National Tay-Sachs & Allied Diseases Announces Research Initiative Grant Recipients

Nation's first patient advocacy organization awards five grants totaling $266,000

September 18, 2015—BOSTON, MA—National Tay-Sachs & Allied Diseases Association (NTSAD), the nation's first patient advocacy organization, today announced the awardees of its 2015 Innovative Research Grants. Reflecting an ongoing commitment to fund research aligned with its mission to lead the fight to treat and cure Tay-Sachs (TSD), GM-1, Sandhoff, Canavan (CD) and other related genetic diseases, NTSAD has solicited proposals for basic, translational or clinical research projects. This year's grants include projects related to newborn screening, gene therapy delivery methods, animal models and international patient registries.

"NTSAD thanks this year's applicants, who submitted our highest-quality pool of proposals to date and reflect strong interest in addressing very pressing patient and scientific needs related to these diseases," said Sue Kahn, Executive Director, NTSAD. "We are gratified to see such innovative ideas aligned with the NTSAD mission."

This year's solicitation and grants reflect the inclusion of Clinical Trial Readiness as a primary strategic objective. Clinical Trial Readiness includes initiatives such as natural history studies, development of patient registries, biomarkers, clinical trial endpoints, and newborn screening.

Five grants of up to $40,000 each per year were selected through a multistep process, with final awards chosen by the experts from NTSAD's Scientific Advisory Committee with inputs from NTSAD's Corporate Advisory Council. Three of them address Clinical Trial Readiness. The one and two-year projects are:

Development and validation of a rapid, MS/MS-based method to detect Hexosaminidase deficiency in Tay-Sachs disease
Denis C. Lehotay, Ph.D., Queens University

Intravascular gene therapy for feline GM2 gangliosidosis
Douglas R. Martin, Ph.D., Auburn University

Defining the Natural History of Canavan Disease through Development of an International Registry
Heather A. Lau, M.D. and Paola Leone, Ph.D., New York University

** Grant co-funded by the Canavan Foundation*

Registry and Repository for Late Onset GM2 Gangliosidoses
Florian S. Eichler, M.D., Massachusetts General Hospital
** Funded by the Katie & Allie Buryk Research Fund*

Generation of a knock-in mutant *Hexb* mouse model
Eric R. Sjoberg, Ph.D., OrPhi Therapeutics
** Funded by the Katie & Allie Buryk Research Fund*

For more information about these grants, please see NTSAD.org.

"NTSAD, and their funding partners, fund the highest quality and relevant research with the goal of preparing for and reaching clinical trials," said Frances Platt, PhD, Professor of Biochemistry and Pharmacology at University of Oxford, and Chair of NTSAD's Scientific Advisory Committee. "These grants reflect a portfolio of therapeutic approaches and incorporate development of tools to better understand and measure disease progression."

About NTSAD Research Grants
NTSAD funds promising research and supports more than 500 affected families and individuals worldwide. The organization's educational programs raise awareness of how to prevent Tay-Sachs, Canavan, Sandhoff, GM-1 and related neurodegenerative diseases, which are each caused by an enzyme deficiency and are frequently life limiting. NTSAD gives help and hope to thousands of individuals and families from many backgrounds and ethnicities who have been affected by lysosomal storage diseases.

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Note to editors: Photos available upon request. Local families affected by Tay-Sachs are available for interviews.

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