Press Release
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Lysogene Establishes Scientific Advisory Board with International Experts in CNS Gene Therapy

FOR IMMEDIATE RELEASE

PARIS, France – July 18, 2017, at 06:00pm CET – Lysogene (FR0013233475 – LYS), a leading, Phase 3, biopharmaceutical company pioneering gene therapy technologies to treat central nervous system (CNS) diseases, today announced the formation of a Scientific Advisory Board (SAB) comprised of world-leading experts in gene therapy discovery and development.

“Gene therapy using recombinant adeno-associated virus (AAV) vectors is changing the course of previously untreatable monogenic diseases,” said Dr. Ronald Crystal, Chairman of the Department of Genetic Medicine and the Bruce Webster Professor of Internal Medicine at Weill Cornell Medicine and member of the newly established Scientific Advisory Board. “Direct to CNS approaches have demonstrated promising safety and tolerability with the potential of achieving high bioavailability and CNS distribution. I look forward to working with Lysogene and the members of the SAB to further explore the potential of this approach to treat CNS diseases.”

"The establishment of this SAB reflects our commitment to becoming a leading company in the field of CNS gene therapy,” continued Dr. Ralph Laufer, Chief Scientific Officer at Lysogene. “The vision of these international renowned experts, in this very promising field, will considerably reinforce our expertise and support our goal of establishing a new paradigm for the treatment of monogenic neurological diseases.” The SAB is a key strategic resource to Lysogene providing scientific expertise and guidance to the team as the Company continues to advance gene therapy candidates to treat CNS diseases, including LYS-SAF302 - currently in Phase 2-3 - for the treatment of Sanfilippo syndrome type A (MPS IIIA), LYS-GM101 - soon to enter Phase 1-2 - for the treatment of GM1 Gangliosidosis and preclinical proof of concept program in Fragile X syndrome.

“We are excited and privileged to have the opportunity to work with this distinguished group of experts on our gene therapy development," said Dr. Sophie Olivier, Chief Medical Officer at Lysogene. "These thought-leaders bring tremendous understanding of gene therapy potential for CNS diseases, as well as extensive experience from discovery to late-stage clinical trials. We look forward to their contributions as Lysogene advances its gene therapy pipeline."

The SAB members are:

Nathalie Cartier-Lacave, Ph.D., Cell and Gene Therapy for Neurodegenerative Diseases, INSERM ICM (*Institute for Brain and Spine*). Dr Cartier-Lacave is a research director at INSERM, where she leads the group "Biotherapies of neurodegenerative diseases". Dr Cartier-Lacave is president of the European Society of Gene and Cell Therapy (ESGCT).

Ronald G. Crystal, M.D., Professor and Chairman, Department of Genetic Medicine, Weill Cornell Medicine. Dr. Crystal performed pioneering seminal work on adenoviral and AAV vectors, which accelerated the translation of gene therapy from the research laboratory to the clinic. His current research focus includes optimizing viral gene transfer vectors for in vivo gene therapy for hereditary and acquired brain disorders. Dr. Ronald Crystal is a paid consultant for Lysogene. He has also conducted lectures, with full content control, supported by Lysogene.

Steven Gray Ph.D., Associate Professor, Department of Pediatrics, UT Southwestern Medical Center. Dr. Gray's core expertise is in AAV gene therapy vector engineering, followed by optimizing approaches to deliver a gene to the nervous system and preclinical studies to apply AAV vectors toward the development of treatments for neurological diseases.

Chester B. Whitley, Ph.D., M.D., University of Minnesota Medical School, Minneapolis. Dr Whitley is a key opinion leader in the field of lysosomal diseases, founding Course Director for WORLD*Symposium*, and Principal Investigator for the global Lysosomal Disease Network. He accomplished the first clinical trial of gene therapy for a mucopolysaccharidosis disease (1998-9).

About Lysogene

Lysogene is a gene therapy company focused on the treatment of orphan diseases of the central nervous system (CNS). The company has built a unique capability to enable a safe and effective delivery of gene therapies to the CNS to treat lysosomal diseases and other genetic disorders of the CNS. A pivotal clinical trial in MPS IIIA in partnership with Sarepta Therapeutics, Inc. is ongoing and a phase 1-2 clinical trial in GM1 Gangliosidosis is in preparation. In accordance with the agreements signed between Lysogene and Sarepta Therapeutics, Inc., Sarepta Therapeutics, Inc. will hold exclusive commercial rights to LYS-SAF302 in the United States and markets outside Europe; and Lysogene will maintain commercial exclusivity of LYS-SAF302 in Europe. Lysogene is also collaborating with an academic partner to define the development strategy for the treatment of Fragile X syndrome, a genetic disease related to autism. www.lysogene.com.

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Lysogene's forward-looking statements

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