Press Release
25 September 2020

Lysogene Reports First Half 2020 Financial Results and Provides Operational Update

- **Cash position of €23.8m as of 30 June 2020 strengthened by a €7.7m capital increase led by OrbiMed and Sarepta, extending cash runway to Q4 2021**
- **Follow-up of the 19 patients treated in the LYS-SAF302 phase 2/3 clinical trial (AAVance) in MPS IIIA**
- **Lysogene's natural history video study in GM1 gangliosidosis 50% recruited**
- **Extended collaboration with Novasep for the development and production of LYS-GM101 for the treatment of GM1 gangliosidosis**
- **Research collaboration signed with Yeda/the Weizmann Institute of Science for the development of a novel AAV gene therapy approach for neuronopathic Gaucher disease and Parkinson's disease**

PARIS, France – 25 September 2020 at 08:00am – Lysogene (FR0013233475 - LYS), a phase 3 gene therapy platform Company targeting central nervous system (CNS) diseases, today announced its 2020 half-year results, approved by the Board of directors on 24 September 2020. 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 said: "*During the first half of 2020 and despite the challenges due to the COVID-19 pandemic, we continued to execute on our clinical programs by treating 19 patients in our phase 2/3 study in Sanfilippo disease, preparing for the initiation of our clinical trial in GM1 gangliosidosis, and strengthening our early-stage pipeline with a collaborative research agreement signed with the Weizmann Institute of Science. Moreover, we completed a fundraising that allows us to strengthen our balance sheet and welcome leading US Pharma investors.*" **Karen Aiach** added*: "I want to express my deep thanks to all our employees who continue to deliver high quality work in these challenging times, as well as our loyal shareholders."*

Selected financial information on 30 June 2020 (IFRS financial statements)

in thousand euros	**30/06/2020**	**30/06/2019**
Operating Income		
- Revenues	9,126	6,794
- Other Operating Income	1,516	1,507
Total Operating Income	**10,642**	**8,301**
Operating Expenses		
- Research & Development expenses	(7,610)	(8,648)
- General & Administrative expenses	(2,949)	(1,852)
Total Operating Expenses	**(10,559)**	**(10,500)**
Operating Income (loss)	**83**	**(2,199)**
Financial Income (loss)	147	293
Net Income (loss)	**230**	**(1,906)**
Net Income per share (€)	**0.02**	**(0.14)**
Net cash as of January 1st	**26,467**	**24,952**
Change in net cash	(2,657)	8,201
Net cash as of June 30th	**23,810**	**33,153**

In the first-half 2020, revenues[1] reached €9.13m versus €6.79m in the fist-half 2019, resulting from the recognition of the Sarepta payments according to IFRS 15 accounting standards. Other Operating Income consisting mainly of the Research Tax Credit reached €1.52m and was stable versus last year.

Research & Development expenses amounted to €7.61m compared to €8.65m in the first-half 2019 due to a decrease as planned in the number of production campaigns for the product LYS-GM101 in the first half of 2020.

General & Administrative expenses amounted to €2.95m compared to €1.85m the previous year. This increase is driven by new recruitments with the reinforcement of the administrative and financial team initiated at the end of 2019, external expenses related to legal and accounting fees as well as the Headquarters' office rental cost.

Financial Income reached €0.15m in the first-half 2020 versus €0.29m in the first-half 2019 resulting from net foreign exchange gains over the period.

The Net Income for the period amounted to €0.23m, versus a loss of €1.91m in the first-half 2019, mainly due to the acceleration of revenue recognition related to the contract with Sarepta.

As of 30 June 2020, the Company had a cash position of €23.81m, including €7.7m from the capital increase in March 2020.

[1] In accordance with the IFRS 15 standard "Revenue from customer contracts", and after analysis with its external auditors, Lysogene is now required to recognize revenues relating to the license agreement signed with Sarepta. Revenues must be spread prorata 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 for LYS-SAF302

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.

In June 2020, following discussions with the FDA, a clinical hold was issued for the LYS-SAF302 AAVance (NCT03612869) phase 2/3 clinical trial in MPS IIIA following MRI observations of localized signals at the intracerebral injection sites. On 2 July 2020, the Company received a letter from the FDA confirming the clinical hold for treatment of new patients. The clinical hold results from a need for additional information to evaluate the MRI findings, and notably that they are not associated with clinical harm. The Company is making encouraging progress in the collection and analysis of the information necessary to respond to the FDA's questions.

LYS-GM101 program: The Company is working closely with regulators to obtain the necessary approvals to start its clinical trial, expected before the end of 2020, despite delays observed in the Agencies' regulatory review processes due to the COVID-19 pandemic. In addition, Lysogene's natural history video study in GM1 gangliosidosis (NCT04310163) has received IRB (Institutional Review Board) approval and has already successfully recruited 50% of participants.

Clinical pipeline: 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. Under the terms of the agreement, Lysogene will provide expertise in AAV vector design and production, while Prof. Futerman's lab will provide glucocerebrosidase variants with enhanced biological properties and conduct biological proof and concept studies. Lysogene has an exclusive option to license the program.

Financing : On 12 March 2020, Lysogene completed a capital increase for an amount of €7,729,440.33, led by investment firm OrbiMed Advisors LLC and the Company's shareholder and partner, Sarepta Therapeutics, Inc. Gross proceeds from the transaction will be used to finance the phase 1/3 clinical trial of LYS-GM101 for the treatment of GM1 gangliosidosis, the commercial launch preparation in Europe of LYS-SAF302 in MPS IIIA, and the Company's overhead costs and expenses.

Update on COVID-19 pandemic

COVID-19 pandemic that appeared in December 2019 in China has gradually spread to a large number of countries around the world, including France, where the Company is located, and countries in which its clinical trials are either planned or underway.

The LYS-SAF302 program has been minimally affected as 19 patients have already been treated and the company was able to continue medically necessary study visits for these participants. For the LYS-GM101 program, the Company confirms its expectation to treat the first patient in the second half of 2020 despite delays from Agencies in their regulatory review processes due to the pandemic.

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 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 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 phase 2-3 clinical trial 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.

Contacts

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