Press release
29 April 2019

Lysogene publishes its Registration Document for the 2018 financial year and recalls the progress of its gene therapy programs

PARIS, France – 29 April 2019, at 7:30pm CEST – Lysogene (FR0013233475 – LYS), a biopharmaceutical company specializing in gene therapy targeting central nervous system (CNS) diseases, today announces the release of its Registration Document for the 2018 financial year, registered by the *Autorité des marchés financiers* under number R.19-016 dated 29 April 2019.

This 2018 Registration Document is available on the websites of Lysogene (http://www.lysogene.com) and the *Autorité des marchés financiers* (http://www.amf-france.org/). Copies are also available on request and free of charge from Lysogene's registered office (18-20 rue Jacques Dulud, 92200 Neuilly-sur-Seine, France).

This 2018 Registration Document includes in particular:

- The 2018 annual financial report;
- The management report; and
- The report of the Chairman of the Board of Directors on corporate governance and internal control.

Investors' attention is drawn to Chapter 4 "Risk Factors" of the Registration Document registered by the AMF.

Development of the drug candidates LYS-SAF302, LYS-GM101 and the X-Fragile project

- The drug candidate LYS-SAF302 developed by Lysogene in MPS IIIA is currently in a Phase 2/3 clinical trial for registration in Europe and the United States. The first patient in clinical trial was recruited in December 2018, and the first patient was treated in February 2019. Lysogene expects to complete the recruitment of the 20 patients of this Phase 2/3 clinical trial in the first half of 2020.

- Lysogene initiates preclinical toxicology studies of the drug candidate LYS-GM101 in GM1 gangliosidosis. The IND request for this drug candidate, prior to the start of Phase 1/2, is scheduled before the end of 2019. The IND is expected to be obtained in the first half of 2020.

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

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

This press release may contain forward-looking statements, in particular regarding the progress of the Company's Phase 2/3 clinical trials statements. 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", "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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