PRESS RELEASE

Lysogene Reports Positive Biomarker data with LYS-SAF302

- **Decrease in heparan sulfate concentration in cerebrospinal fluid**
- **Decrease in GM2 and GM3 ganglioside in cerebrospinal fluid**
- **Full data to be presented at the WORLDSymposium™ 2021**

Paris, France — 28 December 2020 at 08:00am — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today reports positive biomarker data from the ongoing AAVance clinical trial with LYS-SAF302 for the treatment of MPS IIIA (NCT03612869).

Changes in heparan sulfate (HS) concentration in cerebrospinal fluid (CSF) are being monitored in patients treated with LYS-SAF302 to provide evidence of in vivo biological activity of the drug and demonstrate proof of concept.

First results show reductions in the concentration of HS in the CSF of all nine patients analyzed so far, at 6 and 12 months after treatment with LYS-SAF302, relative to pre-treatment values. Average reductions were highly statistically significant. In contrast, there were no statistically significant changes in serum HS concentrations following treatment with LYS-SAF302. These results are consistent with the hypothesis that LYS-SAF302 leads to a reduction of HS entering the CSF from the brain parenchyma, with little or no effect on HS-derived oligosaccharides entering the CSF from extra-parenchymal sources, such as choroid plexus or blood.

Furthermore, statistically significant reductions in the secondary storage products GM2 and GM3 ganglioside, which are thought to be possible contributors to neuronal damage in lysosomal storage diseases, were observed in the CSF of treated patients, relative to pre-treatment values.

Ralph Laufer, Chief Scientific Officer at Lysogene said: "*These first very encouraging results provide evidence of positive biological responses to LYS-SAF302 treatment in patients enrolled in the AAVance trial. The CNS-specific reduction of the disease biomarker HS is consistent with Lysogene's unique intra-parenchymal mode of administration, which enables us to deliver the drug directly into the brain, where accumulation of HS causes the predominantly neurological manifestations of MPS IIIA. The reduction in secondary storage products, GM2 and GM3 ganglioside, confirms the biological activity and therapeutic potential of LYS-SAF302. We look forward to confirming these results in additional patients.*"

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.

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.

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