facilitates the development and accelerates the review of new drugs for serious conditions, which have the potential to address unmet medical needs. The purpose is to expedite the availability of new treatment options for patients. A product that receives Fast Track designation is eligible for more frequent interactions with FDA, potential eligibility for accelerated approval, priority review, and rolling Biologics License Application (BLA) review.

"This Fast Track designation demonstrates the regulators' sustained interest in Lysogene's cutting edge gene therapy program. It complements the Rare Pediatric Disease and Orphan Drug designations granted by the FDA in 2016 and 2017" added **Marie Deneux, Chief Regulatory Officer of Lysogene**. *"We look forward to working closely with the agency in our effort to develop LYS-GM101 as the potential first drug for the treatment of GM1 gangliosidosis."*

The trial is an interventional, multi-center, single-arm, two-stage adaptive-design study evaluating an intracisternally delivered recombinant adeno-associated virus vector serotype rh.10 (AAVrh.10) carrying the human β-galactosidase gene (GBL1) (NCT04273269). 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.

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.

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