NTSAD

NATIONAL TAY-SACHS
& ALLIED DISEASES

Moving Forward Together

2022 IMPACT REPORT

A Message from Leadership

Dear Friends,

It is our pleasure to present the National Tay-Sachs & Allied Diseases Association (NTSAD) Impact Report for the 2022 fiscal year, July 1, 2021, through June 30, 2022. We thank you for your investment in our shared mission of supporting families and driving research. As we reflect on the last year, it was a time of transition, resiliency, and scientific advancement. We remain dedicated to moving forward together, advancing research toward effective therapies, and providing meaningful support for children and adults affected with Tay-Sachs, Canavan, GM1, and Sandhoff diseases.

Moving Forward Together

During the last few years, the coronavirus pandemic presented multiple challenges to our organization. We adapted and offered new programs and services with specialized programming for families dealing with uncertainty, increased isolation, and virtual visits with healthcare professionals. In addition, we hosted Annual Family Conferences, Imagine & Believe events, and the Late Onset Think Tank meetings virtually in 2020 and in 2021.

We are now beginning to move toward a "new normal" environment and taking steps to restore in-person connections.

Our Community is moving forward—together. We are building on our rich history as one of the nation's oldest patient advocacy organizations founded 65 years ago. Promising gene therapies for children with Tay-Sachs, Canavan, GM1, and Sandhoff diseases are evolving, and researchers are planning late onset studies. We are looking ahead with cautious optimism.

Our Most Sincere Thanks

As you read this impact report, we want you to know how grateful we are to you and so many who have made contributions to Our Community. You share in our collective accomplishments. On behalf of the Board of Directors, staff, and the remarkable individuals and families who continue to inspire us, we extend our gratitude to the compassionate clinicians who provide expert care for affected children and adults, the devoted members of the scientific and academic research community who share their expertise; the dedicated industry partners who drive progress, and our generous donors who enable us to provide the support and services Our Community deserves.

Staci Kallish, DO
President, Board of Directors

Kathleen M. Flynn
Chief Executive Officer

A Continuation of Dedicated Leadership

In the spring of 2021, when NTSAD Executive Director **Susan Kahn** announced she was stepping down from her role, the Board of Directors led an extensive national search for the organization's next leader. As Executive Director for 14 years, Sue led the organization's growth, ushered Our Community into the advent of clinical trials, and left the organization fiscally sound and strong.

Kathleen Flynn was appointed Chief Executive Officer in November 2021. With more than three decades of experience in the corporate, academic, and healthcare advocacy sectors, Kathy promptly earned the confidence of the Board, staff, and key stakeholders in the rare disease community. She is well-positioned to lead NTSAD into its promising future along with the support and leadership from the Board of Directors.

Cover: Alya Rasul is living with Juvenile Tay-Sachs

FAMILY SERVICES AND SUPPORT

Connections Flourish Among Patients, Advocacy Groups, Researchers

NOTABLE NUMBERS

40+
families from
14
countries received direct support from NTSAD

300+
families and individuals from
40+
countries were actively engaged in caring for a loved one or themselves

1,000+
individuals participated in private Facebook groups

6 clinical trials and
2 natural history studies are currently underway

4
Jeffrey Alan and Stanley N. Gottlieb Scholarships were awarded to NTSAD Community members

Since 2005, Judith Gottlieb and her family have given
$80K+
in college scholarships to more than 45 siblings

Remembrance

In memory of Community members lost from July 2021 through June 2022.
We sincerely apologize for any unintentional omissions from this list.

Brynlee Jade Able
Stewart Altman
Riley Bushard
Prince Jahadi Vance Davis
Carley Haydel
Lindsay Karlin
Lylah Neace
Camila Recalde
Ava Rose Redfield
Sharon Wagner
Minahil Waseem

Family Services Provides Connection and Community during the Pandemic

One challenge during the COVID-19 pandemic was to keep families connected to each other and sources of support as they managed their child's care, their own well-being, and their grief as they navigated life without their loved ones. NTSAD Family Services programming evolved to meet their needs. With the help of Zoom and social media, the Family Services Team reached out to families and individuals directly and facilitated connections. They also sent specialized newsletters to guide and support families in the year following a diagnosis or the death of a loved one.

Partnering with Industry and Advocacy Groups to Advance Research

The Family Services Team brought in the voices of families and participated on a patient advisory board and two advisory committees, collaborating with industry partners who developed patient-centric educational materials, conducted a caregiver study, and launched a natural history study that will inform future clinical trials.

The Family Services Team also partnered with the Cure GM1 Foundation on the "GM1 Caregiver Experience Study" conducted by Research Triangle Institute (RTI). The study began in late 2020/early 2021 and was completed in 2022. The manuscript is scheduled for publication in 2023-2024.

Family Services Shares Expertise with Genetic Counseling Students

The Family Services Team has a long history of coaching and working with genetic counseling students. They were invited, once again, to speak to first-year genetic counseling students from Massachusetts General Hospital Institute of Health Professionals (MGH IHP). They introduced the students to NTSAD and described how we serve families living with rare genetic diseases. The NTSAD Family Services Team shared insights on how to counsel newly diagnosed families with utmost sensitivity and care. This was the second time the Team spoke to MGH IHP genetic counseling students.

Annual Family Conference Will Return to an In-Person Event in FY2023

Due to the ongoing pandemic, the last NTSAD Annual Family Conference was held virtually in April 2021. At the end of FY2022, plans were well underway for an in-person conference in July 2022 in Denver, Colorado—the first in-person conference since 2019.

RESEARCH

New Grants Inspire Hope for the Future

NOTABLE NUMBERS

64
grants awarded since the Research Initiative began in 2002

43
scientists received funding since 2002

In the past three years since clinical trials began for Tay-Sachs, Canavan, GM1, and Sandhoff diseases,

30+
children received gene therapy

70+
adults participated in drug development trials

Research Initiative Re-launched

Grants Awarded to Support Gene Therapy, Rating Scale, and Newborn Screening

In January 2022, NTSAD re-launched the Research Initiative Program after a pause for extensive strategic planning and review in 2019. The newly refocused program released a Request for Proposals (RFP), inviting researchers to submit applications for consideration.

Seven applications were received, and one was ultimately selected for funding: a two-year, $140,000 grant was awarded to research fellow Amanda Gross, PhD, at Auburn University in Alabama for "Dual-Site Administration of AAV Gene Therapy for Treatment of Feline GM1 Gangliosidosis."

During the year, NTSAD also awarded two additional grants:

- Elise Townsend, DPT, PhD, PCS and Associate Professor, Associate Director of the PhD in Rehabilitation Science program at Massachusetts General Hospital, was awarded a two-year, $139,793 grant for the "Construction and Validation of the Infantile GM2 Rating Scale."
- As part of the work of NTSAD's Newborn Screening Consortium, NTSAD coordinated and contributed to a bridge grant, which included funding commitments from industry co-sponsors (Taysha Gene Therapies, BridgeBio, and Sio Gene Therapies). NTSAD contributed $5,000 to Gelb Labs (Michael Gelb, PhD; Hamid Khaledi, PhD; GelbChem) for the "Development of a Newborn Screening Assay for GM2." The work later received a Small Business Technology Transfer (STTR) grant. The bridge grant accelerated the research more than six months before the STTR funding became available.

Late Onset Tay-Sachs and Sandhoff (LOTSS) Think Tank Brings Key Stakeholders Together

Approximately 30 researchers, clinicians, industry representatives, and patient advocates gathered at the 4th Annual Late Onset Tay-Sachs and Sandhoff (LOTSS) Think Tank Meeting held virtually on October 14 and 15, 2021, to share the most recent developments in the field. The two-day symposium was sponsored by the Katie & Allie Buryk Research Fund and NTSAD. Alexis Buryk, a parent of twin daughters Katie and Allie who have Late Onset Tay-Sachs disease, inspired the creation of the Think Tank to accelerate finding effective treatments for LOTSS. Many participants have remarked on the extraordinary spirit of collaboration and openness that characterized the meeting and contribute to its ongoing success.

DEVELOPMENT AND COMMUNICATIONS

Donors are a Driving Force Behind NTSAD

NOTABLE NUMBERS

6,377
people received our monthly Community e-newsletters

875
gifts

totaling **over**
$241K
were made to the Annual Fund

41,002
people visited our website

25+
families hosted Day of Hope events to raise awareness and funds for research

SOCIAL MEDIA FOLLOWERS

3,200 Facebook

608 Twitter

567 Instagram

467 LinkedIn

84 YouTube

Imagine & Believe

Imagine & Believe Provides Opportunity for Families to Connect

Imagine & Believe, NTSAD's signature annual fundraising event, raised $120,000 for programs and services in 2021, with more than 200 people tuning in virtually for connection and community and a chance to celebrate outgoing Executive Director Sue Kahn as the event's honoree. Families shared their stories and thanked Sue for her 14 years of service and support of rare families and leadership in advancing research toward multiple clinical trials for Our Community.

Day of Hope Raises Nearly $75,000 for Research

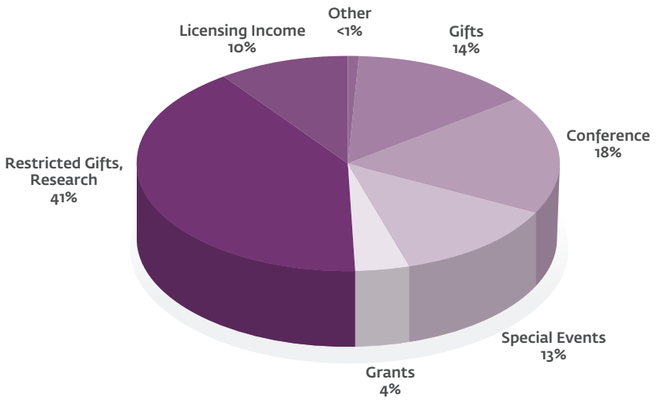
More than 25 individuals, families, and companies hosted events in honor of loved ones and the rare community for the 11th Annual Day of Hope in 2021, raising $74,658 for research. Day of Hope events unite people in our shared cause to find effective treatments. Since September 2011, Day of Hope has raised nearly $550,000 for the NTSAD Research Initiative.

The Evelyn and Leonard Sussman Family Legacy Circle is Expanding

In August 2021, NTSAD received a six-figure legacy gift from one member of the Evelyn and Leonard Sussman Family Legacy Circle, named after one of NTSAD's founding families. Membership is open to individuals and families who include NTSAD in their estate plans.

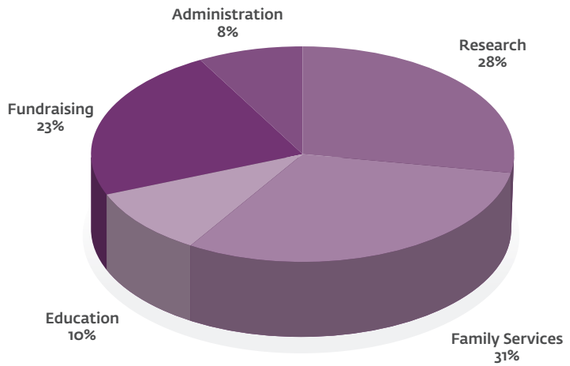
Financial Information

REVENUE



Gifts	$179,875
Conference	$232,337
Special Events	$157,489
Grants	$46,288
Restricted Gifts, Research	$512,638
Licensing Income	$123,070
Other	$1,411

EXPENSES



Research	$212,070
Family Services	$238,290
Education	$79,963
Fundraising	$180,110
Administration	$60,357

Moving Forward Together

As you can see from this Impact Report, NTSAD thrives on the continued support of every member of Our Community—families and individuals, scientific and academic researchers, industry partners, and donors. As we look ahead to the future, we are grateful for the many contributions we've received, and inspired by the possibilities ahead.

National Tay-Sachs & Allied Diseases Association

National Tay-Sachs & Allied Diseases Association
2001 Beacon Street | Suite 204 | Boston, MA 02135
(617) 277-4463 | www.NTSAD.org
EIN# 13-1912877

Follow Us

Vision Statement

NTSAD envisions a world in which Tay-Sachs, Canavan, GM1, and Sandhoff diseases are no longer fatal or debilitating. At NTSAD, we will:

- Above all else, provide compassionate support, advocate for patients and their families, and promote early diagnosis and prevention.
- Empower, educate, and connect affected families and individuals.
- Serve as the preeminent resource for families, industry members, researchers, and clinicians.
- Direct, promote, and invest in research to accelerate the development of treatments and cures.
- Act as a leader within the rare disease community.

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.