PRESS RELEASE

LYSOGENE Reports First Half 2021 Financial Results and Provides Operational Update

- **In the first half 2021, Lysogene advanced and extended its development pipeline**
- **Cash and cash equivalents of €15m[1] as of 30 June 2021, strengthened by €5m non-dilutive PGE loan**

Paris, France — 24 September 2021 at 08:00 am CET — Lysogene (FR0013233475 – LYS), a phase 3 gene therapy platform Company targeting central nervous system (CNS) diseases, today announced its financial results for the first half 2021, approved by the Board of Directors on 23 September 2021. The financial statements were subject to a limited review by the Company's statutory auditors. The full interim financial report is available on the Company's website in the Investors' section.

Karen Aiach, Founder Chairman and Chief Executive Officer of Lysogene commented: *"The first half of 2021 has been very active for Lysogene on the operational front with the publication of positive biomarker data with LYS-SAF302, regulatory clearances and treatment of the first patients with LYS-GM101, and the in-licensing of the global rights of LYS-FXS01 for the treatment of the Fragile X syndrome, a broad CNS disorder".* **Karen Aiach** added: *"The second half of the year should be just as dynamic with notably the continued enrollment of the LYS-GM101 safety cohort and first proof of concept data expected for LYS-FXS01. In parallel, a strong priority will be put on strengthening our balance sheet and adding new partnerships to continue expanding our pipeline."*

[1] *Unaudited figure*

Selected financial information on the first half 2021 (IFRS financial statements)

Income statement *in thousand euros*	**30/06/2021**	**30/06/2020**
Operating income		
- Revenue	2,922	9,126
- Other operating revenue	1,305	1,516
Total operating income	**4,227**	**10,642**
Operating expense		
- Research and development expenses	(6,859)	(7,610)
- General and administrative expenses	(2,566)	(2,949)
Total operating expenses	**(9,425)**	**(10,559)**
Net operating income	**(5,198)**	**83**
Net financial income	(333)	147
Net income (loss)	**(5,531)**	**230**
Net income (loss) per share (€)	**(0.33)**	**0.02**
Net income (loss) per share – Diluted (€)	**(0.30)**	**0.01**

Weighted average number of shares outstanding as of 30 June 2021: 16.794.212

Net cash as of 1st January	**18,780**	**26,467**
Increase/decrease in net cash position	(3,710)	(2,657)
Net cash as of 30 June	**15,070**	**23,810**

In the first half 2021, total operating income reached €4.2 million compared to €10.6m in 2020 with:

- Revenues[2] of €2.9 million vs €9.1m in H1 2020 resulting from the recognition of the Sarepta payments according to IFRS 15 accounting standards. The decrease versus last year was due to lower LYS-SAF302 development expenses as study enrolment was completed in the first half 2020 and to a 2020 basis inflated by a one-off impact from a change in the accounting method.
- Other operating revenues of €1.3 million consisting mainly of the Research Tax Credit for the first half of the year.

Operating expenses reached €9.4 million in the first half 2021 compared to €10.6m in 2020 composed of:

- Research and development expenses of €6.9 million, down €0.8 million from last year due to lower LYS-SAF302 clinical expenses as patient enrolment was completed in 2020, lower LYS-GM101 CMC costs as there were fewer clinical batch production runs in 2021, and eventually a reduction in payrolls reflecting the company's workforce evolution. The decrease in R&D expenses was partially offset by the settlement of a commercial dispute with an industrial partner resulting in

[2]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

the reversal of the remaining provision for risks (€1.2 million) and the payment by Lysogene of $2.8 million (approximately €2.4 million) to the partner on July 28, 2021.

- General and Administrative expenses of €2.6 million, down €0.4 million compared to 2020 with lower external costs related to legal fees and other accounting services that are now internalized as part of the financial team reorganization. This decrease was partly compensated by higher staff expenditure with the IFRS 2 impact on new free share plans and the strengthening of the financial team completed end of 2020.

The net financial result was negative at €0.3 million in the first half 2021 versus a positive €0.1 million in 2020 impacted by the financial interests paid in relation with the settlement of the commercial dispute with an industrial partner and the absence of financial interests on USD term deposit accounts converted into Euro in H1 2020.

The net loss for the period amounted to €5.5 million compared to a net income of €0.2 million in the first half 2020.

As of June 30, 2021, the company's net cash amounted to €15.1 million.

Business update

LYS-SAF302 program

The Company treated a total of 19 subjects in the Phase 2/3 clinical trial with LYS-SAF302 in MPS IIIA. Recruitment of the main cohort was completed in Q2 2020 and was therefore not impacted by the clinical hold issued by FDA in June 2020.

The Company reported positive biomarker data demonstrating the biological activity of LYS-SAF302 at the WORLDSymposium™ in February 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.

The Company continues collecting and analyzing the study data and information per protocol. Results of the clinical trial are expected in 2022.

LYS-GM101 program

During the first quarter of 2021, Lysogene obtained approvals from regulatory authorities and ethics committees in the United Kingdom, the United States and France to initiate its adaptive-design clinical trial with the investigational gene therapy LYS-GM101 for the treatment of GM1 gangliosidosis.

In June and August 2021, the Company treated the first two patients at the Royal Manchester Children's Hospital, UK and at the CHOC Hospital, USA, respectively. The clinical trial includes a safety phase and a

confirmatory efficacy phase. The trial will enroll 16 patients with a diagnosis of early or late infantile GM1 gangliosidosis at sites in the US and Europe.

In July 2021, the FDA granted Fast Track designation for the program which complements the Rare Pediatric Disease and Orphan Drug designations granted by the agency in 2016 and 2017.

By the end of 2021, the company expects to have enrolled all patients in the safety cohort.

LYS-FXS01 program

After collaborating with Dr. Hervé Moine of the Institut de Génétique et de Biologie Moléculaire et Cellulaire (IGBMC) and SATT Conectus since 2018, Lysogene decided in June 2021 to enter into an exclusive, worldwide license agreement with SATT Conectus for the development and commercialization of a gene therapy candidate for the treatment of the Fragile X syndrome.

The Company will be responsible for the nonclinical and clinical developments, manufacturing, regulatory activities, and commercialization of the drug candidate globally and is currently conducting the necessary preclinical work to develop the drug now referred to as LYS-FXS01.

Gaucher and Parkinson's diseases

In June 2020, Lysogene 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 early 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.

The Company's next financial release is scheduled for October 8, 2021 to provide its cash position at the end of September 2021.

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 ongoing. 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 has also entered into an exclusive worldwide license agreement with SATT Conectus for a gene therapy candidate for the treatment of the 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 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.

Contacts

Stéphane Durant des Aulnois
Chief Financial Officer
stephane.durant-des-aulnois@lysogene.com
+ 33 1 41 43 03 99