Press release
September 19, 2017

Lysogene announces first half 2017 financial results and business update

- **Stepping up the strategic development of the two gene-therapy drug candidates**

- **Strengthening the medical and operational management team**

Paris, France and Cambridge, MA, US – September 19, 2017 at 05:45pm CET - Lysogene (FR0013233475 – LYS), a leading clinical-stage biopharmaceutical company specializing in gene therapy technology applied to central nervous system diseases, is today reporting its results for the first half of 2017, as approved by the Board of Directors on September 15, 2017, and providing an update on activities.

Karen Aiach, Lysogene's CEO, made the following comments: *"The successful IPO in February has helped accelerating progress in terms of both our clinical operations and our management team. We achieved a number of important strategic milestones during the first half of 2017, including setting-up a unique international observational natural history study in Sanfilippo syndrome A (MPS IIIA), and signing a manufacturing agreement with a first-class contract manufacturing organization based in the US to produce our gene therapy candidate for GM1 gangliosidosis (GM1). This faster development has been supported by the expertise and enthusiasm of our team, which has been strengthened by the arrivals of Mr Philippe Mendels-Flandre, Chief Operating Officer, and Dr Sophie Olivier, Chief Medical Officer. Lysogene will continue this trajectory in the second half of 2017, as we execute the preparation for the phase II/III trial in MPS IIIA".*

Selected financial information for the six months ended June 30, 2017 (IFRS consolidated financial statements)

In thousands of euros	First half 2017	First half 2016
Revenues	-	-
Other income	1,039	616
Research and development expenses	(6,828)	(2,542)
Selling and administrative expenses	(2,242)	(1,107)
Operating loss	**(8,031)**	**(3,034)**
Net loss	**(8,251)**	**(3,233)**
Net loss per share (€)	(0.73)	(0.39)
Net cash flow related to operating activities	(7,717)	(4,167)
Net cash flow related to financing activities	22,651	(4)
Change in cash position (excl. forex differences)	**14,803**	**(4,185)**
Cash and cash equivalents at the end of the period	**21,025**	**9,079**

N.B. Limited review procedures on the statutory and consolidated financial statements as of June 30, 2017, have been performed in accordance with professional standards applicable in France.

Other income amounted to €1.04 million in the first half of 2017, mainly consisting of the R&D Tax Credit (RTC). RTC amount was higher in June 30, 2017 than in June 30, 2016, due to the increase in R&D expenses in the first half of 2017 to €6.8 million as opposed to €2.5 million in the first half of 2016. The increase reflected the Company's faster development, with a doubling in staff numbers between the first half 2016 and first half 2017, along with activities such as pre-clinical studies on LYS-SAF302 and the manufacturing process development campaign of LYS-GM101, which began in the second quarter of 2017.

Selling and administrative expenses doubled from €1.1 million in the first half of 2016 to €2.2 million in the first half of 2017, mainly because of IFRS 2 impact (share based payment) & IPO-related expenses. Except these impacts, the selling and administrative expenses remain stable.

Lysogene's net loss totalled €8.3 million in the period ended 30 June 2017.

Key events in the first half of 2017

Successful IPO

- *In February*, Lysogene reached an important milestone in its development with a successful initial public offering on Euronext Paris, raising €22.6 million.

Strengthening the management team by appointing a COO and a CMO

- *In May*, Mr Philippe Mendels-Flandre was appointed Chief Operating Officer to strengthen Lysogene's management team and support the company with its strategic growth. Philippe is a member of the Executive Committee and brings along 16 years of healthcare industry experience.

- *In June*, Dr Sophie Olivier was appointed Chief Medical Officer. Sophie was previously Pediatric Coordinator at the European Medicines Agency (EMA), after holding various positions within the Wyeth group. Dr Olivier has more than 20 years of experience managing global clinical and regulatory drug development.

LYS-SAF302, Lysogene's most advanced drug candidate for MPS IIIA

- *In March,* Lysogene presented baseline data from the first International Pivotal Observational Study in MPS IIIA (SAMOS[1]). This study is critical as it will serve as a control group for Lysogene's pivotal (phase II/III) trial.

- *In May,* Lysogene announced the successful recruitment of 23 patients in SAMOS. Preliminary data are in line with Lysogene's expectations.

- *Over this time period,* Lysogene selected the SmartFlow® cannula commercialized by the US company MRI Interventions. This cannula will be used in the phase II/III clinical trial for MPS IIIA, which is expected to start in the first half of 2018.

LYS-GM101, Lysogene's second drug candidate for the treatment of GM1 gangliosidosis

- *In February*, Lysogene received the Orphan Drug Designation from the European Medicines Agency (EMA) for LYS-GM101 and from the US Food and Drug Administration (FDA). The FDA also granted the Rare Pediatric Disease Designation to LYS-GM101 earlier this year. Those designations represent major regulatory milestones highlighting the recognition by the authorities that Lysogene's drug candidates are plausible future drugs.

- *In May,* Lysogene signed a manufacturing agreement with a first-class contract manufacturing organization based in the US, for the production of LYS-GM101, using the AAVrh10 vector, to be used in clinical trials to treat patients with GM1 gangliosidosis (expected to start in 2019). The manufacturing plan is on schedule. The manufacturing platform is one of the leading producers of cell and viral vector-based gene therapy products.

[1] Sanfilippo A Multinational Observational Study

Key events since June 30, 2017

In September, Lysogene set up its Clinical Advisory Board ("CAB"), consisting of internationally renowned experts. The CAB will provide strategic advice to Lysogene as it continues to advance its clinical development programs and devise commercialization paths for its orphan gene therapy candidates to treat rare CNS diseases, beginning with Mucopolysaccharidosis Type IIIA (MPS IIIA) and GM1 Gangliosidosis (GM1) patients. The Clinical Advisory Board is expected to hold its first meeting in the last quarter of 2017.

Besides, Lysogene confirms that its phase II/III trial for MPS IIIA will be a multi-center, international trial. Lysogene is continuing to work with the regulatory authorities in Europe (EMA) and USA (FDA), and in particular is working on a Pediatric Investigation Plan (PIP) with the EMA. At the same time, production of GMP batches of LYS-SAF302 for this pivotal trial has recently begun.

Lysogene's Board of Directors, in its meeting on September, 15, noted Sofinnova's resignation from its position at the Board of Directors. In the same meeting, Ms Rafaele Tordjman was appointed as an independent director, and the Board is delighted that it will continue to benefit from her expertise and advice as it has done for over three years now.

Lysogene's financial report for the first six months ended June 30, 2017 is now available to the public and on the Company's website at www.lysogene.com.

Next financial milestones

- Third quarter 2017 revenue and cash position on October 16, 2017 (after market close)

About Lysogene

Lysogene (www.lysogene.com) is a global biopharma leader in orphan CNS disease research and development. Lysogene has generated five non-cumulative years of clinical safety data to show the efficiency of a direct delivery route to the CNS with its initial gene therapy trial for MPS IIIA. Lysogene has recently completed the enrollment for the first multi-national observational study in MPS IIIA which will function as the non-concurrent control for the first pivotal trial for MPS IIIA in H1 2018. Lysogene also plans a clinical trial for GM1 Gangliosidosis for 2019. Lysogene has obtained orphan drug designation from the EMA and FDA and rare pediatric designation by the FDA for both programs.

Lysogene is listed on the Euronext regulated market in Paris (ISIN code: FR0013233475).

For more information: www.lysogene.com.

Contacts

Media:

Europe
Annie-Florence Loyer
NewCap
afloyer@newcap.fr
+ 33 6 88 20 35 59
+ 33 1 44 71 00 12

North America
Marion Janic
RooneyPartners
mjanic@rooneyco.com
+ 1 (212) 223-4017

Investors:

Chris Maggos
LifeSci Advisors
chris@lifesciadvisors.com
+41 79 367 6254