Press Release
May 30th, 2017

Lysogene Completes Enrollment in First International Pivotal Observational Study in MPS IIIA

- Rapid recruitment of 23 patients in less than a year
- Consensually agreed assessment tools (BSID-III, VABS-II) for cognitive assessment & behavior

FOR IMMEDIATE RELEASE

PARIS, France, and CAMBRIDGE, MA, US – May 30th, 2017, 7:00 am CET – Lysogene (FR0013233475 – LYS), a leading, biopharmaceutical company pioneering gene therapy technologies to treat central nervous system diseases, today announced the completion of enrollment in its "Sanfilippo A Multi-national Observational Study" (SAMOS). Sanfilippo A is also known as Mucopolysaccharidosis Type IIIA (MPS IIIA).

SAMOS is particularly important as there is currently no validated biomarker in MPS IIIA that reflects CNS disease progression and response to future therapy. SAMOS has therefore been designed to evaluate clinical change in untreated MPS IIIA patients. As agreed with the regulatory authorities, this international multi-center study is to function as a non-concurrent control group for the upcoming Lysogene Phase II/III pivotal gene therapy trial, scheduled to start during the first quarter of 2018.

"*Lysogene has taken a very proactive and rigorous approach to gaining a better understanding of MPS IIIA*," said Dr. Benedicte Heron, neuro-pediatrician (Armand-Trousseau Hospital, APHP Paris, France). "*The company's efforts in running this observational study will support its own plans for future research and therapy development while also aiding the scientific community as a whole.*"

"*Enrollment of the 23 children from 5 countries has been rapid, reflecting the strong interest from the key opinion leaders running our clinical sites and the network of patient associations to address the significant unmet needs in MPS IIIA*," stated Samantha Parker, Chief Patient Access Officer at Lysogene.

In designing SAMOS, Lysogene established the first international neurologist and neuropsychologist MPS IIIA expert group. This group determined that the most scientifically rigorous and relevant primary endpoint was cognitive assessment using the *Bayley Scales of Infant and Toddler Development*, 3rd edition (BSID-III). The *Vineland Adaptive Behavior Scale*, 2nd edition (VABS-II) was determined to be the most appropriate as a secondary endpoint measure.

Further validating the choice of endpoints for neuronopathic MPS, a consensus meeting, among international experts, was organised in London, 2-3 December 2016, by an academic group in consultation with the UK Society for Mucopolysaccharide Diseases and US National MPS Society, and chaired by Elsa Shapiro[1]. From all the available instruments the BSID-III is recommended to measure cognitive outcomes and the recommended instrument to measure adaptive behavior is the VABS-II, using the extended interview format (*van der Lee, 2017)*.[2]

Lysogene previously issued baseline data from SAMOS assessing cognitive age using the BSID-III in the first 15 patients, aged between 3 and 8 years old, which confirmed the progressive intellectual decline, hyperactivity and behavior changes in these individuals.

Lysogene has also successfully completed a Phase I/II trial and 5-year follow-up study of four MPS IIIA patients with no adverse events related to the treatment.[3]

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.

For more information on SAMOS: www.ClinicalTrials.gov Identifier: NCT02746341.

About Lysogene

For more information: www.lysogene.com.

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[2] J.H. van der Lee, et al., Cognitive endpoints for therapy development for neuronopathicmucopolysaccharidoses: Results of a consensus procedure, Mol. Genet. Metab. (2017), http://dx.doi.org/10.1016/j.ymgme.2017.05.004
[3] Tardieu, M., et al. Intracerebral Administration of Adeno-Associated Viral Vector Serotype rh.10 Carrying Human SGSH and SUMF1 cDNAs in Children with Mucopolysaccharidosis Type IIIA Disease: Results of a Phase I/II Trial, Human Gene Therapy (2014), http://online.liebertpub.com/doi/abs/10.1089/hum.2013.238

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