

Press Release

October 5th 2017

LYSOGENE



Lysogene to Host Key Opinion Leader Meeting on Gene Therapy for Patients with Sanfilippo Syndrome Type A in New York City

- *Presentation by a Key Opinion Leader, Ronald G. Crystal, MD, Professor and Chairman of the Department of Genetic Medicine at Weill Cornell Medicine*

PARIS, France, and CAMBRIDGE, MA, US – October 5th, 6 PM CET – Lysogene (FR0013233475 – LYS), a leading, biopharmaceutical company pioneering gene therapy technologies to treat central nervous system diseases, today announced that it will host a Key Opinion Leader (KOL) meeting on the topic of *Gene Therapy for Patients with Sanfilippo Syndrome Type A (MPS IIIA)* on Friday, October 13th from 12:00 PM to 1:30 PM EDT in New York City.

The meeting will feature a presentation by Key Opinion Leader, **Ronald G. Crystal, MD** (Professor and Chairman of the Department of Genetic Medicine at Weill Cornell Medicine), who will discuss the current landscape for using gene therapeutics for treating patients with CNS degenerative disorders such as MPS IIIA. Dr. Crystal will be available to answer questions following the lunch.

Lysogene's management team will also provide an overview of the Company's ongoing clinical development program with their *in vivo* gene therapy candidate for MPS IIIA, which is a rAAV vector serotype rh.10 carrying the gene coding for SGSH. 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 corrects phenotypic abnormalities in the CNS.

This event is intended for institutional investors, sell-side analysts, investment bankers, and business development professionals only. Please [RSVP](#) in advance if you plan to attend, as space is limited. For those who are unable to attend in person, a live webcast and replay will be accessible [here](#).

About Ronald G. Crystal, MD

Ronald G. Crystal, MD, is Professor and Chairman of the Department of Genetic Medicine at Weill Cornell Medicine, where he is also the Bruce Webster Professor of Internal Medicine, Director of the Belfer Gene Therapy Core Facility and Attending Physician at NewYork-Presbyterian/Weill Cornell Medical Center. Dr. Crystal is a pioneer in the field of gene therapy. He was the first to use a recombinant virus as a vehicle for *in vivo* gene therapy, and has carried out human trials of gene therapy for cystic fibrosis, cardiac ischemia, cancer and central nervous system disorders. His laboratory also has major programs in deciphering how human genetic variation modulates gene expression in the context of environmental exposure and exploiting these relationships to recategorize human disease at the biologic level and identify who is at risk for disease. Dr. Crystal serves on the editorial boards of numerous biomedical journals. He has published over 850 scientific articles, and his work has been cited over 50,000 times in the scientific literature. He has edited several textbooks, and serves on a number of advisory boards to government and industry; he is a paid member of Lysogene's clinical advisory board. Dr. Crystal is responsible for numerous biomedical patents and is a founder of several biotechnology companies focused on developing gene therapy therapeutics.

About Lysogene : www.lysogene.com

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