



National Tay-Sachs &
Allied Diseases Association



NTSAD Research Initiative Program Request for Proposals (RFP)

About Us

National Tay-Sachs & Allied Diseases Association (NTSAD) is one of the oldest patient advocacy organizations in the country. NTSAD's mission is to lead the fight to treat and cure Tay-Sachs, Canavan, GM-1, and Sandhoff diseases by driving research, forging collaboration, and fostering community. Supporting families is the center of everything we do. NTSAD remains committed to advancing and funding research, and first launched its Research Initiative in 2002, which to date has made direct investments of more than \$4 million in grants that have been leveraged to more than \$30 million of investments in additional research, leading to new therapies in Tay-Sachs, Canavan, GM1, and Sandhoff diseases.

In addition to funds from NTSAD, the Cure Tay-Sachs Foundation (CTSF) and the Blu Genes Foundation (BJF) are partnering with NTSAD in this funding round and will support selected projects that align with the foundations' missions and grant-making priorities. Please note CTSF and Blu Genes' funding priorities are limited to research relating to Tay-Sachs and Sandhoff disease (GM2 gangliosidosis) and gene therapy research for rare disease, with a priority for Tay-Sachs, respectively.

Award Description

Funding will be awarded up to \$70,000 for one year or up to \$140,000 for two years, inclusive of indirect costs not to exceed 5%. Two-year grants will be reviewed after the first year to assess sufficient progress before a second year of funding is provided.

Research Goals

NTSAD's Research Initiative Program is soliciting proposals for innovative research projects that involve basic research, translational studies or clinical studies in the following diseases: Tay-Sachs, Canavan, GM1, and Sandhoff diseases. Basic research and translational studies are strongly encouraged to generate strong preliminary data to enable major funding by other third parties in the future.

The Research Initiative is interested in all aspects of therapeutic discovery. Projects that focus on understanding the pathophysiology and the role of inflammation in these four disease areas, or the process of myelination and how it is affected by disease are of particular interest.

In addition to soliciting proposals for novel research projects, applicants are strongly encouraged to submit proposals for clinical tools that support future trials across the severity spectrum of any given indication. This may include developing severity scales for disease staging, translational biomarkers with clinical utility, or measurable and clinically meaningful efficacy endpoints for clinical trials.

Application Process and Grant Review

There is a two-step application process. First, a one-page letter of intent will be submitted. The letter of intent will be reviewed by a sub-committee of NTSAD's scientific advisors. A select group of applicants will be invited to submit full applications for further review and funding consideration. An invitation to submit a full application is not a commitment to funding.

Pre-application

The pre-application should include:

1. Title of project, names of Principal Investigator (PI) and co-PIs and associated institutions.
2. Overall goal of the project.
3. 3-5 bullet points outlining how the application meets the RFP criteria.
4. Statement of translation describing how the proposed study can be translated into therapeutic development.
5. Specific aims.
6. Requested resources.

Additional pages: Please submit a bio-sketch for the PI in NIH bio-sketch format (no more than five pages).

Pre-applications must be submitted as a PDF document to vgreger@ntsad.org by 5 p.m. EDT February 18, 2022.

Schedule:

Pre-application deadline	February 18, 2022
Invitation to submit a full application	March 11, 2022
Full application deadline	April 1, 2022
Award announcement	June 2022
Earliest anticipated start date	July 2022

