# PRESS RELEASE

# Lysogene awarded Innovation Passport by the UK MHRA for the GM1 gangliosidosis program

- **First step in the recent UK MHRA initiative to accelerate the development and access to innovative medicines in the UK**
- **New designation acknowledges again regulator's position on the potential of Lysogene's LYS-GM101 program for patients with GM1 gangliosidosis**

**Paris, France — 20 December 2021 at 08:00 am CET —** Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform Company targeting central nervous system (CNS) diseases, announced today that the Medicines and Healthcare products Regulatory Agency (MHRA) has awarded LYS-GM101 investigational gene therapy an 'Innovation Passport' for the treatment of GM1 gangliosidosis under the United Kingdom's (UK) Innovative Licensing and Access Pathway (ILAP). The decision was made by the ILAP Steering Group, which is comprised of representatives from MHRA, National Institute for Health and Care Excellence (NICE), Scottish Medicines Consortium (SMC), All Wales Therapeutics and Toxicology Centre (AWTTC) and representatives from the ILAP Patient and Public Reference Group.

The Innovation Passport is the entry point to ILAP, designed to accelerate the development and access to promising medicines in the UK, thereby facilitating and improving patient access to new medicines.

*"We are very pleased to have been granted the Innovation Passport,"* stated **Marie Deneux, Chief Regulatory Officer & QA of Lysogene.** *"Ensuring LYS-GM101 reaches patients with GM1 gangliosidosis is one of Lysogene's key priority. We will now work together with the MHRA and its partners to create a product-specific target development profile (TDP) for LYS-GM101, with the goal of facilitating early patient access to the gene therapy treatment"*.

ILAP was introduced by MHRA in January 2021 and is aimed at facilitating development of and patients' access to promising innovative medicinal products. The regulatory pathway involves close engagement with other stakeholders, including patient representatives. Other benefits of ILAP include a 150-day accelerated assessment, rolling review and a continuous benefit risk assessment.

**About LYS-GM101**

LYS-GM101 is currently in clinical development. P1-GM-101 (NCT04273269) is an open-label, 2 stage adaptive clinical trial with natural history data as external control, conducted at four clinical sites, including one site in the UK. Within P1-GM-101, a video outcome and parent interview sub-study is being conducted, which data will be complementary to the clinical endpoints. In addition, a collection of natural history data using interview and video assessments captured at home by parents/caregivers of their child with GM1 gangliosidosis was launched in early 2020 and is ongoing (NCT04310163). LYS-GM101 has also been granted orphan designation in the European Union (EU) and the United States (US), and rare pediatric disease designation and fast track designation in the US.

**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, (v) potential claims on its products, and (vi) a modification of the terms of its agreements with Sarepta Therapeutics. 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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