

PRESS RELEASE

Lysogene Provides Medical Update

- **Third patient treated in the global clinical trial with LYS-GM101 gene therapy program in GM1 gangliosidosis**
- **Lysogene appoints seasoned Chief Medical Officer, Dr Marie Trad**
- **Two oral presentations scheduled at WORLDSymposium™ 2022 on new LYS-SAF302 biomarker data and preliminary LYS-GM101 safety data**

Paris, France — 07 February 2022 at 08:00 am CET — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, today provides a medical update.

First, Lysogene has dosed the first patient in France with LYS-GM101 investigational gene therapy at the Armand Trousseau Hospital (AP-HP). This is the third patient treated with LYS-GM101 in the global adaptive-design clinical trial (NCT04273269) in children with GM1 gangliosidosis. The enrollment of the 4th patient of the safety cohort is ongoing, following which Lysogene will initiate the treatment of the 12 patients of the efficacy confirmatory cohort.

Second, Lysogene has appointed a seasoned Chief Medical Officer, Dr. Marie Trad, effective February 1st, 2022. With over thirty years of clinical and pharmaceutical development experience in the field of neurologic diseases, Dr. Marie Trad, M.D., brings her in-depth knowledge and expertise to lead Lysogene's gene therapy pipeline global clinical development from early stages throughout the lifecycle up to post-marketing stages. Dr. Trad is a board-certified neurologist and a qualified neuroradiologist from the Pierre and Marie Curie University, Paris, France. After over a decade of clinical practice, Dr. Trad joined the biopharmaceutical industry in 2000 in varied senior strategic roles, both in Bio-Pharma and CROs. Her multivalent skills and significant experience in neurology, as well as her strong relationship with specialized clinical trial centers and key opinion leaders, makes Dr. Trad a strategic addition to Lysogene's team.

Lastly, Lysogene will make two oral presentations at the WORLDSymposium™ 2022, which will take place from 7 to 11 February 2022 in San Diego, USA. Dr. Ralph Laufer, Chief Scientific Officer of Lysogene, will present new biomarker data from the ongoing AAVance clinical trial in children with MPS IIIA. Karen Aiach, Chairman and CEO of Lysogene will present preliminary safety data from the ongoing adaptive clinical study in children with GM1 gangliosidosis.

"We are very pleased that we opened this renowned clinical site in France and have been able to treat a third child affected by GM1 gangliosidosis with LYS-GM101. Our study is progressing well and we look forward to finalizing the recruitment of the safety cohort, which will be an important milestone for this study." said **Karen Aiach, Founder Chairman and Chief Executive Officer of Lysogene**. *"The new data we will present at the WORLDSymposium™ this year demonstrate the progress we have made with our two clinical programs, and the recognition of Lysogene as a key player in the field of lysosomal storage diseases. We continue increasing our focus on clinical execution, and the addition of Marie Trad as our Chief Medical Officer, bringing many years of clinical development experience and a deep expertise in neurology, is a great asset for Lysogene".*

"We are delighted to collaborate once again with Lysogene in this new gene therapy trial," said **Dr. Bénédicte Heron, neuropediatrician at the Armand Trousseau Hospital (AP-HP), Paris, France**. *"GM1 gangliosidosis is a devastating orphan disease that leads to rapid and severe neurodegeneration, which is why it is crucial to bring a targeted treatment to these children and their families."*

Oral Presentations at the WORLDSymposium™ 2022:

Thursday 10 February 2022 – 10:30 PST (07:30 pm CET)

- A study of intracisternal administration of adeno-associated viral vector serotype rh.10 carrying the human β -galactosidase cDNA for the treatment of GM1 gangliosidosis: Preliminary results of the safety cohort
- Presenter: Karen Aiach, Chairman and CEO of Lysogene

Thursday 10 February 2022 – 14:00 PST (11:00 pm CET)

- AAVance gene therapy study in children with mucopolysaccharidosis type IIIA
- Presenter: Dr. Ralph Laufer, Chief Scientific Officer of Lysogene

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 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 is ongoing. An adaptive clinical trial in GM1 gangliosidosis is also ongoing. 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 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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