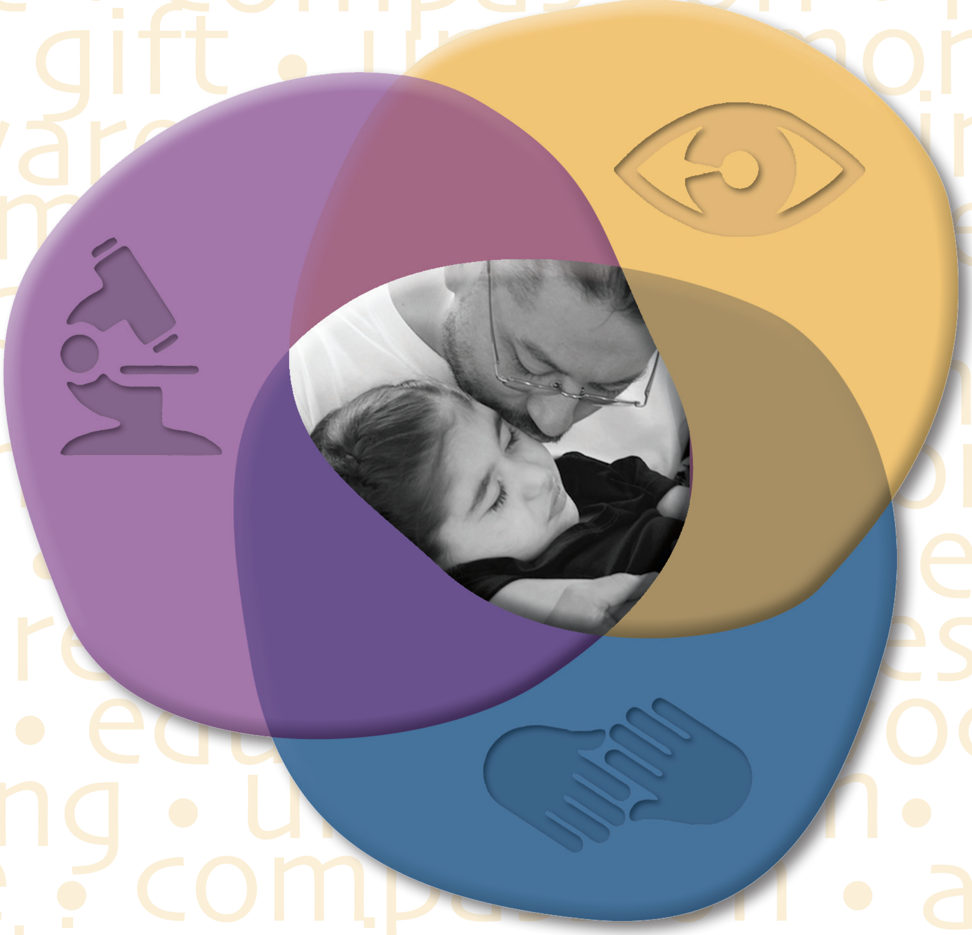
A YEAR WITH

NTSAD

National Tay-Sachs & Allied Diseases Association

SUPPORT • RESEARCH • AWARENESS



2013 ANNUAL REPORT

Dear Friends,

The Oxford Dictionary defines "**empower**" as an act of giving someone the power to do something. Personally speaking, I can say there is nothing more empowering than honoring your child's life by impacting others. While my son, Evan, is no longer with us, he has empowered me beyond what I ever imagined I could do.

Every ounce of support NTSAD receives gives us the power to **make a difference** in the unique world of rare genetic diseases. NTSAD provides family support services, funds research and collaborates with grassroots organizations to provide education and awareness about the diseases and need for carrier screening.

I am proud to say that NTSAD's role as **a leader in the field of rare diseases** serves as a beacon for other related patient groups. Recently, two well-respected organizations have turned to NTSAD to continue their work:

- **Jacob's Cure** closed their doors in 2013 after 14 years of raising funds for Canavan Disease research. They asked NTSAD to carry on their work through the newly created Jacob's Cure Fund of NTSAD.
- **The NTSAD New York Area chapter** will merge into the national organization in June 2014 after serving the Tri-State area for more than 40 years. NTSAD will continue their work and its commitment to raise money for research and awareness about proper carrier screening.

It was a remarkable year and I hope you see how every gift made to NTSAD empowers us to excel. Thank you for believing in NTSAD, our families and our mission. Together, we will make a difference today for a brighter future tomorrow.

Warmest regards,

Shari Ungerleider

Shari Ungerleider, President

ducation • advocacy • compassion • common goal • uncommon determination • hope

RESEARCH

Hope on the Horizon

The broad scope of NTSAD's support of research encompasses all facets of research from funding, administering grants, and vetting feasible projects to networking with biotech companies and other research organizations.

The U.S. Food and Drug Administration (FDA) granted **Orphan Drug Designation for Tay-Sachs and Sandhoff** disease gene therapy to facilitate development of these potential therapies. It is rare for a patient organization like NTSAD to sponsor this type of application.

The flock of Jacob Sheep, whose naturally-occurring Tay-Sachs carrier status has been helpful in research, was moved to Auburn University in Alabama where studies for the Tay-Sachs Gene Therapy Consortium are being conducted. These NTSAD-funded larger animal studies are significant in determining safety and efficacy for a possible gene therapy treatment. The sheep will help to better understand disease progression for future clinical trials.

The Cameron & Hayden Lord Foundation

The Lord family created the Foundation to honor the memories of cousins, Cameron and Hayden, both of whom had Tay-Sachs. The Foundation has given **nearly $400,000** to NTSAD's Research Initiative in support of ground-breaking research. Their generous funding of all aspects of NTSAD's mission has had a profound impact on families, children and individuals worldwide.

$3,000,000

Amount NTSAD has awarded in research grants since 2002, which has led to

OVER $10,000,000

in NIH grants awarded toward finding a cure.

2013 Research Initiative Grants

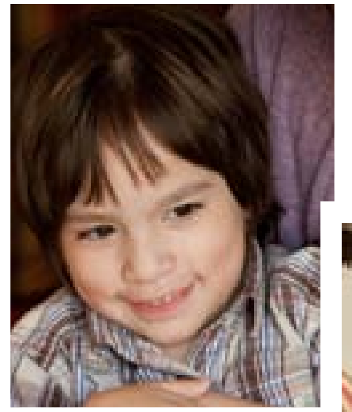
Alessandra d'Azzo, PhD, St. Jude Children's Research Hospital
- Studies of the molecular and biochemical bases of neurodegeneration in sialidosis

Doug Martin, PhD, Auburn University
- Histological analysis of the central nervous system after intra-cranial injection of AAV vectors in normal cats
- Breeding flock for the Sheep Model of Tay-Sachs Disease

Miguel Sena-Esteves, PhD, University of Massachusetts Medical School
- Selection of new AAVrhB vector design and safety testing (co-funded with Cure Tay-Sachs Foundation)

To read more about projects funded by NTSAD, visit **www.NTSAD.org**.

Guangping Gao, PhD, of University of Massachusetts Medical School, received a 2011 NTSAD Research Initiative Grant for his work on an effective gene therapy for Canavan Disease. Visit **www.NTSAD.org** to read his 2013 publication about his research progress.



FAMILY SUPPORT

The Heart & Soul of NTSAD

NTSAD's Family Services offers **unparalleled support** to families and individuals, including a mentorship program; access to invaluable health care and home care resources; and the opportunity to attend the NTSAD Annual Family Conference, the highlight of the NTSAD year.

Last April, NTSAD launched **"Parenting a Child with Life-limiting Illness"**, a film funded by a 2011 Patient Advocacy Leadership (PAL) Award from **Genzyme, a Sanofi Company**. It was created to lessen the feelings of isolation families can experience. The film and accompanying guide book inspired Global Genes, a rare and genetic disease patient advocacy organization, to develop a comprehensive toolkit for other rare disease groups.

NTSAD's **"The Rare Siblings Project"** also received a 2013 PAL Award from Genzyme. The goal of this project is to address the issues siblings of affected children experience through a series of thoughtful interviews. NTSAD and the newly formed Courageous Parents Network will develop and produce this latest project.

Allie Colaco, recipient of a Stanley & Jeffrey Gottlieb Sibling Scholarship, is earning her PhD from Oxford University. Her research in the labs of NTSAD Scientific Advisory Committee members, Tom Seyfried, PhD, and Fran Platt, PhD, has led her down a career path that honors her twin brother, Keith, who died of Tay-Sachs in 1993.

The Sibling Scholarships are given annually to college students to honor their important role as a sibling to an affected brother and/or sister.

OVER 750

Number of families NTSAD supports worldwide

Argentina
Australia
Belgium
Brazil
Bulgaria
Canada
Columbia
Cuba
Czech Republic
Egypt
France
Germany
Guatemala
India
Iran
Ireland
Israel
Italy
Japan
Jordan
Kuwait
Lebanon
Mexico
Nepal
Netherlands
New Zealand
Pakistan
Philippines
Poland
Romania
Russia
Saudi Arabia
South Africa
South Korea
Spain
Sri Lanka
Sweden
Thailand
Turkey
United Arab Emirates
United Kingdom
United States

AWARENESS

Education = Prevention = Cure

Until a cure is found, **education**, paired with carrier screening, is the **most effective tool** in the campaign to prevent Tay-Sachs, Canavan, Sandhoff, GM1 and related diseases. NTSAD fully supports families and individuals with the resources and materials they need to **raise awareness** in their communities.

Day of Hope

Rallying & Raising Funds for Research

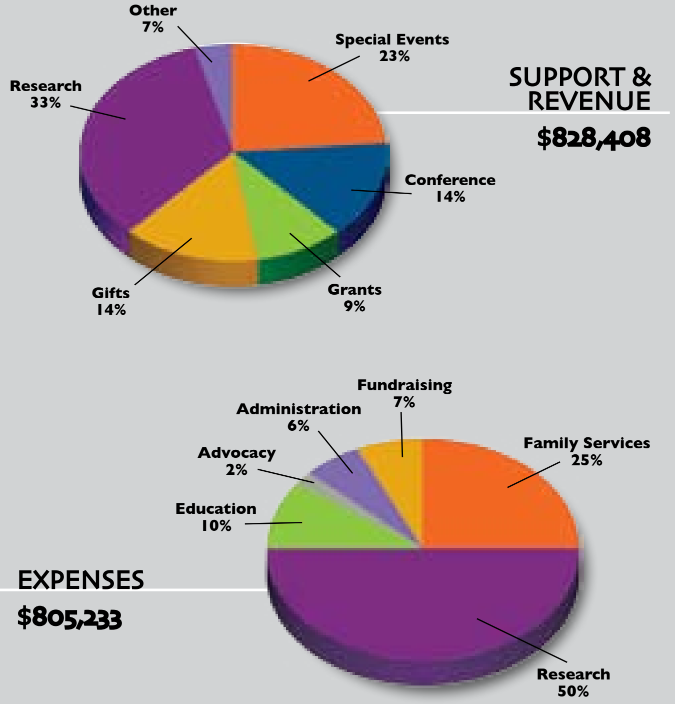
Every September, NTSAD family and friends observe the International Day of Hope which NTSAD began in 2010. The focus is research which translates to hope for families and individuals worldwide. Lemonade stands, bake sales, runs, walks and motorcycle rides have **raised nearly $90,000** for research.

5th Annual 'Fore' Jack Memorial Golf & Tennis Outing

Since 2009, the Kliger family has honored their son Jack's memory by raising **nearly $500,000** to support families coping with their loved one's diagnoses of Canavan disease.

FISCAL YEAR 2013

AUDITED FINANCIAL STATEMENTS



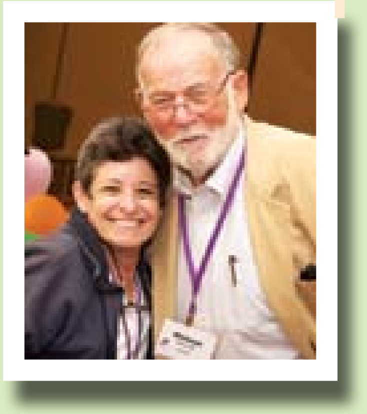
SUPPORT & REVENUE

$828,408

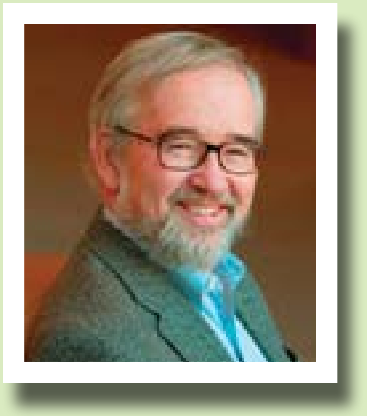
EXPENSES

$805,233

HONOREES



Michael Kaback, MD, with NTSAD Late Onset Tay-Sachs member, Julie Stein, was honored at the 35th Annual Family Conference for his long-standing commitment to awareness and carrier screening through his work as founder of the International Quality Control Tay-Sachs Testing Program.



Phil Reilly, MD, JD of Third Rock Ventures, was honored at the Imagine & Believe Benefit in Boston for his commitment to developing new therapies for rare genetic diseases. The event raised **$80,000** to support NTSAD's mission to care for families and find a cure.

Many thanks to **ACOM Healthcare** and **ARTCO Printing** for their generous pro bono production of a new brochure promoting NTSAD's work. To receive a copy, please contact the NTSAD office at **info@ntsad.org**.

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.

350,000,000 people worldwide are estimated to suffer from rare diseases.*

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

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

* Source: Global Genes

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