Press release
11 April, 2019

Lysogene Reports Full Year 2018 Financial Results and Provides Operational Update

- **Cash position of €25 million on 31 December 2018.**
- **Global Collaboration with leading US Biotechnology Company, Sarepta, on LYS-SAF302, providing potential payment of up to $125 million (€108 million), if all milestones are met.**
- **First patient recruited in the LYS-SAF302 Phase 2/3 Clinical Trial (AAVance) in MPS IIIA triggered a milestone payment from Sarepta of $18 million (€16 million).**
- **Completion of GM1 preclinical efficacy studies. IND scheduled for 2020.**

PARIS, France, - April 11, 2019 at 7:00 a.m. CEST - Lysogene (FR0013233475 - LYS), a biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, today announced its 2018 financial results, approved by the Board of Directors on April 9, 2019. Audit procedures on the Company's 2018 consolidated financial statements were completed by the Company's statutory auditors.

"*2018 was a very important year for Lysogene. The signature of a strategic collaboration with Sarepta Therapeutics, leading US player in rare diseases and innovative treatments, validates Lysogene's strategic approach, expertise, and capacity to deliver. The payments associated with this partnership, significantly extend Lysogene's cash runway*," **said Karen Pignet-Aiach, Founder and CEO of Lysogene.** "*LYS-SAF302, the first gene therapy for Sanfilippo Type A (MPS IIIA), is now in a Pivotal Phase 2/3 clinical trial to treat this lethal pediatric disease. In addition, significant progress was made on Lysogene's pipeline: We are anticipating opening the IND for the treatment of GM1 gangliosidosis with LYS-GM101 in the first half of 2020 and an external collaboration was signed to complete the pre-clinical work for our new drug candidate targeting the unmet medical need of Fragile X syndrome*."

Selected financial information on 31 December 2018 (IFRS consolidated financial statements)

In thousands of euros	2018	2017
Revenues	3,590	0
Other operating income	2,354	2,687
Research and development costs	(10,705)	(15,330)
General and administrative expenses	(6,194)	(4,573)
Operating income (loss)	**(10,955)**	**(17,216)**
Net income (loss)	**(10,925)**	**(17,794)**
Earnings per share (€]	(0.87)	(1.52)
Net cash flows from operating activities	(9,393)	(14,615)
Net cash flows from financing activities	1,853	23,149
Change in net cash and cash equivalents	**10,859**	**7,837**
Cash and cash equivalents at the end of the year	**24,952**	**14,089**

Lysogene achieved €3.59 million Revenues [(1)] in 2018 (IFRS recognition in connection with the partnership with Sarepta, as described in the Company's financial statements).

In 2018, research and development expenses amounted to € 10.7 million compared with €15.3 million in 2017, mainly explained by a decrease in costs for pre-clinical studies, manufacturing and the natural history study for LYS-SAF302, compared to 2017. General and administrative expenses amounted to €6.2 million compared with €4.5 million in 2017, an increase mainly explained by exceptional costs booked in 2018 in connection with the strategic partnership with Sarepta.

Operating income stood at € (10.9) million in 2018, compared with € (17.2) million in 2017. Net income amounted to € (10.9) million in 2018, compared with € (17.8) million in 2017.

As of December 31, 2018, and following the signature of the Global Collaboration with Sarepta, the company had a cash position of €25 million, allowing the Company to fully fund its Phase 2/3 clinical trial on LYS-SAF 302.

[(1)] In accordance with the new 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 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 October 15, 2018 until the end of the Phase 2/3 clinical trial for LYS-SAF302. Based on the estimated direct costs incurred over the period from 15 October to 31 December 2018, which represent 7.7% of total termination costs, Lysogene recorded revenues of €3.59 million for the 2018 financial year, corresponding to 7.7% of the transaction price, taking into account the amount of upfront and milestones acquired or highly probable.

Operational update

Partnership with Sarepta

- In October 2018, Lysogene entered into a Global Collaboration with Sarepta Therapeutics Inc, a leading US company in genetic precision medicine for rare diseases, to develop LYS-SAF302. The agreement also provides Sarepta Therapeutics Inc. with an option on an additional Lysogene gene therapy candidate targeting the central nervous system (CNS). The terms of this partnership have been announced on 15 October 2018, and the collaboration is progressing well.

Launch of the LYS-SAF302 international Phase 2/3 clinical study (AAVance) for the treatment of MPS IIIA

- In February 2018, Lysogene obtained approval from the Paediatric Committee (PDCO) of the European Medicines Agency (EMA) on the LYS-SAF302 Paediatric Investigation Plan (PIP). This regulatory milestone confirmed the design of the single-arm phase 2/3 clinical trial in MPS IIIA. As a result, Lysogene will be eligible for a 2-year marketing exclusivity extension - in addition to the 10-year exclusivity associated with the orphan drug designation granted by the EMA.

- In September 2018, the Food and Drug Administration (FDA) approved the Investigational New Drug (IND) application, authorizing the initiation of the Phase 2/3 (AAVance) clinical trial in the United States.

- In February 2019, Lysogene treated the first patient in the Phase 2/3 clinical trial (AAVance) evaluating LYS-SAF302 gene therapy in MPS IIIA, triggering milestone payments totaling $18 million (€16 million) from Sarepta to Lysogene.

Progress of other programs

- In April 2018, Lysogene expanded its portfolio of programs by entering into a partnership to develop an AAV-based gene therapy for the treatment of Fragile X syndrome, the most common inherited form of intellectual disability and autism spectrum disorders. This program is built on Lysogene's existing expertise in CNS diseases and gene therapy, and capitalizes on the company's clinical and manufacturing capabilities.

- In June 2018, Lysogene held a Scientific Advice Meeting at the European Medicines Agency (EMA) to define the development plan for LYS-GM101, and IND is scheduled for the first half of 2020.

Upcoming financial events

- April 22, 2019 (after market close): Turnover and cash flow for the first quarter of 2019
- April 30, 2019: Reference Document 2018
- June 26, 2019: Ordinary General Assembly
- July 25, 2019 (after market close): Turnover and cash flow for the 2nd quarter of 2019

About Lysogene

Lysogene is a gene therapy company focused on treatment of orphan diseases for 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. 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 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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Lysogene's forward-looking statements

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