Press Release
15 October 2020

Lysogene provides update on the AAVance Clinical Trial Evaluating LYS-SAF302 in Patients with MPS IIIA

Paris, France — 15 October 2020 at 08:00am — Lysogene (FR0013233475 – LYS), a Phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today announced that a patient has passed away in the AAVance clinical trial (NCT03612869), a global Phase 2/3 gene therapy trial for the treatment of Mucopolysaccharidosis Type IIIA (MPS IIIA, also known as Sanfilippo syndrome type A). MPS IIIA is a serious, life-threatening, inherited neurodegenerative lysosomal storage disorder characterized by intractable behavioral problems and developmental regression resulting in early death.

The immediate cause of death is currently unknown and additional information is being collected. At this time, there is no evidence that the event is linked to the study drug administration.

Lysogene is diligently following per study protocol the 18 patients who have been treated in the clinical trial.

Lysogene is profoundly saddened by the passing of this child and extends its deepest sympathies to the family. The company remains committed to the LYS-SAF302 development program and the Sanfilippo patient community. The company plans to provide further information on the LYS-SAF302 program based on both ongoing data collection and future regulatory status updates.

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 on a gene therapy approach 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, forecasts and estimates with respect to Lysogene's clinical trials, clinical trial data releases, clinical development plans, anticipated future activities and cash runway of Lysogene. 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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