

Having trouble viewing this email? [Click here](#)



National Tay-Sachs &  
Allied Diseases Association, Inc.

## Keeping Up with NTSAD

### July 2014 - In This Issue:

[NTSAD News](#)

[Research News](#)

[2015 Conference Location Announcement](#)

[2014 Quality Control Results](#)

[Meet Xavier](#)

[Upcoming Events](#)



**TOGETHER.**  
**We can make a difference.**

## NTSAD News

### A New Fiscal Year...

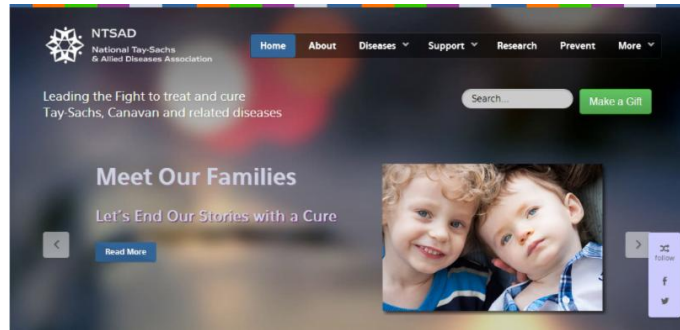
It is a new year for NTSAD and with that comes a refreshed look at the year ahead. The **NTSAD website** has a new look due to a move to a mobile-friendly platform. It is a fresh look that can be viewed on smartphones, tablets and laptops. One of the dynamic features will allow families to have their own giving pages in honor of their loved ones. As the site is fine-tuned, feedback is always welcome.

Another change as of July 1st is the closing of the **NTSAD New York Area Chapter**. It has transferred its administration to the national office of NTSAD. The



chapter's focus on education, awareness and research will remain active in the Tri-State Area. Its activities will quietly transition over the next several

months. The work of the chapter, however, was celebrated with quite a bang at an NTSAD fundraiser on May 8th in Manhattan. Co-president, Marion Yanovsky was honored for her nearly 50 years of volunteering for NTSAD. Over \$80,000 was raised to continue the work of the NTSAD New York Area Chapter.



## RESEARCH

### Latest Tay-Sachs Gene Therapy (TSGT) News

The **TSGT Consortium** team met a significant milestone after passing the 90 day mark post-injection for the non-human primates. The MRIs indicate no changes and further testing will confirm the levels of enzyme found in the brains. The next steps will plan for production of the vectors for further toxicology studies, as well as preparing for interactions with the Food & Drug Administration (FDA) and regulatory filings. No start date has been set for clinical trials.

### 2014 Research Initiative Grant Award

We are happy to announce that NTSAD awarded a 2014 Research Initiative grant to BioStrategies, LC with David Radin, PhD as Principal Investigator for the project, "Lectin-assisted transnasal delivery of corrective enzyme for GM1 gangliosidosis." The grant is a two-year \$80,000 milestone-based grant.



A summary of the proposal can be found [here](#) on NTSAD's website.

### New NTSAD Corporate Advisory Council

NTSAD's Board of Directors has been engaged in Strategic Planning to develop Roadmap 2018, which outlines how NTSAD will advance research to treatments, better serve our families, and promote accurate and effective carrier screening. Research Strategy was recognizing that NTSAD would benefit from expert industry insights as we navigate future clinical trials, the regulatory process, and getting treatments to people who need them. Additionally, we realized that some NTSAD friends working in the biotech industry have extensive experience and could advise us in these areas, as well as use their networks to explore funding and partnering options.

Thus, the idea of the **Corporate Advisory Council (CAC)** was born. The CAC will help NTSAD as we prepare for future treatments for this family of rare genetic diseases and will complement the scientific, clinical, and lab expertise brought by our **Scientific Advisory**

**Committee (SAC).** Together, the SAC and CAC will help NTSAD advance research programs, build our clinical trial readiness, and make our diseases appealing to companies to study.

### Million Dollar Bike Ride for Rare Disease Research

The **Million Dollar Bike Ride**, held on May 3, 2014 in Philadelphia, exceeded its goal and raised \$1.4 million for rare disease research! UPenn's Center for Orphan Disease Research and Therapy more than matched the funds raised which means that **\$35,000** will allow for a dedicated post-doctoral fellowship grant on NTSAD's behalf! The Request for Applications (RFA) will be available in early August. Please contact [ingrid@ntsad.org](mailto:ingrid@ntsad.org) if you wish to receive a copy of the RFA.



**Mark your calendars for next year's ride - May 9, 2015!**

## FAMILY SERVICES

### 2015 Annual Family Conference Location Announced

NTSAD's 37th Annual Family Conference will be held **April 16 to April 19, 2015** at the Hyatt Regency Hotel in Reston, Virginia. The location is accessible by three major airports - Dulles International (IAD), Reagan National (DCA), and Baltimore (BWI). There are plans to have metro service extended to Reston by next spring which will offer easy access into the nation's capital!

### Seeking Sponsors for 2015 Annual Family Conference

The Annual Family Conference is made possible by the exceptional generosity of individuals and families through sponsorships. Sponsorships range from a table at a meal, childcare, sibling retreat to sessions, full meals, or helping a family attend the conference (travel, hotel). Sponsor levels and the forms will be available on our website soon.



If you, your family and friends, your employer or a foundation you know would like to underwrite a portion of the conference, please contact Joan Lawrence, Development Director at [joan@ntsad.org](mailto:joan@ntsad.org) or (617) 277-4463.

## EDUCATION

### International Quality Control Tay-Sachs Testing Program 2013-2014 Quality Control Lab Results

Until a cure is found, carrier screening along with education and awareness are the best tools to stop Tay-Sachs and related diseases. While NTSAD works towards a future

cure, we continue to support prevention through accurate carrier screening. NTSAD administers a laboratory proficiency testing program to ensure quality enzyme testing results.

The 2013-2014 [International Tay-Sachs Carrier Screening Quality Control Program](#) results are now available here. Over twenty-four labs around the world participated. These labs tested over 30,000 potential carriers last year.

NTSAD administers this program, which was founded by Dr. Michael Kaback and run at the University of California-San Diego for 25 years. Since 2007, the program has been based at the lab of Dr. Mimi Blitzer and Dr. Erin Strovel at the University of Maryland.

To read more about the program, visit [this link](#) on our website.



## Meet Xavier

His quiet spirit defies the aggressive nature of the Juvenile Tay-Sachs that is robbing him of his childhood and future. As one of five children, Xavier increasing needs are overwhelming for his parents. NTSAD's resources give them respite and reassurance as they cope day-to-day.

**Give today** and help NTSAD provide services to families like Xavier's.

## Upcoming NTSAD Happenings

NTSAD's work is made possible thanks to the support of our annual events and gifts to the Annual Fund. NTSAD solely relies on the generosity and philanthropy of private gifts and grants.

### [Sixth Annual 'Fore' Jack Memorial Golf & Tennis Outing](#)

**Wednesday, September 17, 2014**

**Piping Rock Club, Locust Valley, NY**

The Kliger family will host their sixth golf and tennis outing on Wednesday, September 17th in memory of their son, Jack, whose valiant battle with Canavan disease ended when he was 14 years old. To take part in this wonderful event, contact Joan Lawrence, Development Director, at [joan@ntsad.org](mailto:joan@ntsad.org).



### **Fourth Annual Day of Hope, September 20, 2014**

It is an NTSAD tradition now to raise and rally funds for research in the months leading up to September every year. This day gives families, their friends and community the opportunity to shout their children's names, raise awareness and fundraise for research. Activities range from lemonade stands and bake sales to community walks and spaghetti dinners. Our goal

is to raise at least \$50,000 this year for research. To see if something is happening near you or to get ideas, visit the NTSAD Events page [here](#).

If you'd like to do some kind of activity or donate to support this special day, visit [www.NTSAD.org](http://www.NTSAD.org) for more information.

**Seventh Annual  
Boston Benefit  
Imagine & Believe,  
November 13, 2014**

The date has been set for another fantastic evening celebrating NTSAD's work. It will take place on



Thursday, November 13th at the Royal Sonesta Hotel in Cambridge, MA. We are thrilled to honor [Stephen Groft, Pharm.D.](#), former head of the Office of Rare Disease Research at the National Institutes of Health in Washington, D.C.

To sponsor this special evening, purchase an ad in the program book or buy tickets, contact Joan Lawrence, Development Director, at [joan@ntsad.org](mailto:joan@ntsad.org) or (617) 277-4463.



**I want to give NTSAD  
my support today.**

**Give a gift [here](#).**

Sue Kahn, Executive Director ([skahn@ntsad.org](mailto:skahn@ntsad.org))  
Kim Kubilus, Family Services Director ([kim@ntsad.org](mailto:kim@ntsad.org))  
Joan Lawrence, Development Director ([joan@ntsad.org](mailto:joan@ntsad.org))  
Ingrid Miller, Office Manager ([ingrid@ntsad.org](mailto:ingrid@ntsad.org))  
Diana Pangonis, Communications Manager ([diana@ntsad.org](mailto:diana@ntsad.org))

NATIONAL TAY-SACHS  
& Allied Diseases Association, Inc.  
2001 Beacon St - #204  
Boston, MA 02135  
(617) 277-4463 \* [www.NTSAD.org](http://www.NTSAD.org)

STAY CONNECTED



[Forward this email](#)



This email was sent to [diana@ntsad.org](mailto:diana@ntsad.org) by [skahn@ntsad.org](mailto:skahn@ntsad.org) |  
[Update Profile/Email Address](#) | Rapid removal with [SafeUnsubscribe™](#) | [Privacy Policy](#).



National Tay-Sachs & Allied Diseases Association | 2001 Beacon Street, #204 | Boston | MA | 02135