



**National Tay-Sachs & Allied
Diseases Association, Inc.**

Request for Proposals

The National Tay-Sachs & Allied Diseases Association Inc. (NTSAD) is considered to be the nation's oldest genetic disease nonprofit organization. The "Allied Diseases" represent a family of almost 100 rare diseases including lysosomal storage diseases (LSD) and leukodystrophies affecting the central nervous system. A major element of NTSAD's mission is to lead the fight to treat and cure Tay-Sachs, Canavan and related genetic diseases; therefore, NTSAD is committed to funding research projects that advance this mission.

Grants will be awarded for an initial period of one year at \$40,000 direct costs per year (10% indirect cost rate); funding for a second year is predicated by adequate progress during year 1. The application will use a two-step process. First, a one page pre-application will be submitted. From these pre-applications, investigators will be chosen to submit a full application. The application format and review criteria are specified on page 2 of this RFP. The deadline for submission of pre-applications is January 2, 2015.

NTSAD is soliciting proposals for innovative research projects that involve basic research, translational studies or clinical studies in the areas of neurodegenerative disorders affecting the CNS, including lysosomal storage disorders (particularly Tay-Sachs, GM-1, Sandhoff) and pediatric leukodystrophies (in particular Canavan disease). Basic research and translational studies should generate strong preliminary data to enable future major funding by other third parties. Projects may be in such areas as drug delivery to the brain, new animal models designed to facilitate translational research and drug discovery, assay development for drug screening, substrate reduction, stem cells, molecular chaperones, gene therapy, and biomarkers, as well as exploring other novel therapeutic strategies.

NTSAD's updated research strategy has identified the development of Clinical Trial Readiness as a primary strategic objective. Therefore, in addition to soliciting proposals for novel research projects, we strongly encourage applicants to submit proposals that address one or more of the following: understanding disease progression and natural history as well as unmet needs from a

patient perspective across the severity spectrum of any given indication, development of patient registries, translational biomarkers with clinical utility, measurable and clinically meaningful efficacy endpoints for clinical trials, clinical research networks, and newborn screening.

The pre-applications will be reviewed by sub-committees of NTSAD's Scientific Advisory Committee and Corporate Advisory Council. Selected applications will be invited to submit full applications for further peer review and funding consideration. Grant awards are based on proposal evaluation by a Research Evaluation Subcommittee of NTSAD's Scientific Advisory Committee. The grant awards will be announced in April. The funding period begins approximately on July 1, 2015, pending the Institution's agreement to NTSAD's Research Grant Policies.

Content of the 1-page pre-application

- Title, PI and co-PIs and associated institutions
- Overall goal of the project
- 3-5 Bullet points outlining how the application meets the RFA criteria
- Brief background of the investigator and the research proposal
- Specific Aims to be achieved over year 1 and year 2 provided as a brief list
- Requested resources in terms of 1 year funding and expected total costs for a two year funding period

Proposal Content for the Full Application (page limits noted below)

- **Executive Summary:** Major objectives and impact of research. *(1 page)*
- **Detailed Research Plan:** Design of research; process for collecting and analyzing data; potential risks and their management; outline of research for each year in the proposal including defining milestones, which will be reported in six month interim report(s) to demonstrate progress relative to budget. *(up to 3 pages)*
- **Detailed Budget:** To be linked to the schedule as described above. The first half-payment (50%) will occur at inception of project. A second quarter-payment (25%) will occur upon completion of the interim six-month progress and budget report, and the final quarter-payment (25%)
- **Curriculum Vitae for Principal Investigator and key personnel.** Please include the CV in NIH bio-sketch format.
- **Facility description:** An outline of the facility or laboratory in which research will take place with a listing of existing equipment applicable to project. *(not to exceed 1 page)*

- **Other sources of financial and collaborative support:** This should include support already applied for (even if in process) and/or received. *(not to exceed 1 page)*

Funding awarded will be up to \$40,000 for one year or up to \$80,000 for two years, inclusive of indirect costs not to exceed 10%. The two-year grants will be reviewed after one year to assess satisfactory progress.

The deadline for the one page pre-application is 5:00 p.m. (EST), Friday, January 2, 2015. It should be submitted to:

Sue Kahn
Executive Director, NTSAD
Phone: 617-277-4463
E-mail: skahn@ntsad.org

Sue will be available for questions up to and including December 19, 2014.

Timelines:

Pre-application deadline: Friday, January 2, 2015

Invitation to submit a full application by: Thursday, January 22, 2015

Full application deadline (by invitation only) Monday, February 16, 2015

Earliest anticipated start date: Wednesday, July 1, 2015