Press release
October 30, 2018

Lysogene announces the publication of its 2018 half-year financial report and cash position as at end-September and provides an update on its performance in the first half of 2018

CAMBRIDGE, MA, United States, and PARIS, France – October 30, 2018, 08:00am– Lysogene (FR0013233475 – LYS), a leading clinical-stage biopharmaceutical company specializing in gene therapy technology applied to central nervous system diseases, today announces the publication of its half-year financial report for the period from January to June 2018.

The company's half-year financial report will be available in the Investors > Regulated information section of the www.lysogene.com website.

Karen Aiach, Lysogene's Founder and Chief Executive Officer, comments: "*During the first nine months of 2018, we continued to take key steps forward with our most advanced drug candidate, LYS-SAF302 against MPS IIIA, allowing us to confirm the start of the Phase II-III pivotal trial in the second half of 2018. We also held a Scientific Advice Meeting with the European Medicines Agency to refine the development plan for our second drug candidate, LYS-GM 101 against GM1 gangliosidosis and we have enlarged our portfolio of programs by forming a partnership with a view to developing an AAV-based gene therapy for the treatment of Fragile X syndrome. Lastly, we are delighted about the partnership recently announced with Sarepta, which constitutes a major step forward for Lysogene. The strengthening of our financial resources will also enable us to continue with our commitment to providing treatment for sufferers of MPS IIIA.*"

Selected financial information for the six months ended June 30, 2018 (IFRS consolidated financial statements)

In thousands of euros	2018	2017
Revenues	-	-
Other income	1,203	1,039
Research and development expenses	(6,373)	(6,828)
Selling and administrative expenses	(2,082)	(2,242)
Operating loss	**(7,252)**	**(8,031)**
Financial income	106	119
Financial expenses	(70)	(339)
Net financial income (expense)	**36**	**(219)**
Income tax	-	-
Net loss	**(7,215)**	**(8,251)**

N.B. Limited review procedures on the statutory and consolidated financial statements as of June 30, 2018, have been performed in accordance with professional standards applicable in France.

Comments on the income statement

Operating loss

The Company still does not have any commercial activities and does not generate any revenues.

Other operating income totalled €1,203 thousand in the first half of 2018 compared with €1,039 thousand in the first half of 2017. This growth relates to a higher amount of research tax credits in 2018, reflecting the company's increased activity.

Operating expenses

Operating expenses decreased between the first half of 2017 and the first half of 2018 from €9,070 thousand to €8,454 thousand, a fall of €616 thousand.

This is mainly due to the lower IFRS 2 expense relating to the implementation of share-based payment plans adopted in March 2017.

External R&D purchases and expenses increased in connection with R&D activities over the period, in particular the manufacturing process development campaigns for LYS-SAF302 and LYS-GM101 drug candidates and the end of pre-clinical activities in MPS IIIA.

Net loss

The company sustained a net loss of €7,215 thousand in the first half of 2018 compared with €8,251 thousand in the first half of 2017.

Following the signing of a partnership agreement with Sarepta (see Key events since June 30), the financial statements were approved according to the going concern principle.

Cash position at September 30, 2018

The company's cash position as at September 30, 2018 was €5,938 thousand. This amount does not include payments received from Sarepta within the framework of the partnership agreement signed in October 2018 (see Key events since June 30, 2018 for more details).

Key events in the first half of 2018

Progress in the LYS SAF-302 program – Phase III drug candidate for MPS IIIA

In February 2018, Lysogene obtained a positive opinion from the Pediatric Committee (PDCO) of the European Medicines Agency (EMA) concerning the pediatric investigation plan (PIP) for LYS-SAF302. This major regulatory stage confirmed the planned Phase II-III single-arm clinical trial on children with MPS IIIA. As a result of this approval, Lysogene is eligible to extend exclusive commercial rights for a further two years – in addition to the 10-year exclusive commercial rights associated with EMA's orphan designation for LYS-SAF302.

The company has finalized the preclinical trials needed to launch the pivotal clinical trial for the LYS-SAF302 drug candidate. These trials, which obtained positive results in terms of efficacy and toxicology, were used to support the Investigational New Drug (IND) application submitted to the FDA in July 2018 and approved in late August 2018 (see Subsequent events).

In April 2018, the company announced that the launch of the Phase II-III pivotal trial was now planned for the second half of 2018.

Strengthening of board of directors and management team

In April, Dr. Peter Lichtlen was appointed to the company's Board of Directors as an independent member. This appointment was ratified by the general shareholders' meeting in June.

In May 2018, Lysogene strengthened its management team with the appointment of Dr. Ralph Laufer as Scientific Director.

Progress in other programs

During the first half of the year, Lysogene also enlarged its portfolio of programs by forming a partnership with a view to developing an AAV-based gene therapy for the treatment of Fragile X syndrome, the most common inherited cause of learning difficulties and autism spectrum disorders. The Fragile X syndrome program draws on Lysogene's existing expertise in CNS diseases and capitalizes on the company's clinical and manufacturing capabilities.

In June 2018, Lysogene held a Scientific Advice Meeting with the European Medicines Agency (EMA) to refine the development plan for LYS-GM 101.

Key events since June 30, 2018

FDA approval of the IND application to initiate a Phase II-III trial for MPS IIIA

On September 5, 2018, the company announced that the Food and Drug Administration (FDA) had approved the Investigational New Drug (IND) application, thereby authorizing the launch in the United States of the Phase II-III (AAVance) international clinical trial of LYS-SAF302 for the treatment of mucopolysaccharidosis type IIIA (MPS IIIA).

Changes to the Board of Directors

In September, the company was informed of Bpifrance Investissement's resignation as a director. Bpifrance Investissement's permanent representative on the Board of Directors was replaced in his duties on the audit committee by Mr. Peter Lichtlen, director.

Exclusive license agreement for LYS-SAF302 with Sarepta Therapeutics, Inc.

On October 15, the company announced the signing of a collaboration and license agreement with Sarepta Therapeutics, Inc., a leading name in precision genetic medicine for the treatment of rare diseases. The agreement concerns primarily LYS-SAF302. The agreement also gives Sarepta an option on another Lysogene gene therapy candidate targeting the CNS.

In accordance with the terms of the agreement, Lysogene will be in charge of conducting the pivotal trial due to begin in the fourth quarter of 2018. Sarepta acquires exclusive commercial rights to LYS-

SAF302 in the United States and markets outside Europe, and Lysogene maintains commercial exclusivity of LYS-SAF302 in Europe, which is of strategic interest for Lysogene as Europe constitutes a major market with a centralized procedure for obtaining marketing authorization for drug candidates as well as a structured network of reference centers. Sarepta will be in charge of global manufacturing of LYS-SAF302 and will supply Lysogene in its markets.

In 2018, Sarepta paid Lysogene a total of €22 million in cash, in addition to a €2.2 million investment in Lysogene's share capital. In 2019, Sarepta will pay Lysogene an additional amount of up to €16 million. In total, Sarepta could pay Lysogene €108 million in respect of all payments and milestones, in addition to royalties. The financial terms of the agreement considerably strengthen Lysogene's cash position, allowing it to also continue to develop its other assets.

Within the framework of this partnership, Sarepta has invested €2.2 million in Lysogene's share capital. Lysogene intends to use the proceeds of this capital increase to finance its activities and in particular the continuing development of LYS-GM101 in GM1 gangliosidosis. Sarepta' subscription to the share capital increase was completed on October 29, 2018.

According to the company's current development plan, in view of the amounts paid to the company, the signing of the license agreement with Sarepta will ensure that it has the financial means needed to exercise its activities in full.

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 pivotal clinical trial in MPS IIIA in partnership with Sarepta Therapeutics, Inc. and a phase 1-2 clinical trial in GM1 Gangliosidosis are 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 a major partner to define the strategy of development for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

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Forward looking statement

This press release may contain certain forward-looking statements. 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, and (ii) factors beyond the Company's control. 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," "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 2017 registration document (*Document de référence*), registered with the French Markets Authorities on June 4, 2018, under number R. 18-047, 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.