FOR IMMEDIATE RELEASE

Media Contact:
Edna Kaplan
kaplan@kogspr.com
781-639-1910

National Tay-Sachs & Allied Diseases Association Receives U.S. Orphan Drug Designation for Novel Gene Therapy

Investigational Treatment for Tay-Sachs and Sandhoff Diseases in Phase 1 Planning

Boston, MA, June 12, 2013 -- The U.S. Food and Drug Administration (FDA) has granted orphan drug designation to National Tay-Sachs and Allied Diseases Association (NTSAD) for development of the first–ever treatment for Tay-Sachs and Sandhoff, rare diseases that are fatal in young children and extremely debilitating in their late-onset form.

Orphan drug designation, which is intended to facilitate drug development for rare diseases, provides substantial benefits to the sponsor, including the potential for funding of certain clinical studies, study-design assistance and several years of market exclusivity for the product upon regulatory approval.

The gene therapy in development would correct an enzyme deficiency that causes the progressive neurodegeneration that marks these diseases. Both Tay Sachs and Sandhoff are lysosomal storage diseases, a group of more than 50 genetically inherited disorders characterized by deficiency of a vital enzyme that prevents the proper breakdown of undigested material inside cells.

“This orphan drug designation is a giant step forward in our efforts to bring hope to Tay-Sachs patients and their families, as today there are no treatments,” said NTSAD President, Shari Ungerleider. “Gene therapy has the potential to be a one-time transformative therapy for patients suffering from rare neurodegenerative genetic disorders such as Tay-Sachs. NTSAD, along with its funding partners, is committed to advancing the clinical and commercial development of our gene therapy platform because of the potential life-changing benefit it could have for patients and their families.”

Based on promising results of animal studies that have been ongoing since 2007, the Tay-Sachs Gene Therapy Consortium research team is completing pre-clinical studies in advance of a Phase I clinical trial.

About the Tay-Sachs Gene Therapy Consortium

The Tay-Sachs Gene Therapy (TSGT) Consortium was founded in 2007 to advance human clinical trials in the quest for a gene therapy treatment for Tay-Sachs and Sandhoff diseases. The multidisciplinary team, led by Miguel Sena Esteves, Ph.D., recipient of the 2011 Outstanding New Investigator Award from the American Society of Gene & Cell Therapy, includes scientists and clinicians from Auburn University, Boston College, Cambridge University-UK, Massachusetts General Hospital/Harvard Medical School, University of Massachusetts Medical School, and New York University Medical School.

About NTSAD

The oldest rare disease advocacy organization in the nation, National Tay-Sachs and Allied Diseases Association (NTSAD) was founded in 1957 by concerned parents whose children were affected by Tay-Sachs disease or related rare genetic lysosomal storage diseases and leukodystrophies. Today NTSAD continues its multifaceted support of affected families and funds research seeking a treatment or cure. NTSAD also pioneered the development of community education about carrier screening programs for Tay-Sachs and related diseases, which became models for all genetic diseases. More information is available at http://www.ntsad.org.

###