



The NTSAD Research Initiative is our comprehensive strategic research program that funds cutting-edge efforts to find a cure and promote scientific collaboration to accelerate these efforts. In 2002, NTSAD committed itself to finding a cure and founded the Research Initiative; **41 projects and \$2.9 million later** in grants, we are closer than ever to a cure. Several projects have led to larger NIH grants resulting in over \$10 Million toward this big goal.

NTSAD received a record **23 grant** proposals from around the world in response to its annual Request for Proposal (RFP) process this year. These grants were evaluated by **16 experts**, many of whom are on NTSAD's Scientific Advisory Committee. (Many thanks to our reviewers for contributing their time and expertise!) The grants are ranked by the scientists based on using an NIH rating scale of 1 (best) to 10 (worst) with comments about strengths and weaknesses. They also consider the significance of the proposal, the research plan quality, the researcher(s)' qualifications, and their ability to complete the project on time and within the proposed budget.

The Research Initiative (RI) Committee reviews the SAC recommendations to make the final funding decision. The members of the RI Committee are Staci Kallish (chair), Rod Marquardt, Sophia Pesotchinsky, and Davis Yang. This year there were several other grants of interest that we may still consider funding at some point in time.

We are awarding a single grant at this time while we are clarifying research priorities and funding required. Other current priorities include the **Tay-Sachs Gene Therapy Consortium** and developing **clinical trial readiness**. Our updated Research Strategy recognizes the importance of clinical trial readiness as we move closer to clinical trials. Pulling together the expertise of our Scientific Advisory Committee and Corporate Advisory Council, NTSAD will 'make our diseases easy to study' by developing a clinical data repository, promoting and supporting research efforts that identify bio-markers (used to evaluate clinical efficacy) and natural history studies. Moving forward in this area may have an impact on our funding priorities.

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Tay-Sachs Gene Therapy Consortium News

The six non-human primates that received Tay-Sachs gene therapy using the newly developed vectors continue to display normal behavior. The Tay-Sachs Gene Therapy Consortium is cautiously optimistic that the new vector is working.

Race for Lena

Jola & Maciek Marchewicz from Poland were thrilled to attend the 2013 Family Conference in San Diego with their children, Borys and Lena. Lena is affected with juvenile GM1. They were inspired to become part of the [2013 Worldwide Day of Hope](#) and set up a group of amateur and professional runners who wanted to dedicate their running events for Lena. [The 1st Running Race for Lena](#) took place in Gdansk, Poland on September 14, 2013. They designated the money raised during the event for funding research for a GM1 cure. Because of tax rules in Poland, however, they needed to transfer this money in connection with a specific research center and project. They were so happy to hear that a research proposal for

We are happy to announce that NTSAD is awarding a Research Initiative grant to David Radin, PhD, Principal Investigator of BioStrategies, LC, for the project, **Lectin-assisted transnasal delivery of corrective enzyme for GM1 gangliosidosis**. The grant is a two-year \$80,000 milestone-based grant.

BioStrategies LC is a start-up biotechnology company located at Arkansas State University. The company develops and manufactures human and animal protein therapeutics in plant - based production systems. Dr. David Radin and Dr. Carole Cramer, the co-founders of BioStrategies, previously conducted breakthrough research which was funded by several NIH SBIR (Small Business Innovation Research) grants and which led to FDA approval of a treatment for Gaucher disease, a lysosomal storage disease. If they are successful in this project with GM1 gangliosidosis, this approach will apply to other similar diseases.

A summary of the proposal follows:

- A major limitation to enzyme replacement therapies (ERTs), which are used to treat many lysosomal storage diseases, is that the blood brain barrier protects the central nervous system, preventing needed enzyme from reaching the brain. Much research has been done to find a way to get treatments to the brain, but there has been only limited success so far.
- Dr. Radin's group has worked with an experimental approach to crossing the blood brain barrier, attempting to deliver enzyme into the brain through the nose. His team has experience working in transnasal delivery. They are currently working on a vaccine candidate using transnasal delivery and have previously worked on delivering an enzyme using this method.
- This group has developed a new method to do this using a plant protein called RTB that has been shown to facilitate delivery through the lining of the nose. They then developed a fusion protein linking RTB to B-galactosidase, the enzyme deficient in GM1.
- With support from NTSAD's Research Initiative, Dr. Radin's group aim to treat mice with GM1 with this new therapy, looking for evidence of B-galactosidase reaching the brain. They will also look to see if levels of stored GM1 decrease and if the mice have improvements in health.
- Because the transnasal approach is new, they plan to compare mice treated by nasal delivery and mice treated by intravenous administration.
- They believe that their new drug targeting/delivery technology has the potential to provide significant future advances in ERT drug delivery challenges and also in treating symptoms affecting brain function.

Like other Research Initiative grants, this grant will be paid over time, with payments contingent upon successfully completing research plan milestones every 6 months.

GM1 would receive a Research Initiative grant award so they could dedicate the funds raised to this project. They plan to continue supporting this project and new research grants with future events.



Research + Funding = Hope

Support Research with a gift [here](#).

Read more about current and past grants funded by NTSAD on our website [here](#).



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If opportunity doesn't knock, build a door.
Milton Berle

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