

## Press Release

February 1<sup>st</sup>, 2018

LYSOGENE



## Lysogene to Present Five Year Clinical Data in MPS IIIA at the 14<sup>th</sup> Annual *WORLDSymposium*<sup>TM</sup>

**Paris, France and Cambridge, MA, US — February 1<sup>st</sup>, 2018 at 06:30pm CET** — Lysogene (FR0013233475 – LYS), a gene therapy company pioneering therapies to treat diseases of the central nervous system (CNS), today announced that it will be participating in and presenting at the 14th Annual *WORLDSymposium*<sup>TM</sup> being held Feb. 5-9, 2018, in San Diego, California, U.S.A.

Sophie Olivier, M.D., Chief Medical Officer of Lysogene, will present the results of the phase 1-2 clinical trial and the design of the Company's upcoming pivotal trial in MPS IIIA. As announced last year, Lysogene has formed a robust international natural history study in MPS IIIA which, as agreed with regulatory authorities, will function as a non-concurrent control group. Details on Dr. Olivier's presentation are as follows:

**Presentation Title:** Five Years of Clinical Data in a Direct to CNS Gene Therapy Trial  
**Presenter:** Sophie Olivier, M.D.  
**Date/Time:** Thursday, Feb. 8, at 11:15 a.m. PT  
**Location:** Manchester Grand Hyatt, 1 Market Place, San Diego, California, U.S.A.

Lysogene will also present a poster, entitled: "*Design, baseline characteristics, and early findings of the MPS IIIA (mucopolysaccharidosis type IIIA) clinical observational study in 23 patients*" during the poster session on February 7th from 4:30 pm to 6:30 pm.

The *WORLDSymposium*<sup>TM</sup> is a leading annual research conference dedicated to lysosomal diseases. Since 2002, the W.O.R.L.D. (*We're Organizing Research on Lysosomal Diseases*) meeting has grown to an international research conference that attracts over 1600 participants from more than 50 countries around the globe.

### About Lysogene

Lysogene is a gene therapy company focused on orphan diseases of the central nervous system (CNS), and a leader in MPS IIIA. 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 first gene therapy trial for MPS IIIA. The company 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 pivotal trial for MPS IIIA that is expected to start in H1 2018. A phase 1 clinical trial for GM1 Gangliosidosis is expected to start 2019. Both programs have orphan drug designation from the EMA and the U.S Food and Drug Administration (FDA) and rare pediatric designation from the FDA.

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

For more information: [www.lysogene.com](http://www.lysogene.com).

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