PRESS RELEASE

Lysogene Provides Clinical Update on AAVance Phase 2/3 Clinical Trial with Gene Therapy LYS-SAF302 for the Treatment of MPS IIIA at the 25th Annual ASGCT Meeting

- **Promising efficacy data in young patients with persistent increase or stabilization in 3 main developmental domains (cognition, motor and language) in all patients enrolled under the age of 30 months**
- **Final and comprehensive results of the 24 months pivotal data for the main cohort are expected in Q3 2022 along with the results of the ancillary PROVide video study**
- **Forthcoming discussions with regulatory authorities on next steps**

Paris, France — 18 May 2022 at 8:00 am CET — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, provided updated clinical data from the ongoing AAVance phase 2/3 clinical trial with the investigational gene therapy LYS-SAF302 for the treatment of MPS IIIA (NCT03612869) during the 25th Annual Meeting of the ASGCT (American Society for Gene & Cell Therapy) in Washington, DC.

The preliminary results from the AAVance study evaluating LYS-SAF302 in patients with Sanfilippo syndrome type A (MPS IIIA) who have been followed for at least two years demonstrated improvement, stabilization, or slowing down of decline in cognitive developmental age (DA) in about half of the patients. Notably, in all 6 patients enrolled under the age of 30 months, a persistent increase or stabilization of the cognitive, language and motor domains of the BSID-III was observed in the 24 months period post-treatment. Two

(2) of these 6 patients, including 1 patient over 4 years of age, have already exceeded by several months the highest cognitive DA (35 months) observed in the natural history cohort. The 2 patients reached 41 and 42 months of cognitive DA in the 24 months period post-treatment with an increase of 25 and 17 months compared to their baseline, respectively.

These results, which suggest that younger patients are more responsive to the gene therapy treatment, need to be corroborated and confirmed by the final statistical analyses. They will also include secondary behavioral and imaging endpoints as well as data from the observational study in children treated with LYS-SAF302, using video and parent interviewing (also called the Patient Reported Outcome Videos [PROVide] study). Full results will be available in Q3 2022.

Considering these promising efficacy signals and the observed general trend towards stabilization and/or decrease of the total white matter lesional volume at injection sites, from 12 months post-treatment onwards, with no apparent clinically significant consequences, the company will plan further discussions with regulatory authorities to determine next steps.

Karen Aiach, Founder and CEO of Lysogene said: *"We are pleased to share these promising efficacy data in patients treated with LYS-SAF302, after a 2-year follow-up period. The data show encouraging signals of efficacy on cognitive, language and motor development in young patients. We look forward to confirming and supporting these observations by analyzing all the data collected since the beginning of the study, including secondary behavioral and imaging endpoints and real-life behaviors assessed through the video study. Following this full analysis expected in Q3 this year, we will have all the necessary elements to discuss the path forward with the regulatory authorities."* **Karen Aiach added:** *"These promising data will support our effort to raise funds, which remains Lysogene's top priority. In a context of challenging market conditions for innovative life sciences companies, Lysogene is reviewing on an ongoing basis all options available to extend its cash runway."*

AAVance is an open-label single-arm multicenter trial aimed at evaluating the effectiveness of a one-time intracerebral delivery of a recombinant adeno-associated virus vector rh.10 carrying the N-sulfoglucosamine sulfohydrolase (SGSH) gene (LYS-SAF302, olenasufligene relduparvovec) in children with MPS IIIA. MPS IIIA is caused by mutations in the SGSH gene, which produces an enzyme involved in the catabolism of heparan sulfate. LYS-SAF302 is intended to deliver a functional copy of the SGSH gene and allow the brain to secrete the missing enzyme.

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 disorders of the CNS. A phase 2/3 clinical trial in MPS IIIA is ongoing. An adaptive clinical trial in GM1 gangliosidosis is also ongoing. Lysogene is also developing an innovative AAV gene therapy approach for the treatment of Fragile X syndrome, a genetic disease related to autism. The Company also entered into an exclusive worldwide license agreement with Yeda, the commercial arm of the Weizmann Institute of Science, for a novel gene therapy candidate for neuronopathic Gaucher disease and Parkinson disease with GBA1 mutations. 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. 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 2021 universal registration document, registered with the French Markets Authorities on April 19, 2022, 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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