Compassion. Commitment. Community.

NTSAD

NATIONAL TAY-SACHS &
ALLIED DISEASES ASSOCIATION

ANNUAL REPORT 2018

OUR COMMITMENT TO OUR COMMUNITY

In 1957, a small group of families came together in a search to find answers. They formed the National Tay-Sachs & Allied Diseases Association (NTSAD), among the first patient advocacy groups established in the country. Founded on principles of compassion, commitment, and community, today we continue our focus on bringing families together, supporting affected families and individuals in leading fuller lives, funding research, and raising awareness to prevent Tay-Sachs, Sandhoff, Canavan, and GM1 gangliosidosis.

In 2018, NTSAD built on existing programs and developed new initiatives to broaden our reach. We made significant advances in bringing potential treatments to the families and individuals facing diagnoses and living with disease. Through family engagement, funding, trusted and new partnerships, and a reputation of determination spanning six decades, NTSAD has enabled breakthroughs leading to what will be our first clinical trials, anticipated in 2019.

Thank you for being an essential part of the NTSAD community; a community that together has made treatments a real possibility on the horizon.

A MILESTONE YEAR
ONE STEP CLOSER TO TREATMENTS

For the first time in our history, clinical trials are being planned for Tay-Sachs and Sandhoff (collectively GM2), GM1 gangliosidosis and Canavan diseases. We are working with companies who have the experience and knowledge to initiate trials which may lead to treatments for our affected family members.

In May, Aspa Therapeutics licensed the Canavan gene therapy program from UMass Medical School to work toward a clinical trial. This research was led by Guangping Gao, PhD, and was initially funded by an NTSAD Research Initiative seed grant.

In December, Axovant Gene Therapies licensed the GM1 and GM2 gene therapy programs from UMass Medical School with the goal to launch clinical trials in 2019. The research was led by scientists from UMass Medical School, Auburn University and other members of the Tay-Sachs Gene Therapy Consortium. NTSAD has provided grant funding to UMass Medical School and other members of the Consortium since its inception in 2007.

NTSAD was, and remains, instrumental in bringing together the families, funders, scientists, clinicians, and industry partners who made these steps possible.

RESEARCH ENGAGING FAMILIES

Our families are a vital part of the research process: raising money, providing targeted funding, and sharing information about their experiences. Their participation is a critical factor leading to the clinical trials, and we share their hope for treatments.

- NTSAD families raised almost $125,000 during the 8th Annual Day of Hope. Since its inception in 2011, the Day of Hope family initiative has raised nearly half a million dollars for research.
- The Katie and Allie Buryk Research Fund hosted and funded a one-day **Late Onset Think Tank** meeting led by Drs. Cynthia Tifft and Steve Walkley. It was attended by 20 thought leaders in Late Onset Tay-Sachs and Sandhoff diseases. They shared their research expertise, focused their attention on possible approaches for Late Onset therapies, and developed a roadmap for communications and collaboration moving forward.
- NTSAD initiated a GM2 Patient Insights Network (PIN), and now all NTSAD families can take an active role in participating in research as PINs already exist for Canavan and GM1. These PINs give research teams a clearer picture of the impact of these diseases through patient data and experiences.
- NTSAD worked with the TREND Community to turn anecdotes into evidence by understanding the patient experience regarding off-label use of a drug.

NOTABLE NUMBERS

65
Number of grants since the Research Initiative was created in 2002

$4.1 million
Amount of NTSAD funding toward research projects ranging from basic research to clinical trial readiness

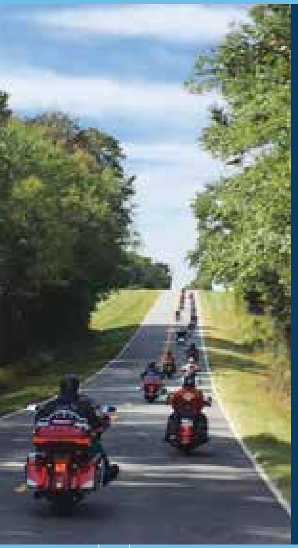
$30+million
Amount of National Institutes of Health (NIH) and other outside funding awarded to NTSAD grant recipients subsequent to their NTSAD-funded research

RESEARCH **INITIATIVE GRANTS**

NTSAD awards grants for innovative research projects that may lead to treatments for lysosomal storage diseases or leukodystrophies impacting the central nervous system. We require our grant recipients to submit progress reports and notify us of resulting publications and intellectual property.

NEW GRANTS AWARDED IN 2018:

- *Role of Plasma membrane-ER Contact Sites in GM1-mediated Neuronal Cell Death*
  Alessandra D'Azzo, PhD, St. Jude Children's Research Hospital
- *Role of Microglia in Sandhoff Disease Pathology*
  Tony Futerman, PhD, Weizmann Institute of Science
- *Oligosaccharide Biomarkers for Disease Progression and AAV Therapeutic Efficacy in GM1 Gangliosidosis*
  Xuntian Jiang, PhD, Washington University
- *Million Dollar Bike Ride Award: Brain MRI signatures in infants with infantile forms of GM1 and GM2*
  Igor Nestrasil, MD, PhD, University of Minnesota



Jessie's
RIDE OF HOPE

Jessie Jackson has Juvenile GM1 gangliosidosis. Her parents, Merlie and Dave Jackson, launched Jessie's Ride of Hope to raise money for research as part of NTSAD's Annual Day of Hope.

FAMILY SUPPORT

NTSAD's Family Support services continue to empower, inform and connect families as they wrestle with the challenges that come with a diagnosis and living with disease. In 2018, Family Support served the global NTSAD community through extensive outreach and programming.

ANNUAL
FAMILY CONFERENCE

2018 marked the 40th consecutive year we gave families a safe space to feel supported and empowered. Over 80 families attended with more than half receiving Helping Hand grants to ease the financial burden. Research was the highlight of robust breakouts for GM1, GM2 and Canavan diseases. We marked the fourth year of collecting data and performing assessments with adults affected by Late Onset Tay-Sachs and Sandhoff diseases to help develop clinical trial endpoints for use in prospective clinical trials.

Camp Snuggle and Camp Active gave families and their affected children the opportunity to have as close a camp experience as their children will likely have in their lifetimes. Activities range from art, music and massage therapies to an endless amount of snuggling thanks to many volunteers. The full-day camps give parents the peace of mind to attend conference sessions while connecting meaningfully with other families.

CARE TIP SERIES

Launched in 2017 with the goal of educating families in caring for an affected child, new Care Tip videos were released in 2018. They focus on demonstrating physical therapy routines and the best practices for utilizing the vest respiratory therapy system. Auto-translated subtitles are available through YouTube, so international families can access the videos in their native language.

The Ethel Berkman Power of One award was presented to NTSAD parent Bonnie Davis (left), an NTSAD volunteer since 2001. The Above & Beyond award was given to Susan Finkelstein, RN, who dedicated 35 years to caring for children including several affected by Tay-Sachs disease, like Bonnie's son, Adam.

REMEMBRANCE

Isaiah Ackerman
Luke Ackerman
Embree Alexander
Fernando Arcanjo
Haylie Binder
Ariel Coover
Islah Faint
Brayden Gloege
Theia Kar
Phoebe Massung
Keegan Overvold
Priyan Sapkota
Jacob Schwartz
Chaya Tova Schwartzman
Allan Stevenson
Richard Strauss
Kaiden Sturgeon
Carson Witte

Katie and Allie Buryk, diagnosed with Late Onset Tay-Sachs in 2015, with Katie Couric

"We have to help shape our future – the future is not inevitable. We can do something about it; that's our job." BARRY SHRAGE

PHILANTHROPY AND EVENTS

In 2018, NTSAD secured significant funding through a variety of events, programs and special appeals. Two families whose loved ones were given comfort, guidance, and a sense of community, made two of the largest, one-time gifts in the history of NTSAD. These were a $100,000 gift from a family who lost a child to Tay-Sachs more than 50 years ago, and a $50,000 gift from a grateful family, who, due to carrier screening developed by NTSAD more than 40 years ago, has healthy children and grandchildren.

MAJOR EVENTS

Three key events brought together the NTSAD community, our partners, and even some celebrities to support the work of NTSAD and our families.

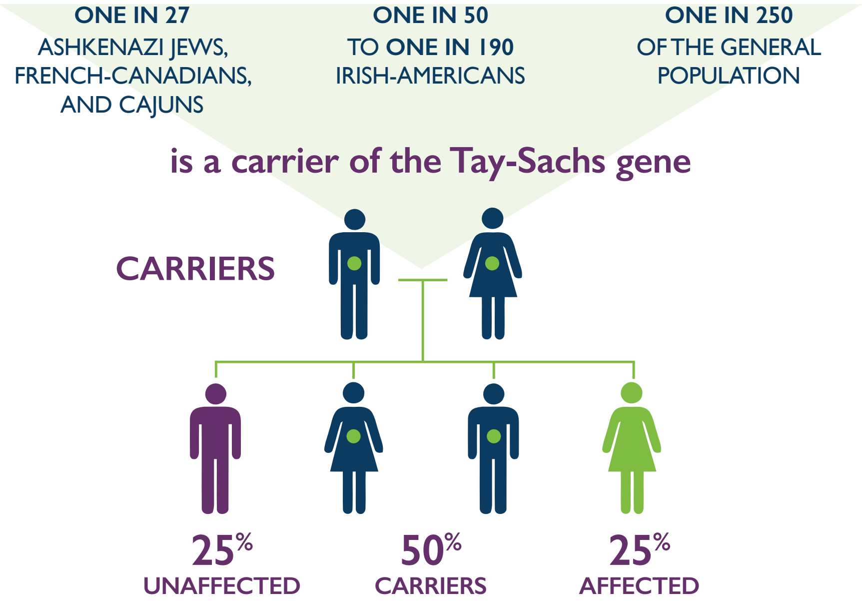
- **VOICES OF DETERMINATION**
  Chaired by NTSAD parent and advocate Alexis Buryk, this event raised awareness of Late Onset Tay-Sachs and raised over $130,000. It featured a conversation on determination and perseverance with Katie & Allie Buryk, twin daughters of Alexis and Bill Buryk who face Late Onset Tay-Sachs, and Katie Couric, Barbara Corcoran and Jill Abramson.
- **ANNUAL FORE JACK KLIGER MEMORIAL GOLF AND TENNIS OUTING**
  Hosted by Sam and Benedetta Kliger in memory of their son Jack, the Kliger family raised over $100,000 in Jack's memory in 2018.
- **IMAGINE & BELIEVE**
  Special guest Barry Shrage, former President of Combined Jewish Philanthropies, kicked off NTSAD's annual Boston fundraising event. He was followed by a conversation between NTSAD parent Mandy Ronaldson and UMass Medical School's Miguel Sena-Esteves, PhD. Mandy and Miguel (left) spoke about the value of affected families engaging in research. The evening raised almost $100,000 for NTSAD.

PLANNED GIVING

THE EVELYN AND LEONARD SUSSMAN LEGACY CIRCLE
Michael Sussman and Renee Licht made a generous gift to establish the Evelyn and Leonard Sussman Legacy Circle. The Sussman Legacy Circle, named for two of NTSAD's co-Founders, Evelyn and Leonard Sussman, is designed to recognize every individual and family in the NTSAD community who has included NTSAD in their will, estate, or long-term charitable giving.

PREVENTION EDUCATION & AWARENESS

Carrier screening remains essential for prevention of these diseases. Advances in screening methods, including expanded carrier screening, allow for individuals at risk to be identified, regardless of ethnic background. NTSAD continues to promote carrier screening for prevention of Tay-Sachs, Sandhoff, Canavan, and GM1 gangliosidosis. In pursuit of this objective, expert members of NTSAD's Education Committee developed an online education module, "Pre-pregnancy Carrier Screening" for the American College of Obstetricians & Gynecologists.



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

THE CARRIER RATES FOR OUR OTHER DISEASES ARE AS FOLLOWS:

CANAVAN DISEASES

ONE IN **50** ASHKENAZI JEWS
ONE IN **400** WORLDWIDE

GM1 GANGLIOSIDOSIS

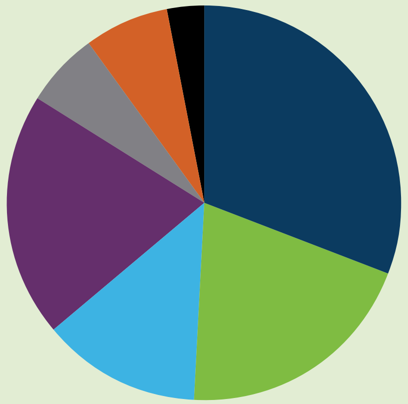
ONE IN **300** WORLDWIDE

SANDHOFF DISEASE

ONE IN **290** WORLDWIDE

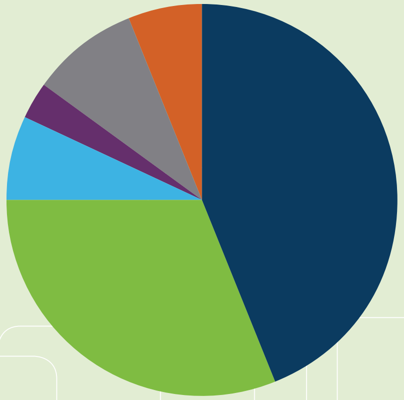
2018 FISCAL YEAR
Audited Financial Statements

SUPPORT & REVENUE
$1,254,385



31% RESEARCH	$390,156
20% GIFTS	$250,647
13% CONFERENCE	$165,382
20% SPECIAL EVENTS	$256,977
6% GRANTS	$73,100
7% RESTRICTED GIFTS	$80,926
3% OTHER	$37,197

EXPENSES
$899,380



44% RESEARCH	$397,270
31% FAMILY SERVICES	$277,823
7% EDUCATION	$66,684
3% ADVOCACY	$22,646
9% FUNDRAISING	$85,152
6% ADMINISTRATION	$49,805

NTSAD

NTSAD MISSION

NTSAD leads the fight to treat and cure Tay-Sachs, Sandhoff, GM1, Canavan and related genetic diseases, and supports affected families and individuals in leading fuller lives.

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Allied Diseases Association
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