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National Tay-Sachs &
Allied Diseases Association, Inc.

Keeping Up with NTSAD

a monthly e-newsletter

January 2015 - In This Issue:

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

RESEARCH

A Profound Impact on LOTS Research

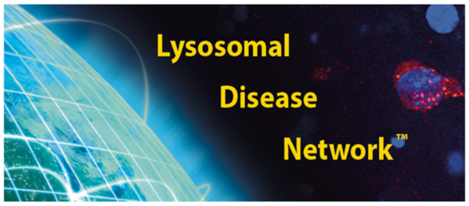
Katie and Allie, two dynamic and independent young women, are twins who recently learned, after eight years of searching, that they have **Late Onset Tay-Sachs.** Now that they have an answer, the family is now channeling their energy into fundraising for research. Through their NTSAD giving page they have now raised over $100,000 for the **Katie & Allie Buryk Research Fund** and Late Onset Tay-Sachs research. In just five weeks they have not only surpassed their initial goal, they have introduced Late Onset Tay-Sachs into the vernacular of their friends, family and communities. Gifts continue to come in to support them as they come to terms with their diagnoses. To learn more, visit their page **here**.

NTSAD's Scientific Advisory Committee Subcommittee on Therapies

The landscape of research is vast and thus there is a lot of information that can be found on the internet. Unfortunately some of the information is not beneficial or safe for families. This new NTSAD subcommittee's goal is to "evaluate clinical trials or other proposed therapies for Tay-Sachs, GM-1, Sandhoff, Canavan and related rare genetic diseases." They will look at the information "regarding clinical trials and how to asses a proposed new or untested therapy." The first paper, featured in this month's *Research Review* focused on substrate reduction therapies for lysosomal storage diseases. Read the paper **here** and learn more about the **Subcommittee on Therapies** **here**.

Read the recent issues of NTSAD's ***Research Reviews*** in NTSAD's website library **here.**

Lysosomal Disease Network (LDN) Fellowship Applications OPEN



One of NTSAD's partner organizations, LDN, is currently accepting applications forthe LDN Fellowship. The grant, using NIH funding, will provide $50,000 in total costs retroactively for the period of September 1, 2014 to August 31, 2015.

Applications will be accepted that have a focus on *clinical research of lysosomal diseases* with priority given to those applicants who seek a career with a focus on clinical research of lysosomal diseases.

Awards will be announced February 13, 2015 and will begin immediately.

Read more about the applicant criteria on LDN's website **here**.

FAMILY SUPPORT

NTSAD Family Conference 2015

The Annual Family Conference is a highlight of the year for NTSAD's families.
The conference will be held this year at the Hyatt Regency in Reston, Virginia from Thursday, April 16th to Sunday, April 19th. The sessions give families and individuals the

tools to care for their loved ones, themselves and the opportunity to connect with others. It often is the one time of year where they can feel "normal;" and that includes the healthy siblings, many of whom have grown up together.

Be a Part of the Conference!

A gift of sponsorship is a wonderful way to support the conference experience, the families, their children and the adults affected by these rare genetic diseases. The opportunities range from underwriting a particular session to either sponsoring an entire meal or simply a table. All sponsors are recognized in conference materials, NTSAD's website and at meals. To learn more, please contact Joan Lawrence, Director of Development, at **joan@ntsad.org**.

[The conference] is the one place I look forward to going every year where I can be with my family and it's OK to laugh and cry at the same time - where I don't have to say anything, everyone knows and understands exactly how I'm feeling.

ADVOCACY

Rare Disease Day ® 2015

This year, on Saturday, February 28, 2015, the rare disease community comes together to honor the millions whose lives have been touched by rare diseases. Hence, the theme for this year, "Living with a Rare Disease." Many of NTSAD's families recognize the day by changing their Facebook profile pictures to an "I Love Someone Who is Rare" image. In Boston, the national office attends events being held in the Greater Boston area saluting the rare disease community. Anyone can be involved.

Ways to Get Involved

The **National Organization for Rare Disorders (NORD)** shared a list of ways to get involved on their Rare Disease Day website. From "Sharing Your Handprints" to attending local state house events you can join the rare disease community in paying tribute! Visit **here** to learn more.

Boston State House Event

If you live in the Greater Boston area, consider joining the rare disease community at the Great Hall in the State House on Monday, February 23, 2015. Pre-register for this free event **here**.

There will be an opportunity to meet and speak with legislative representatives through prearranged meetings to discuss in particular the importance of Bill S2293 - an act relative to patient financial protection which will greatly impact patients with rare diseases. Information and talking points will be provided in advance of meeting with legislators.

Connecting with Empowered Patients & Inspire

Inspire together we're better

Free 1/2 day seminar
Wednesday, February 4, 2015
9:00am to 1:00pm
BIO Offices in Washington, D.C.

The seminar will convene leaders from the patient advocacy community and life sciences industry to discuss how social media can be utilized to empower patients and engage advocates. Space is limited. Breakfast and lunch will be provided.

To participate, please RSVP directly to Gautami Inamdar at **ginamdar@bio.org**.

HELP BOOST OUR IMPACT

There are many ways to make a ripple that will have a long-lasting effect on what NTSAD does every day.

Riders from Left to Right are: Brian Matthews, Peter Scott, Cedric Bru, Patrick Heron, Mike Ward, Josh Wilson, Bradley Campbell, Rick Orr, Douglas Roeder, Jake Nunn

On January 15, 2015 at the JP Morgan Healthcare Conference in San Francisco, several bike enthusiasts took to the road to benefit NTSAD. Led by NTSAD Board member and past president, Bradley Campbell, the "Ride to Freedom" raised close to $2,000. Thank you to the team for raising awareness about NTSAD and our family of rare genetic diseases!

As they say, "the squeaky wheel gets the oil" and NTSAD's families are committed to being as loud as they can on behalf of their children and loved ones until an effective treatment or cure is found. Whatever you can do to stand and shout with them would be greatly appreciated. **Gifts supporting NTSAD can be made here.**

☑ I want to give NTSAD my support today.

Give a gift here.

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