



Lysogene Enters 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

- **Lysogene exercises its option after a conclusive research collaboration period**

Paris, France — 12 April 2022 at 06:00 pm CEST — Lysogene (FROO13233475 – LYS), a phase 3 gene therapy platform Company targeting central nervous system (CNS) diseases, announced today that it has exercised its option to enter into an exclusive worldwide license agreement with Yeda Research and Development Co Ltd, the commercial arm of the Weizmann Institute of Science, for the development and commercialization of a gene therapy candidate for the treatment of neuronopathic Gaucher disease and Parkinson disease (PD) with GBA1 mutations.

Since 2020, Lysogene has been collaborating with 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. Lysogene has provided expertise in AAV vector design and production, while Prof. Futerman's lab provided glucocerebrosidase variants with enhanced biological properties. After approximately 20 months of successful collaboration, Lysogene decided to exercise its option to license the program on the basis of positive preliminary biological proof of principle studies.

Under the terms of the agreement, Lysogene will be responsible for the preclinical and clinical development, manufacturing, regulatory activities, and commercialization of the drug

candidate, globally. Yeda Research and Development Co Ltd will be eligible for royalties and other payments.

Ralph Laufer, CSO of Lysogene, commented: *"We are thrilled to add to our R&D pipeline a new promising gene therapy asset, which addresses high unmet medical needs in Gaucher disease, as well as broader indications, such as PD-GBA. The first results obtained by Prof. Anthony Futerman's team are promising and we look forward to expanding them in additional preclinical studies. The cost of these preclinical studies will not affect our cash runway. We continue to replenish our early-stage pipeline to position Lysogene as a key player in the field of gene therapy translational science."*

"We are excited to expand our successful research collaboration with Lysogene based on the novel research by Prof. Futerman and his colleagues," said **Elik Chapnik, Sr. Director of Business Development at Yeda**. *"We are confident that this new licensing deal has the potential to bring transformative therapeutic options to patients suffering from genetically-defined CNS diseases with significant unmet need, such as neuronopathic Gaucher disease."*

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. www.lysogene.com.

About Yeda

Yeda Research and Development Company Ltd. is the commercial arm of the Weizmann Institute of Science. Yeda currently manages approximately 500 unique patent families and has generated the highest income per researcher compared to any other academic technology transfer operation worldwide. Through the years, Yeda has contributed to the commercialization of a number of groundbreaking therapies, such as Copaxone, Rebif, Tookad®, Erbitux®, Vectibix®, Protrazza®, Humira®, and recently the CAR-T cancer therapy Yescarta®. For more information, visit www.yedarnd.com/.

About the Weizmann Institute of Science

The Weizmann Institute of Science in Israel is one of the world's top-ranking multidisciplinary research institutions. Noted for its wide-ranging exploration of the natural and exact sciences, Weizmann Institute's scientists are advancing research on the human brain, artificial intelligence, computer



science and encryption, astrophysics and particle physics, and are tackling diseases such as cancer, while also addressing climate change through environmental, ocean, and plant sciences.

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 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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