Press Release
30 September 2019

Lysogene Reports First Half 2019 Financial Results and Provides Operational Update

- **Cash and cash equivalent of €33.2 million as of June 30, 2019**
- **On track for full patient inclusion (11 out of 20 patients included) in the MPS IIIA AAVance Phase 2-3 gene therapy study of LYS-SAF302**
- **On target to submit the Phase 1-2 GM1 gangliosidosis clinical trial IND at end 2019**
- **Signature of a cooperation agreement with IRBM to develop novel gene therapy technology**

PARIS – September 30, 2019 at 06:00pm – Lysogene (FR0013233475 – LYS), a pioneering, Phase 3, biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, today announced financial results and major advancements for 2019.

The full interim financial report is available on the Company's website in the Investors section. The 2019 half-year financial statements were subject to a limited review by the Company's statutory auditors and approved by the Board of directors on September 25, 2019.

"The first half of 2019 was highlighted by progress made in both of our MPS IIIA and GM1 programs," said ***Karen Aiach, Founder, Chairman and Chief Executive Officer.*** *"We have already treated nine children in our international MPS IIIA Phase 2-3 trial, our partnership with Sarepta therapeutics considerably reinforces our presence and we have progressed our GM1 gangliosidosis pre-IND discussions with regulatory authorities. Our team remains highly focused on our lead programs, including the start of our GM1 clinical trial and continued enrollment in the MPS IIIA trial, and on strengthening our pipeline."*

Financial highlights of the first half of 2019[i]

In million euros	H1 2019	H1 2018
Revenues	6,8	-
Other incomes	1,5	1,2
Research and development expenses	(8,6)	(6,4)
General and administration expenses	(1,9)	(2,1)
Operating profit (loss)	**(2,2)**	**(7,3)**
Financial income (loss)	0,3	0,1
Net income (loss)	**(1,9)**	**(7,2)**
Net cash flow	8,2	(6,0)
Cash and cash equivalent at the end of the semester	**33,2**	**8,1**

Revenues and other income, according to IFRS15 standard, Lysogene achieved €6.8 million revenues in the first half of 2019. This revenues recognition relates to the license agreement signed with Sarepta Therapeutics in October 2018. Other incomes €1.5 million were essentially in the form of research tax credit (Crédit d'Impôt Recherche).

Research and development expenses, increased by 34% from €6.4 million in H1 2018 to €8.6 million in H1 2019. This increase is primarily due to the development of the manufacturing processes for LYS-SAF302 and LYS-GM101 drug candidates.

General and administrative expenses, remained stable over the period, amounting to €2.1 million and €1.9 million in the first halves of 2018 and 2019, respectively.

The Company's net loss, for the first half of 2019 amounted to €1.9 million compared to €7.2 million in the first half of 2018.

Net cash flows, amounted to €8.2 million in the first half of 2019, primarily as a result of the milestone payments made by Sarepta Therapeutics to Lysogene in March and May 2019 for a total amount of €16.6 million ($18.75 million) minus €8.4 million of total expenses within the first half of 2019.

Cash and cash equivalents, as of June 30, 2019 amounted to €33.2 million, compared to €25 million as of December 31, 2018 and €8.1 million as of June 30, 2018. Cash runway and cash position are perfectly on line with expectations.

2019 Corporate and Pipeline Developments

Significant progress has been made on the MPS IIIA program over the period with nine patients treated in the Phase 2-3 international trial, an additional two patients enrolled and is on track to achieve full inclusion of 20 patients by H1 2020.

Further advancement towards the Phase 1-2 study in GM1 gangliosidosis. Preclinical toxicology studies are ongoing, and a GMP clinical trial product has been manufactured to treat the first cohort of patients in the international study. A successful pre-IND meeting with FDA in 2Q 2019 cleared the path towards the submission of the IND by end 2019.

Strengthening and advancement of a robust gene therapy pipeline. The pipeline is being further expanded with the initiation of an undisclosed innovative discovery project. This project is in collaboration with IRBM, a global partner research organization in Pomezia (Rome, Italy) with proven experience and track record in integrated neuroscience drug discovery.

Enhancements to the Board of Directors to enable next phase of growth. Mathieu Simon and Carole Deffez were appointed as independent board directors and Karen Aiach was elected as the board of directors' chair.

Establishment of new Scientific Advisory Board (SAB). The newly established SAB will considerably reinforce Lysogene's expertise and support the establishment of a new paradigm for the treatment of monogenic neurological diseases.

Nomination for the Prix Galien MedStart up 2019 in the category of “Best collaboration dedicated to the developing or underserved populations worldwide.” This prestigious nomination marks the excellence of Lysogene’s innovation and performance.

Presentations in several leading scientific, biotech and policy events to present company and pipeline progress.

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 1-2 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 development strategy for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

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Lysogene's forward-looking statements

This press release may contain forward-looking statements, especially on the Company’s progress of its Phase 2/3 clinical trial. Although the Company believes that its expectations are based on reasonable assumptions, any statements other than statements of historical facts that may be contained in this press release relating to future events are subject to (i) change without notice, (ii) factors beyond the Company's control and (iii) the Company's financial capabilities. These statements may include, but are not limited to, any statement beginning with, followed by or including words or phrases such as "objective", "believe", "anticipate", "foresee", "aim", "intend", "may", "anticipate", "estimate", "plan", "project", "will", "may", "probably", "should", "could" and other words and phrases of the same meaning or used in negative form. Forward-looking statements are subject to inherent risks and uncertainties beyond the Company's control that may, if any, cause actual results, performance or achievements to differ materially from those anticipated or expressed explicitly or implicitly by such forward-looking statements. A list and description of these risks, contingencies and uncertainties can be found in the documents filed by the Company with the Autorité des Marchés Financiers (AMF) pursuant to its regulatory obligations, including the Company’s 2018 registration document, registered with the AMF on 29 April 2019 under number R. 19-016, as well as in the documents and reports to be published subsequently by the Company. In addition, these forward-looking statements speak 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 does not undertake any obligation to publicly update these forward-looking statements or to update the reasons why actual results could differ materially from those anticipated by the forward-looking statements, including in the event that new information becomes available. The Company's update of one or more forward-looking statements does not imply that the Company will make any further updates to such forward-looking statements or other forward-looking statements.

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[i] Limited review procedures on the financial statements as of June 30, 2019, have been performed in accordance with professional standards applicable in France.