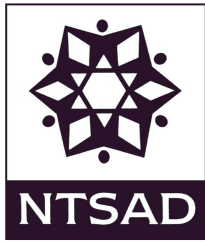


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National Tay-Sachs &
Allied Diseases Association, Inc.

Keeping Up with NTSAD
a monthly e-newsletter

September 2014 - In This Issue:

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Global Genes Summit

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TOGETHER.
We can make a difference.

NTSAD in Action

Vision without action is merely a dream. Action without vision just passes the time. Vision with action can change the world. ~ Joel A. Barker

NTSAD's mission is to change the course of the rare diseases that currently bind children and adults to a fate out of their control. Being part of a larger global rare disease community enables NTSAD and families to affect change on a government, legislative and industry level. Within the NTSAD family, however, families take action every time they share their stories with a stranger, host an event to raise funds for research or educate a physician, nurse or home care worker. These actions all have an impact on the future of diseases like Tay-Sachs, Sandhoff, Canavan, and GM-1.

NTSAD sibling, Jessica, whose younger sister, Rachel is affected by **Canavan Disease**, is now working with **Guangping Gao, PhD** at his lab at the University of Massachusetts Medical Center. Dr. Gao is a researcher at the forefront of **Canavan gene therapy**. Jess is a currently a senior in high school and has set her sights on becoming a neuroscientist to help find a cure for the disease that she is all too familiar with at her age. Her sister, Rachel was diagnosed as an infant and has lived all 16 years at the mercy of the effects of Canavan disease.



Rachel and Jess



Justin's first day in the lab, albeit overdressed for the occasion!

Another sibling, Justin, had the opportunity this summer to work with Miguel Sena-Esteves, PhD, in his lab at the University of Massachusetts Medical Center. Dr. Sena-Esteves is the principal investigator of the **Tay-Sachs Gene Therapy Consortium**. Justin's goal to be a neurosurgeon is inspired by his brother, Evan, who died of Tay-Sachs when Justin was just a couple of years old. Justin wrote:

"Having the opportunity to work in Miguel's lab this summer was incredible. Working on finding a cure for **Tay-Sachs**, the disease that my brother Evan died from, was by far the most meaningful experience of my life. It gave me a true sense of fulfillment knowing that I had a hand in the research that will hopefully save other children and families the suffering Evan and our family lived through. I hope to be a neurosurgeon in the future and deliver and be able to deliver the treatment to affected children."

EDUCATION, AWARENESS & ADVOCACY



Global Genes Rare Patient Advocacy Summit Held September 11-13th, 2014 in California

The saying "there is strength in numbers" was clearly evident during the recently held **Global Genes** summit in California. The summit gave patient advocates the opportunity to learn from experts, network and through discussions and shared insights gain the tools to "move down the advocacy path, equipped and prepared." NTSAD's Executive Director, Sue Kahn, attended the summit weekend. She found that their sessions focusing on requirements for successful drug development and the patient voice were helpful in guiding how NTSAD moves forward advocating for our families and research. "I'm proud that NTSAD is collaborating with Global Genes as part of the larger rare disease community where one voice is a powerful tool."



MEET ANNABEL.

Sister to Amelia. Daughter to Casey and Tommy. She is just 22 months old and has Tay-Sachs. The disease is robbing her of a bright and beautiful future that should lie ahead of her. Her parents, however, are embracing every minute and day they have and generously share her with all of us. Please **make a gift** to research today to afford children in the future happy and healthy lives!

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.



Every dollar will go to research, so your gift will make a huge impact!

Fourth Annual Day of Hope - Results to Date

Families, their friends and communities have been busy this last month observing the Fourth Annual Day of Hope to rally and raise funds for research! We thank the nearly 1,000 people who have supported these events in some way worldwide! To date **nearly \$30,000** has been raised, bringing us 60% of the way to our goal! **We ask you to consider making a gift to boost the efforts of the remaining events to be held. If just 100 people give \$100 in the next week, we will make it!** Stay tuned for the final results in the coming weeks!

Events on Saturday, September 27, 2014

Day of Hope Lemonade & Bake Sale

Courtesy of the Lord Family in memory of Cameron & Hayden
Millennium Park Soccer Fields
West Roxbury, MA
8:30m to 2pm

Make a gift [here](#) in honor of Cameron & Hayden.

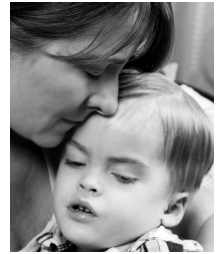


A Spaghetti Dinner to Remember in Memory of Kaleb

American Legion Winter Garden
271 W. Plant St.
Winter Garden, FL 34787
From 3:00pm-7:00pm
Silent Auction - Bidding online until 10:00pm, Friday, September 26th

<http://ogdenfamily.weebly.com/silent-auction-items.html>.

Make a gift [here](#) in honor of Kaleb.



Brianna's Day of Hope Poker Run for NTSAD

Country Keg
1790 N Walker St
Graytown, Ohio 43432



First bike out at 11am. Last bike in at 5pm.

Stops include:
Portage Inn, Kate & Al's, Blue Eagle,
Little Bar on the Prairie and Happy Hour

\$15 per rider * \$5 per passenger * \$20 per car
\$10 cover charge for those who don't want to go bar to bar

Entertainment by: R & J MUSIC MIX

Pizza & Salad buffet \$\$
50/50, silent auction, raffles, FOOD, FUN, PRIZES.

CALL TO PRE-REGISTER TODAY 419-637-7586 and have your name put in a special pre-registration drawing for a prize.

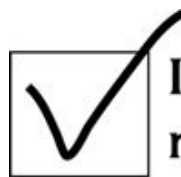
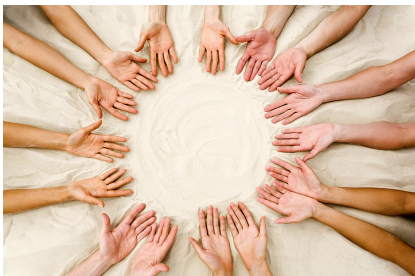
Make a gift [here](#) in honor of Brianna.

7th Annual Boston Benefit:Â Imagine & BelieveÂ
Thursday, November 13, 2014
Royal Sonesta Hotel, Cambridge, MA

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. or (617) 277-4463. You can also [RSVP online here](#).



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

Give a gift [here](#).

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