Press release
October 16th, 2017

Lysogene: financial information for the third quarter of 2017

PARIS, France and CAMBRIDGE, MA USA – October 16, 2017 – Lysogene (FR0013233475 – LYS), a leading clinical-stage biopharmaceutical company specializing in gene therapy technology applied to central nervous system diseases (CNS), today reports its cash position at the end of the third quarter of 2017.

Cash position and revenues as of September 30, 2017

As of September 30, 2017, cash and cash equivalents amounted to €17.3 million[1] compared to €21.1 million[1] as of June 30, 2017.

As expected, Lysogene did not generate revenues during the third quarter of 2017.

Key operational highlights since June 30, 2017

- *July 10, 2017:* as part of the 'CNS AAV Gene Therapy' discussion at the 2017 MPS Symposium, Orphan Disease Center's, Philadelphia, United States, Ms. Aiach made a presentation detailing the research that Lysogene is conducting related to Mucopolysaccharidosis Type IIIA[2] (MPS IIIA), one of four types of Sanfilippo Syndrome, a rare and lethal genetic disease that affects the central nervous system. Lysogene is developing a gene therapy treatment for MPS IIIA using a rAAV vector serotype rh.10 that is delivered directly into the CNS. Ms. Aiach's presentation focused on clinical and pre-clinical data, as well as clinical trial design and trial endpoints.

- *August 23, 2017*: Lysogene presented its Gene therapy potency assay development at The Bioprocessing Summit 2017 (Boston) that evaluates the enzymatic activity induced by its AAV-vector.

- *September 12, 2017:* Lysogene launched a world class Clinical Advisory Board composed of the most eminent international gene therapy, neurosurgery and Lysosomal Storage Disease 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 MPS IIIA and GM1 Gangliosidosis[2] (GM1) patients. The launch of the CAB further positions Lysogene to successfully reach its goal of

[1] Unaudited and not subject to approval by the board of directors
[2] These diseases are rare lethal neuropathic lysosomal storage disorders leading cause of patients' premature death

bringing transformational medicines to patients with severe neurological rare diseases. The first meeting is scheduled to take place during the last quarter of 2017.

- *September 21, 2017*: Lysogene held the first meeting of its Parent Advisory Board for parents and caregivers of children with MPS IIIA. The Parent Advisory Board, dedicated solely to patient-related needs, views, and concerns, was organized by Lysogene in collaboration with the National MPS Society, the Cure Sanfilippo Foundation and the Center for Information and Study on Clinical Research Participation (CISCRP). The objectives were to better understand patient and caregiver experiences with MPS IIIA, assess general perceptions of clinical trials for MPS IIIA, and gather input to the proposed clinical trial design for the treatment of MPS IIIA. The value of the meeting more than met Lysogene's expectations. The Company will incorporate this valuable feedback in the design of its pivotal clinical trial in MPS IIIA.

- In *September*, Lysogene also confirmed that its phase II/III study in MPS IIIA will be multi-centric and international. Lysogene is still liaising with the European (EMA) and US (FDA) regulatory authorities, and particularly with the EMA in regard to the Pediatric Investigation Plan (IPP). In parallel, production of the LYS-SAF302 GMP batches for this pivotal study has started.

Upcoming events

- *November 14-15, 2017*: Jefferies 2017 London Healthcare conference (London, UK)
- *November 23-24, 2017*: Salon Actionaria 2017 investors fair (Paris, France)
- *January 11, 2018*: publication of fourth-quarter 2017 cash

About Lysogene

Lysogene is a leading, clinical stage biotechnology company, specializing in the basic research and clinical development of AAV gene therapy for CNS disorders with a high unmet medical need. Since 2009, Lysogene has established a solid platform and network, with lead products in MPS IIIA and GM1, to become a global leader in orphan CNS diseases. Lysogene has obtained ODD from the EMA and FDA and rare pediatric designation by the FDA for both its MPS IIIA and GM1 programs.

Lysogene is listed on the Euronext regulated market in Paris (ISIN code: FR0013233475). For more information, please visit www.lysogene.com.

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