

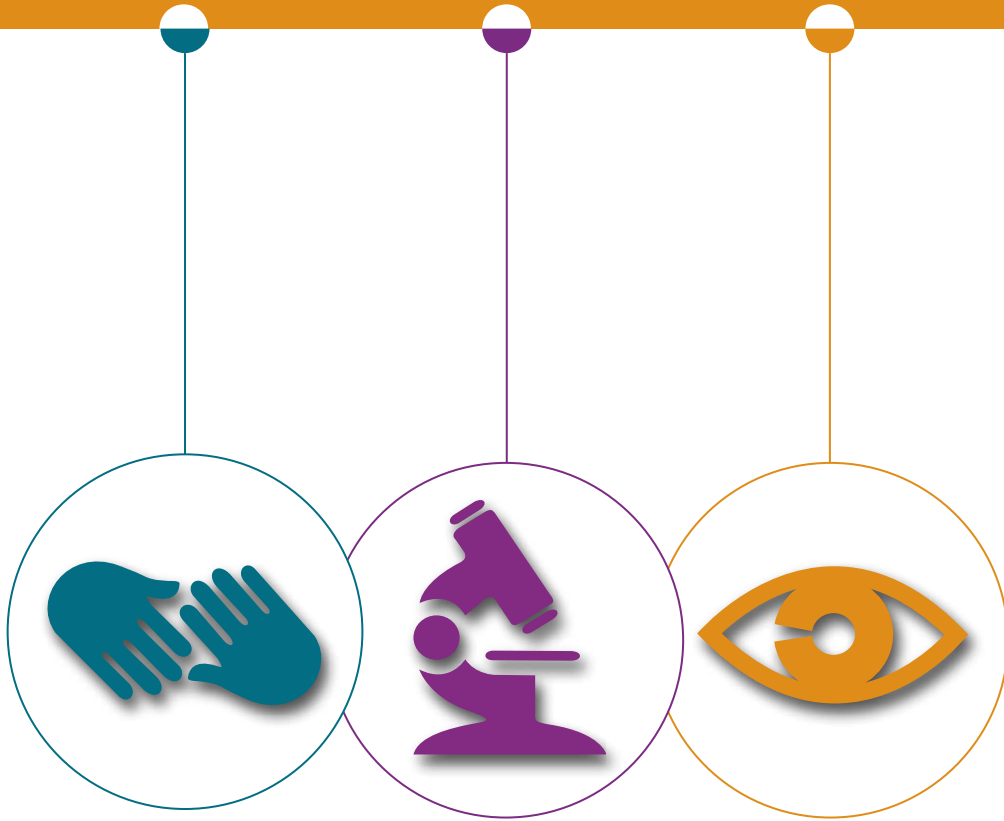
# NTSAD

National Tay-Sachs & Allied Diseases Association

SUPPORT

RESEARCH

AWARENESS



2014  
ANNUAL REPORT

Hope knows no boundaries

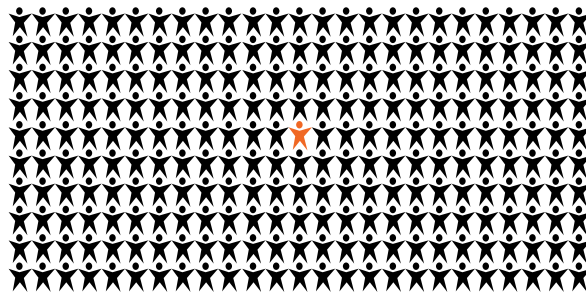


NTSAD's VISION — To fund global cutting-edge research, to provide families with compassionate care, and to support and collaborate effectively with the healthcare community to achieve our goals.

*"The small family that is NTSAD is proof that the human spirit and the human community is at its best when faced with great adversity."*

## By the numbers

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



**1 in 10** Americans is affected by a rare disease\*



**350,000,000** people worldwide suffer from a rare disease\*

NTSAD supports over **750 families** around the globe



\* Source: Global Genes



# RESEARCH

## Hope on the Horizon

### *Corporate Advisory Council*

The NTSAD Corporate Advisory Council, established in 2014 as part of an overall Research Strategy, provides guidance to NTSAD as it prepares for future treatments, advances its research programs, and builds for clinical trial readiness. Council members are professionals involved in rare diseases and industry, and passionate about giving families hope.

### *GM2 Clinical Research Network*

The GM2 Clinical Research Network is a global collaborative network of healthcare professionals and researchers that supports clinical and translational research while working together to find a cure for GM2 and related diseases. Their July 2014 meeting focused on the importance of clinical data and the necessity of natural history studies to better understand the progression of GM2 and related diseases.

### *Million Dollar Bike Ride*

The University of Pennsylvania Orphan Disease Center (ODC) held their first annual Million Dollar Bike Ride to raise funds for rare disease research. Team NTSAD raised \$35,000 including a match from ODC that led to a grant awarded to **Annette Bley, MD**. The project will supply data that will be useful for outcome measures and future clinical trial designs for Canavan disease.

### *2014 Research Initiative Grant*

#### **BioStrategies, LC**

**David Radin, PhD**, Principal Investigator

**Carole Cramer, PhD & Alessandra d'Azzo, PhD**, Co-investigators

*"Lectin-assisted transnasal delivery of corrective enzyme for GM1 gangliosidosis"*



Meet Rachel and her mom, Sherri. Rachel was diagnosed nearly 16 years ago with Canavan disease. Her parents were told there was no treatment and there was nothing they could do to help her. Recently, Sherri visited the lab of NTSAD funded researcher, Gaungping Gao, PhD, and held two mice affected by Canavan disease. Sherri wrote, *"As I watched both mice scurry across my palms, I could not tell the difference (between) the normal mouse and the "blind" and disabled mouse. I (still) can't stop shaking my head in amazement as to the miracle that unfolded before my eyes! There is still more years of research before they can bring it to a human clinical trial, but I know now that it will happen!"*



# FAMILY SUPPORT

## The Heart & Soul of NTSAD



### Annual Family Conference

The Conference provides families the resources, tools, camaraderie and comfort to cope with rare genetic diseases like GM1, Tay-Sachs, Sandhoff, and Canavan diseases. Researchers, physicians and caregivers also attend to reap the benefits of meeting families and individuals affected by the diseases face to face. The connections made are invaluable on all fronts.



Conference honorees, Rose Urban and Doug Martin, PhD. for their tireless work

### Helping Hand Grant Fund

NTSAD provides grants to assist families with the expenses of attending the Annual Family Conference. Priority is focused on newly diagnosed, newly bereaved and first-time attendees. Out of 62 families attending in 2014, 35 families received grants totaling over \$40,000.

### A Global Affair

Families and individuals worldwide turn to NTSAD after receiving their diagnosis. The NTSAD Family Support Group on Facebook is a safe and private forum for over 330 individuals and families (and growing) around the world to offer advice, ask questions and provide support to one another.

### NTSAD Family Care Packages

Care packets are tailored to newly diagnosed families or individuals depending on the diagnosis. Invaluable resources such as the NTSAD "Home Care Manual" and DVD film, "*Parenting a Child with Life-limiting Illness*" are provided to every family. Resources for healthy siblings and grieving parents are currently being developed.

Meet Fiona and her parents, Dee and Paxton. Dee recently wrote about her experience joining the NTSAD community after receiving the news that their daughter has GM1. The power of the connections made is abundantly clear. *"In the beginning, I didn't want to be part of this community, but as time passed these parents became friends and ended up as an extended family. We call each other and our children by first name. We send each other messages when we are discouraged and have questions about caring for our child. We cheer and encourage our children. This community celebrates and mourns with each other on the journey of caring for our very rare children. We realize that with our children, we aren't guaranteed tomorrow."*



# EVENTS & AWARENESS

**Education = Prevention = Cure**

## *Imagine & Believe: A Boston Benefit*

Nearly 150 friends, family, and rare disease community partners raised **nearly \$80,000** on November 13, 2014 to support families, raise awareness and fund hopeful research!

Our honoree, **Stephen Groft**, former Director of the Office of Rare Diseases Research (ORDR), eloquently spoke about how patient advocacy groups like NTSAD are responsible for bringing more attention to rare diseases. His passion for advocating research for rare diseases was evident and appreciated by all who attended. The rare disease community and NTSAD salute his work and commitment!

**Becky Benson**, an NTSAD mom, shared how connecting with other families was paramount to her family as they cared for her daughter, Miss Elliott, during her all too brief life with Tay-Sachs. *“NTSAD not only fosters togetherness, but it also leads the fight in the research for a treatment and eventual cure of diseases like Tay-Sachs. NTSAD itself is the epitome of hope to families like mine. Because of the efforts made here tonight newly diagnosed families will know that hope truly is on the horizon. Hope for treatment, hope for a cure, hope for their child’s life.”*

## *NTSAD’s Day of Hope*

The Annual Day of Hope rallies families and friends to raise money solely for research. In September 2014, 20 events were held across the United States with even NTSAD’s partner, The CATS Foundation, holding Day of Hope events as well in the United Kingdom. Close to \$40,000 was raised and directed toward research this year. Since 2011, the Annual Day of Hope events have now raised \$132,563 to fund research and HOPE.



## *An Evening in New York*

Friends, family and colleagues of the NTSAD New York area chapter came together in May 2014 and raised **over \$85,000** to honor the accomplishments of the chapter as well as the outstanding commitment of long-time volunteer and co-president, **Marion Yanovsky**. An incredible group of volunteers remain committed to promote awareness and raise funds for research as the chapter transitions its administration to the national office of NTSAD.

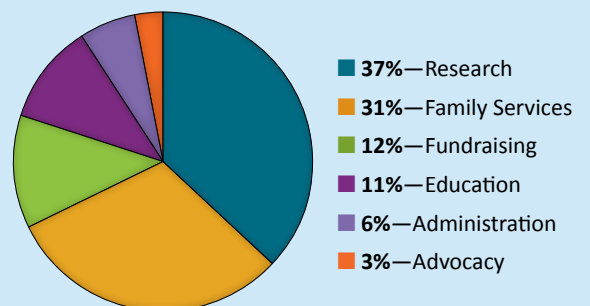
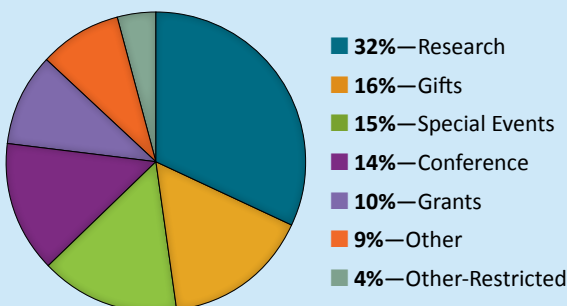


**Support & Revenue: \$801,148**

**Expenses: \$784,503**

**FISCAL  
YEAR  
2014**

**Audited  
Financial  
Statements**





# MISSION STATEMENT

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.

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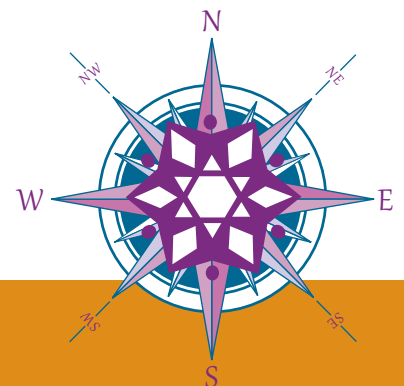
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A COMMON BOND • AN UNCOMMON DETERMINATION