Press Release
March 28th, 2017

Lysogene Announces Baseline Data from First International Pivotal Observational Study in MPS IIIA

Study will Function as Pivotal Trial Clinical Control and Assist in a Better Understanding of the Disease

FOR IMMEDIATE RELEASE

PARIS, France, and CAMBRIDGE, MA, US – March 28th, 2017 – Lysogene (FR0013233475 – LYS), a leading, biopharmaceutical company pioneering in gene therapy technology applied to central nervous system diseases, today announced baseline data from its Sanfilippo A Multi-national Observational Study (SAMOS). Data from this first international pivotal observational study was the topic of a poster presentation made at the 13th Annual WORLD*Symposium™* in San Diego, Calif. Sanfilippo A is also known as Mucopolysaccharidosis Type IIIA (MPS IIIA).

SAMOS has been designed to evaluate the clinical progression in untreated MPS IIIA patients. As agreed with the regulatory authorities, this study is to function as a non-concurrent control for the upcoming Lysogene phase II/III pivotal gene therapy trial.

Toward refinement of the study design, including selection of appropriate clinical endpoints and assessment tools, Lysogene formed a clinical expert panel and established the first international neurologist and neuropsychologist expert group.

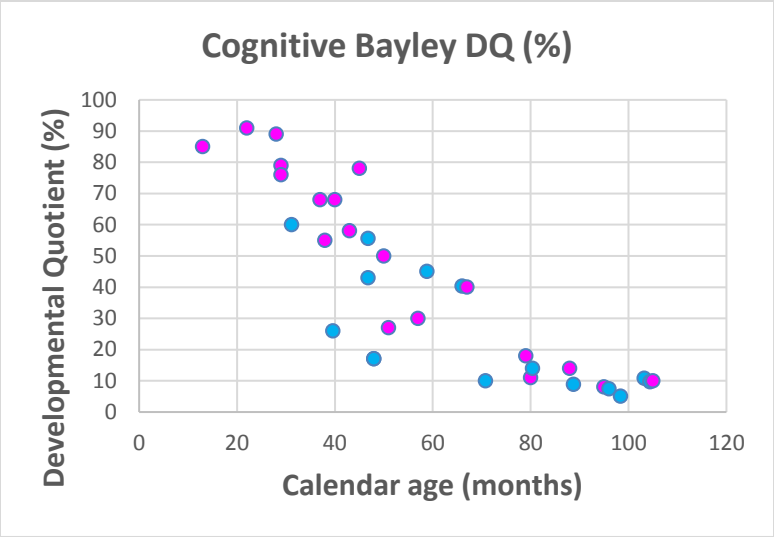
As a result of this advisory meeting, the most relevant primary endpoint was determined to be cognitive assessment using the **Bayley Scales of Infant and Toddler Development, 3rd edition (BSID-III),** and the **Vineland Adaptive Behaviour Scale, 2nd edition** was defined as a useful secondary endpoint measure. The cognitive assessment is particularly important in MPS IIIA in the absence of **a validated biomarker** that correlates with CNS disease progression and clinical response to future therapy.

Cognitive age assessed on the first 15 patients, aged between 3 and 8 years old, using the BSID-III confirms the progressive intellectual decline, hyperactivity and behaviour changes in these individuals. Lysogene's collaborative approach has allowed for meta-analysis with a natural history study performed at the University of Minnesota (Shapiro et al., 2015).

"*This baseline data is just the first of several sets of data points that we hope to glean from this investigational study in order to further understand Sanfilippo A*," stated Soraya Bekkali, Chief Medical

Officer**.** "*We anticipate that this and forthcoming data will represent new learnings in this disease area and we know it will prove extremely valuable as Lysogene prepares for future pivotal studies in MPS IIIA"*

Figure 1: Merging Lysogene data with published data on Developmental Quotient (DQ) based on BSID-III at baseline 15 Lysogene patients (blue dots), 19 patients from published data (pink dots)-(Shapiro 2016)



Source: Lysogene internal data merged with data from Shapiro (2015)

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

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

www.lysogene.com

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