PRESS RELEASE

Lysogene Reports Full Year 2020 Financial Results and Provides Operational Update

- **Positive biomarker data reported with LYS-SAF302 for the treatment of MPS IIIA**
- **New program entering the clinic with MHRA and FDA, and newly ANSM approvals to dose first patient with LYS-GM101 for the treatment of GM1 gangliosidosis**
- **Pipeline extension with new research collaboration agreement with the Weizmann Institute of Science for neuronopathic Gaucher and Parkinson's diseases**
- **Strengthened cash position with €7.7m capital raise led by top tier US investors and recent €5m non-dilutive financing**

Paris, France — 31 March 2021 at 08:00 am — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform Company targeting central nervous system (CNS) diseases, today announced its 2020 full-year results, approved by the Board of Directors on 30 March 2021. Audit procedures on the Company's 2020 financial statements were completed by the Company's statutory auditors.

Karen Aiach, Founder, Chairman and Chief Executive Officer of Lysogene said: *"2020 has been marked by challenges of a new sort with the COVID-19 pandemic, and I want to warmly thank our employees, partners and providers for their resilience during these tough times. Despite this new environment, Lysogene continued to execute on its two lead programs with positive biomarker data on LYS-SAF302 and regulatory clearances to initiate the clinical trial with LYS-GM101. In addition, we continued to expand our early-stage pipeline with a new collaboration with the Weizmann Institute for gene therapy approaches for neuronopathic Gaucher and Parkinson's diseases. Eventually, we strengthened our balance sheet with a capital raise, welcoming new US investors providing further validation of our technology."* **Karen Aiach** added: *"2021 is off to a good start, and this year will be marked by a sustained focus on executing on our clinical programs and further developing our pre-clinical pipeline."*

Selected financial information on 31 December 2020 (IFRS financial statements)

Income statement *in thousand euros*	**31/12/2020**	**31/12/2019**
Operating income		
- Revenue	13,369	13,373
- Other operating revenue	3,213	3,279
Total operating income	**16,582**	**16,652**
Operating expense		
- Research and development expenses	(16,307)	(17,624)
- General and administrative expenses	(5,352)	(4,111)
Total operating expenses	**(21,659)**	**(21,735)**
Net operating income	**(5,077)**	**(5,083)**
- Net financial income	(14)	801
- Income tax expense	-	-
Net income (loss)	**(5,091)**	**(4,282)**
Weighted average number of shares outstanding	*15,799,304*	*13,594,489*
Résultat de la période par action	**(0.32)**	**(0.31)**

Number of shares oustanding as of 31 March 2020: 16,841,104

- Net cash at the beginning of the year	26,467	24,952
- Increase/decrease in net cash position	(7,687)	1,515
Net cash at the end of the year	**18,780**	**26,467**

Total operating income reached €16.6 million in 2020, stable versus 2019, composed of:

- €13.4 million revenues[1] derived from the recognition of milestone payments from Sarepta in accordance with the IFRS 15 accounting standard;
- €3.2 million in other operating income, mainly consisting of the Research Tax Credit.

Operating expenses reached €21.7 million in 2020, stable versus 2019, composed of:

- Research and development expenses of €16.3 million, down €1.3 million from last year, due to a high basis of comparison in 2019 impacted by an accrual for a commercial dispute with an industrial partner and a milestone payment made to REGENXBIO in the first quarter of 2019 following the treatment of the first patient with LYS-SAF302. In addition, clinical expenses for the LYS-SAF302 drug candidate also decreased due to the end of patient enrollment and travel constraints related to COVID-19, offsetting increased expenses on the LYS-GM101 program;

[1]In accordance with the IFRS 15 standard "Revenue from contracts with customers", Lysogene is required to recognize revenue related to the license agreement signed with Sarepta. The revenue is allocated pro rata to the direct internal and external costs associated with the development of the LYS-SAF302 product, from the date of signature of the license agreement on 15 October 2018 until the end of the phase 2/3 clinical trial with LYS-SAF302

- General and Administrative expenses of €5.4 million, up €1.2 million compared to 2019. This increase mainly results from the full-year impact of the internalization of some key finance and business development functions. To support its growth, the Company also increased its legal and business development budgets, as well as its communication expenses with the redesign of its website.

The net financial result was close to null at (€0.01 million) in 2020, compared to €0.8 million in 2019. The 2019 financial gain resulted from positive interest rates and unrealized foreign exchange gains on the USD term accounts. In 2020, the Company no longer recorded such financial gain as it converted its USD term accounts into Euros to hedge against the negative evolution of the USD, considering that most of the Company's expenses are now denominated in Euros.

The net loss for the period was €5.1 million compared to €4.3 million in 2019.

As of December 31, 2020, the company's net cash amounted to €18.8 million, recently strengthened by a €5.0 million State-Guaranteed Loan (PGE) received in February 2021. This cash position provides the Company with financial visibility until the second quarter of 2022.

Business update

LYS-SAF302 program

On 25 February 2020, the Company announced that the program was granted Fast Track Designation by the U.S. Food and Drug Administration (FDA) after having previously received Orphan Drug Designations in the European Union in 2014 and in the US in 2015, as well as Rare Pediatric Disease Designation in the US.

The Company treated a total of 19 patients in the Phase 2/3 clinical trial with LYS-SAF302 in MPS IIIA (NCT03612869). Recruitment of the main cohort is completed, therefore it is not impacted by the clinical hold issued by FDA in June 2020. The Company is making encouraging progress in the collection and analysis of the information necessary to respond to the FDA's questions.

The Company reported positive biomarker data demonstrating the biological activity of LYS-SAF302 at the WORLDSymposium™ 2021. In 9 patients analyzed, significant reductions in heparan sulfate concentrations in the cerebrospinal fluid (CSF) were observed 12 months after treatment. In addition, the abnormally high concentration of GM2 and GM3 gangliosides, considered possible contributors to neuronal damage in lysosomal storage diseases, was reduced in the CSF of patients at 12 months post-treatment.

LYS-GM101 program

On 30 March 2021, the Company obtained approval from the ANSM in France, after having received authorizations from the MHRA in the U.K. and the FDA in the U.S. earlier this year, to initiate the adaptive clinical trial with LYS-GM101 for the treatment of GM1 gangliosidosis (NCT04273269).

Lysogene is initiating its global, multi-center, single-arm, two-stage, adaptive-design clinical trial of LYS-GM101 in patients with a diagnosis of early or late infantile GM1 gangliosidosis. The clinical trial will include a safety phase and a confirmatory efficacy phase. The Company intends dosing a total of 16 patients, with dosage of the first patient expected in the first half of 2021.

In addition, Lysogene's natural history video study in GM1 gangliosidosis (NCT04310163) has received IRB (Institutional Review Board) approval and has already successfully recruited 67% of participants.

Fragile X syndrome program

Lysogene is engaged in a discovery collaboration with the lab of Hervé Moine at the IGBMC Strasbourg and SATT Conectus. The aim of the collaboration is to explore the therapeutic potential in Fragile X syndrome (FXS) of an AAV vector carrying a modified form of diacylglycerol kinase kappa. Pre-clinical proof of concept studies in a mouse model of FXS are progressing as planned, with initial results expected this year.

Gaucher and Parkinson's diseases

In June 2020, Lysogene has entered into a collaborative research agreement with Yeda, the commercial arm of the Weizmann Institute of Science, with the aim of developing a novel AAV gene therapy approach for neuronopathic Gaucher disease, Parkinson's disease, and other diseases associated with mutations in the GBA1 gene. Initial results are expected by Q1 2022.

Capsid Discovery program

Lysogene is engaged in a discovery collaboration aiming at the development of novel AAV capsids with IRBM, a global partner research organization in Pomezia (Rome, Italy) with proven experience and track record in integrated neuroscience drug discovery. The collaboration is proceeding according to plan.

Impacts of COVID-19 on the Company's activities

In the context of the Phase 2/3 study with LYS-SAF302, the organization of visits and the follow-up of patients was made more difficult, forcing the Company to submit urgent safety measures to the health authorities and to amend certain protocols. For instance, the Company had to reorganize some hospital visits of patients by involving new doctors and sending them to other hospitals. All these new arrangements have resulted in additional direct clinical costs for the Company.

In addition, the start of the clinical trial with LYS-GM101, initially planned for 2020, has been postponed to the first half of 2021 due to delays in the regulatory review processes. Despite this delay of several months, LYSOGENE had to bear the costs related to contractual payments of CRO fees and other service providers.

These additional clinical costs were offset by savings on travel expenses for all employees, which have been significantly reduced from March 2020.

Furthermore, since the beginning of the pandemic, the Company has strictly complied with government guidelines and recommendations. It is committed to preserving the integrity and safety of its employees, partners, patients, and their families.

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 in partnership with Sarepta Therapeutics, Inc. is ongoing. An adaptive 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 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 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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