

## Press Release

July 10, 2017

LYSOGENE



**FOR IMMEDIATE RELEASE**

## Lysogene to Present at The Orphan Disease Center's 2017 MPS Symposium

**PARIS, France, and CAMBRIDGE, Mass., US — July 10, 2017 at 07:00am** — Lysogene (FR0013233475 – LYS), a leading clinical-stage biopharmaceutical company specializing in gene therapy technology applied to central nervous system diseases, announced today that Karen Aiach, CEO and Founder of Lysogene, will present at The Orphan Disease Center's 2017 MPS Symposium. The symposium is scheduled to take place on Monday, July 10 and Tuesday July 11 in Philadelphia.

The following are specific details regarding Lysogene's presentation:

Event: **2017 MPS Symposium**

Date: July 10, 2017

Time: 3:00 pm ET

Location: The Inn at Penn, 3600 Sansom Street, Philadelphia, PA 19104

Ms. Aiach will make a 15 minute presentation as part of the discussion 'CNS AAV Gene Therapy'. Specifically, she will discuss the research that Lysogene is conducting related to 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.

The presentation from Ms. Aiach will focus on clinical and pre-clinical data, as well as clinical trial design and trial endpoints. A panel discussion will follow the presentations and will be hosted by the session chairs.

### About The Orphan Disease Center

The Orphan Disease Center was formed in 2011 with a generous gift from philanthropist and Wharton graduate, George A. Weiss. The Center, which is the first of its kind, was created to facilitate translational research and therapy development to address the prevalence of certain orphan diseases that affect a marginalized population of individuals internationally. The goal of the Orphan Disease Center is to address these growing needs through continued collaboration and partnership, identifying and providing funding sources, and by providing technological and educational resources to academic researchers, biotech and pharmaceutical companies alike, to foster therapeutic development and innovative research initiatives.

## **Lysogene is Targeting Treatment for the Neurological Symptoms of MPS IIIA:**

MPS IIIA is a lethal CNS disease requiring targeted treatment. Lysogene's gene therapy candidate for MPS IIIA is a rAAV vector serotype rh.10 carrying the gene coding for SGSH. This in vivo gene therapy offers the possibility of a one-time treatment by inserting a healthy copy of the SGSH gene and allowing the body to start making the missing enzyme, therefore slowing or halting disease progression. Lysogene's gene therapy is delivered directly to the CNS in one neurosurgical procedure. By delivering the missing SGSH gene, Lysogene believes MPS IIIA patients will be provided a permanent source of functional enzyme in the brain that reverses phenotypic abnormalities of CNS neural cells.

## **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 Mucopolysaccharidosis Type IIIA and GM1 Gangliosidosis, to become a global leader in orphan CNS diseases. Lysogene has also obtained ODD by 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](http://www.lysogene.com).



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