NTSAD

National Tay-Sachs & Allied Diseases Association

all rare.









all hopeful.



all in common.


all empowered.

“Individually we are one drop. Together, we are an ocean.”

— Ryunosuke Satoro

NTSAD**Research**

2015 NTSAD Science Symposium & Workshop for Professionals

NTSAD brought healthcare professionals and researchers together to discuss topics from basic science to clinical trial readiness for a symposium preceding the 2015 NTSAD Annual Family Conference. They gathered together to develop ways in which we can partner to make progress. Topics explored ranged from looking at the common elements among these neurodegenerative diseases to clinical trial design to specific issues relating to the gangliosidoses and leukodystrophies.

Second Annual Million Dollar Bike Ride

Team NTSAD rode to raise $20,000 on May 8 in Philadelphia which was matched by UPenn's Orphan Disease Center to support a grant award made to Marlene Jacobson, PhD, of Temple University. Her project, "Patient-Derived Phenotypic Assay to Discover Treatments for Tay-Sachs disease," will enable exploration of small molecules including known FDA-approved drugs that, if proven to be effective, could have the potential for rapid progression into clinical trials.

2015 Research Initiative Grants Awarded

Development and validation of a rapid, MS/MS-based method to detect Hexosaminodase deficiency in Tay-Sachs disease
Principal Investigator: Denis Lehotay, PhD
Queens University, Ontario

Registry and repository for Late Onset GM2 gangliosidoses
Principal Investigator: Florian Eichler, MD
Massachusetts General Hospital

Defining the Natural History of Canavan Disease through Development of an International Registry
Principal Investigator: Heather Lau, MD, New York University
Co-investigator: Paola Leone, PhD, Rowan University
Co-funded with The Canavan Foundation

Intravascular Gene Therapy for Feline GM2 Gangliosidosis
Principal Investigator: Doug Martin, PhD
Auburn University College of Veterinary Medicine

Generation of a Knock-in Mutant HexB Mouse Model
Principal Investigator: Eric Sjoberg, PhD
OrPhi Therapeutics

Pre-clinical Studies of AAVrh8-Hex Gene Delivery
Principal Investigator: Miguel Sena-Esteves, PhD
University of Massachusetts Medical Center

NTSAD**Families**

"It is only through NTSAD that we are able to find other families to connect with and help us on our journey. I cannot fathom doing this alone."

The heart and soul of NTSAD is our family services program. We advocate for children affected by Tay-Sachs, GM1, Sandhoff and Canavan diseases, connect parents coping with the heartache and support the adults struggling with the degenerative effects of the Late Onset forms. Each and every person connecting with NTSAD will find solace and invaluable support within this small but mighty community.

Annual Family Conference

April 2015, Reston, Virginia

This long weekend provides families with a caring environment that gives them the tools to feel empowered and connected as they care or grieve for their loved ones. Over half the families who attend the conference receive a Helping Hand Grant award from a fund supported by generous donors, family foundations and family funds within NTSAD.

78 FAMILIES

- **15** Late Onset adults plus their caregivers, parents or spouses
- **10** bereaved families
- **10** newly diagnosed
- **71** extended family, friends and professionals
- **44** family attendees thanks to a Helping Hand Grant award

Rare Siblings Project

This website project was inspired by a sibling and brought to fruition thanks to a Genzyme Patient Advocacy (PAL) grant and collaboration between NTSAD and Courageous Parents Network. Its purpose is to give siblings a place to address the issues they face as rare siblings and share their stories with rare siblings worldwide.

NTSAD**Vision**

To fund global cutting-edge research, to provide families with compassionate care, and to support and collaborate effectively with the healthcare community to achieve our goals.

ABOVE AND BEYOND AWARD

MICHAEL SUSSMAN, a longtime NTSAD supporter, received the NTSAD Above & Beyond award at the 2015 Annual Family Conference for his generous support of NTSAD. He continues to extend his parents' legacy which started when they, with other families, founded NTSAD in the late 1950s. Michael shared how proud his parents would be to see those in attendance there that day and the work that they and all of NTSAD continues to do.

POWER OF ONE AWARD

ORALEA MARQUARDT, with her family, received the NTSAD Power of One award at NTSAD's 2015 Family Conference for her contributions to the development of a pediatric hospice in Florida, her commitment to support other NTSAD families and her work with her local hospice. Her son, William, who had GM-1, is her inspiration and guides everything she has done including her pursuit of a Masters of Social Work degree.

NTSAD**Support**

It was a milestone year for NTSAD because for the first time NTSAD reported revenues totaling over $1 million in its 2015 fiscal year. It was possible thanks to the 2,735 donors who supported NTSAD and who believe in the mission to care and advocate for families, thoughtfully fund research in all its stages, and raise awareness about these rare genetic disease that affect all too many.

Imagine & Believe

NTSAD's 8th Annual Boston benefit took place at the Royal Sonesta in Cambridge, MA, on November 5th. A stellar silent auction, enjoyable food, and a wonderful mix of friends, partners and families made for a successful evening raising over $80,000 for research and support for NTSAD. Guests heard from a local NTSAD mom, Kate Hubbard who shared her story about caring for daughter Brook and the rest of her family during Brook's all too short life living with Tay-Sachs. It was also an honor to recognize the work of Bob Coughlin, President and CEO of Massachusetts Biotechnology Council and to listen to his story about his personal connection with rare diseases and his contribution to the rare disease community.

Day of Hope

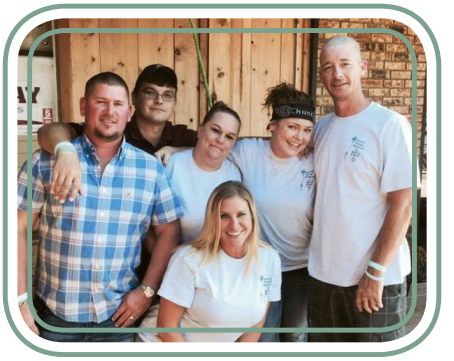
$168,283
RAISED SINCE 2010

43
FAMILY EVENTS IN TOTAL

Fifth Annual Day of Hope

Rallying & Raising Funds for Research

Every September, the NTSAD community rallies behind Day of Hope as a way to be empowered and raise funds for research. In 2015, the Annual Day of Hope raised over $35,825 for research.



Ashley Watson held her annual Ride for Jase in memory of her son, Jase, raising over $7,000 for Day of Hope in 2015.

NTSAD Awareness

21st Century Cures

The 21st Century Cures Act made it through the House in 2015 and now waits its turn on the Senate floor. This bill could accelerate the pace of discovery, development, and delivery of rare disease treatments and cures, as well as provide hope for patients and their families. Through partnering with the National Organization for Rare Disorders, EveryLife Foundation and Global Genes, NTSAD brings news of the latest rare disease legislation to its families so they can connect with their local representatives to put a face on rare diseases.

Moving Forward

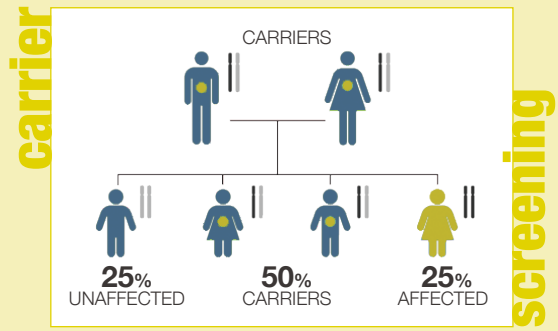
Carrier screening remains our strongest weapon in the fight against diseases like Tay-Sachs, GM1, Sandhoff and Canavan. NTSAD recently announced its partnership with JScreen, Emory University, and Counsyl, to fund and conduct a research study that may provide essential information for the future transition of Tay-Sachs enzyme screening to newer genetic screening testing methods. NTSAD — together with the Mathew Forbes Romer Foundation, Evan Lee Ungerleider Fund, New York Area Fund of NTSAD and the Cameron and Hayden Lord Foundation — is funding this important study.

KNOW THE FACTS:

one in 27 Ashkenazi Jews, French-Canadians, and Cajuns

one in 50 to 1 in 190 Irish-Americans

one in 250 of the general population is a carrier of the Tay-Sachs gene



NTSAD encourages meeting with a genetic counselor to understand your risks before getting tested.

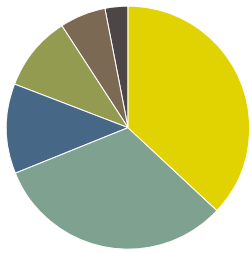
NTSAD Family Support
FACEBOOK GROUP

GERMANY
UNITED KINGDOM
MEXICO
ARGENTINA
ISRAEL
PHILIPPINES
439 MEMBERS
HONDURAS
BRAZIL
FRANCE
NEW ZEALAND
UNITED STATES
CANADA
AUSTRALIA

2015 Fiscal Year
Audited Financial Statements

$725,393

EXPENSES



- 37% — Research
- 32% — Family Services
- 12% — Fundraising
- 10% — Education
- 6% — Administration
- 3% — Advocacy

$1,106,524

SUPPORT & REVENUE

- 36% — Research
- 17% — Restricted Gifts
- 15% — Gifts
- 11% — Conference
- 10% — Special Events
- 8% — Grants
- 3% — Quality Control

NTSAD**Mission**

NTSAD leads the fight to treat and cure Tay-Sachs, Canavan and related genetic diseases, and supports affected families and individuals in leading fuller lives.

NTSAD

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Allied Diseases Association

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