FOR IMMEDIATE RELEASE

Lysogene to Host First-Ever Research Symposium Dedicated to GM-1 Gangliosidosis

Partners with Cure GM-1 Foundation and the National Tay-Sachs and Allied Diseases Association

Paris—March 9, 2016—Lysogene, a leading clinical-stage biotechnology company developing gene therapy for rare disease, today announced its sponsorship of the first-ever scientific workshop focused solely on GM-1 gangliosidosis (GM1) research for families and others with an interest in the disorder. GM-1 is a severe rare lysosomal storage disorder with a birth prevalence estimated at 1 per 200,000. Cynthia Tifft, M.D., Ph.D., Director of the Pediatric Undiagnosed Diseases Program at the National Human Genome Research Institute/NIH, a preeminent GM-1 research scientist and clinician, will chair the workshop on Saturday, April 9, 2016. It is being held in conjunction with the National Tay-Sachs and Allied Diseases Association (NTSAD) 38th Annual Family Conference in Orlando, Florida.

Dr. Tifft will open the workshop with an overview presentation on GM-1. Expert scientists will then discuss the latest research and therapeutic approaches, including gene therapy, pharmacological chaperones, and enzyme replacement therapy.

A live broadcast will be openly available on NTSAD’s YouTube channel and will be accessible through the NTSAD, Cure GM1 Foundation and Lysogene websites.

“We are very grateful for this opportunity to bring together those affected by GM-1 Gangliosidosis for this unique workshop,” said Christine Waggoner, Cure GM-1 Foundation President “with so few biotechnology companies working on GM-1 treatments, Lysogene shines very brightly in the constellation of hope.”

“NTSAD welcomes the opportunity to bring an additional dimension to our Annual Family Conference, and the addition of Cure GM-1 Foundation to our family.” said Susan Kahn, Executive Director, NTSAD, “The more resources and attention we bring to all rare disease, the sooner we will see the realization of our hopes for successful new therapies for patients and their families.”

The NTSAD Annual Family Conference is organised for families and individuals affected by Tay Sachs and related diseases, including GM1, to enable the sharing of experiences and information, to bring understanding, care and support and for the many families impacted by these rare genetic disorders.

Families and researchers interested in attending are invited to register for the Conference, which comprises sessions of support, experts and resources. Register here or contact the NTSAD office for more information.

About Cure GM1 Foundation
The Cure GM1 Foundation is dedicated to hope and to directly funding research for a cure for GM1 Gangliosidosis, a lysosomal storage disease that attacks the brain and spinal cord and is always fatal.
in children. GM1 is a progressive and degenerative condition with an extremely broad and debilitating array of symptoms and complications. This non-profit organization was founded by parents of children who suffer from GM1 who seek to save the lives of all those who suffer from this wretched condition.

About NTSAD
The nation’s longest-standing rare disease advocacy organization, National Tay-Sachs & Allied Diseases Association (NTSAD), founded in 1957, funds research toward treatments and a cure for Tay-Sachs and related genetic neurodegenerative lysosomal storage diseases and leukodystrophies. NTSAD also provides comprehensive support to affected families worldwide. Having pioneered community education about carrier screening that became a model for all genetic diseases, NTSAD’s education initiatives promote screening and prevention to the public and healthcare community. More information about NTSAD, Tay-Sachs, GM-1, Canavan, Sandhoff and related diseases is available at http://www.ntsad.org.

About Lysogene
Lysogene is a clinical stage biotechnology company pioneering in the basic research and clinical development of AAV gene therapy for CNS disorders with a high unmet medical need. Since 2009, Lysogene has established a unique platform and network, with lead products in Mucopolysaccharidosis Type A (Sanfilippo A) and GM1 Gangliosidosis, to become a global leader in orphan CNS diseases.

For more information www.lysogene.com.

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