Press Release
01 July 2020

Lysogene announces a research collaboration with the Weizmann Institute of Science

- **Novel AAV gene therapy approach for neuronopathic Gaucher disease and Parkinson disease with GBA1 mutations**

Paris, France — 01 July 2020 — Lysogene (FR0013233475 – LYS), a Phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today announced that the company has entered into a collaborative research agreement with Yeda Research and Development Co Ltd, the commercial arm of the Weizmann Institute of Science.

The agreement involves collaboration between Lysogene and the lab of Prof. Anthony Futerman at the Weizmann Institute of Science, with the aim of developing a novel AAV gene therapy approach for neuronopathic Gaucher disease, Parkinson disease, and other diseases associated with mutations of the GBA1 gene. Under the terms of the agreement, Lysogene will provide expertise in AAV vector design and production, while the lab of Prof. Futerman will provide glucocerebrosidase variants with enhanced biological properties and conduct biological proof and concept studies. Lysogene has an exclusive option to license the program.

Ralph Laufer, Chief Scientific Officer at Lysogene said: *"We are thrilled to start this research collaboration with Prof. Futerman, a leading expert in the field of sphingolipid biology. The Weizmann Institute is one of the world's leading multidisciplinary research institutes, and the source of numerous groundbreaking medical discoveries and technological applications. Lysogene is developing gene therapy approaches for monogenic neurological disorders, including neuronopathic lysosomal storage diseases. This collaboration is a perfect fit with Lysogene's strategy, providing the opportunity to develop a novel therapy for a rare lysosomal disease, with the potential to expand into neurological diseases with much larger patient populations, such as Parkinson."*

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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