



Lysogene: First Half 2020 Business Update

- **Cash and cash equivalent of €23.8 million¹ as of 30 June 2020**
- **FDA letter on LYS-SAF302 received as planned on July 2, 2020, with no change to expected regulatory timelines**

Paris, France — 24 July 2020 at 08:00am CEST— Lysogene (FR0013233475 – LYS), a Phase 3 gene therapy platform company targeting central nervous system (CNS) diseases, provides H1 2020 Business update.

As of 30 June 2020, cash and cash equivalents amounted to €23.8 million¹ (compared to €29.9 million as of 31 March 2020). This amount includes the €7.7m gross proceeds from the successful capital increase in March 2020 led by OrbiMed and Lysogene shareholder and partner Sarepta. Lysogene considers that this cash position provides the company with sufficient financial runway until Q4 2021.

The license and collaboration agreement with Sarepta Therapeutics, Inc also generated in the first half of 2020 revenues of €9.1 million¹ in application of standard IFRS 15 “Revenues from contracts with customers” and recognized revenues under the percentage-of-completion method.

Moreover, and as expected, the company received on 02 July 2020 the FDA letter confirming the clinical hold for recruitment and treatment of new patients in the clinical trial AAVance (NCT03612869), a global Phase 2/3 clinical trial of LYS-SAF302 for the treatment of MPS IIIA. The clinical hold results from a need for additional information to evaluate the MRI findings, and notably that they are not associated with clinical harm. The company will gather the required information to address these questions as soon as possible. To date, 19 patients out of the 20 planned have been treated, and all patients remain in the study and are being followed per study protocol. The primary and secondary trial endpoints are based on the analyses of these 19 patients already enrolled and there is no anticipated impact on the current development timelines.

¹ Unaudited. Eur/USD exchange rate of 1.1198. Eur/GBP exchange rate of 0.9124

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 phase 2/3 clinical trial in MPS IIIA in partnership with Sarepta Therapeutics, Inc. is ongoing and a phase 1/3 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 an academic partner on a gene therapy approach for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

Forward Looking Statement

This press release may contain certain forward-looking statements, forecasts and estimates with respect to Lysogene's clinical trials, clinical trial data releases, clinical development plans, anticipated future activities and cash runway of Lysogene. 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, (ii) factors beyond the Company's control, (iii) clinical trial results, (iv) increased manufacturing costs and (v) potential claims on its products. 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," "objective", "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 2019 universal registration document, registered with the French Markets Authorities on April 30, 2020, under number D.20-0427, 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.

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