



## **Lysogene Holds First Parent Advisory Board in MPS IIIA**

- *First Advisory Board comprised of parents of children with MPS IIIA, a rare and lethal disease of the central nervous system (CNS)*
- *Advisory Board provides valuable insight concerning parents' expectations of therapy and the urgent need to address the neurological component of the disease*
- *Parents support the feasibility of Lysogene's recruitment strategy and trial design*

### **FOR IMMEDIATE RELEASE**

**PARIS, France, and CAMBRIDGE, MA, US – September 21<sup>st</sup> 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 first meeting of the Parent Advisory Board for parents and caregivers of children with Mucopolysaccharidosis Type IIIA (MPS IIIA), also known as Sanfilippo A, in August, 2017 in Minneapolis (US).

The Parent Advisory Board, dedicated solely to patient-related needs, views, and concerns, was organized by Lysogene in collaboration with the National MPS Society, the Cure Sanfilippo Foundation and the Center for Information and Study on Clinical Research Participation (CISCRP). The objectives were to better understand patient and caregiver experiences with MPS IIIA, assess general perceptions of clinical trials for MPS IIIA, and solicit thoughts or reactions to proposed clinical trial design for the treatment of MPS IIIA.

All Advisory Board members had experience as a parent or caregiver of a patient with MPS IIIA and provided their personal experiences with the disease and the challenges of clinical trial participation. During the meeting, parents confirmed the significant life-changing impact this disease has on families and that symptoms and severity can vary between children. Children with MPS IIIA suffer neurological symptoms, including developmental delay, difficulty sleeping, hyperactivity, and issues with motor functions (e.g., difficulty grasping or head turning one way).

“Lysogene is dedicated to placing the patient at the center of the clinical trial design, and feedback from the Parent Advisory Board will be a tremendous addition to our efforts,” said Samantha Parker, Lysogene’s Chief Patient Access Officer. “The value of the meeting was beyond our expectations, and we thank the families for their candid input. We intend to use the valuable feedback in the design of our pivotal clinical trial in MPS IIIA.”

“We appreciate the opportunity to organize this Parent Advisory Board in conjunction with Lysogene as it gives voice to families impacted by this devastating disease,” said Annick Anderson, Director of Research Services at CISCRP. “Understanding the path families take in getting a diagnosis and the challenges in caring for children with MPS IIIA will ultimately result in better treatments for patients.”

**About CISCRP:** [www.CISCRP.org](http://www.CISCRP.org)

**About Lysogene:** [www.lysogene.com](http://www.lysogene.com)

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