NTSAD

national tay-sachs & allied diseases association

RESEARCH REVIEW | February 10, 2017

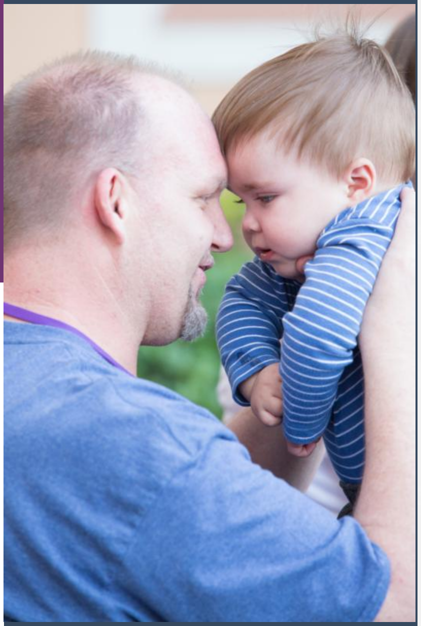
2017 NTSAD ANNUAL FAMILY CONFERENCE

Research Session | March 31, 2017 | Dallas, TX

The NTSAD Research Session is a highlight of the Annual Family Conference and an opportunity for families to learn the latest news on Tay-Sachs, Sandhoff, GM1 and Canavan research developments. It is sponsored by the **Mathew Forbes Romer Foundation**, NTSAD's South Florida affiliate.

This year, we have put together a new format for this session based on what we heard from families and what has been in the news. We have planned a panel on Gene Therapy for the gangliosidoses (Tay-Sachs, Sandhoff, GM1) and Canavan disease. Following this discussion will be a panel about Families' Involvement in Research and ongoing Clinical Trial Readiness. Each discussion will be followed by a Question & Answer period and an opportunity to speak with researchers at the luncheon that follows.

This session will be recorded and shared on our website after the conference. An companion booklet featuring summaries of research in our rare diseases will be available to download on our website and will be in every conference packet at registration.



CLICK HERE TO REGISTER & LEARN MORE ABOUT NTSAD'S 39TH ANNUAL FAMILY CONFERENCE.

MARCH 30 - APRIL 2, 2017
DALLAS, TX

Gene Therapy Panel

The goal of this panel discussion is to

Clinical Trial Readiness

The goal of this discussion is to understand

The goal of this panel discussion is to understand the state of gene therapy today as it relates to the gangliosidoses and Canavan disease.

Moderator:

Fran Platt, PhD

Oxford University (UK)

NTSAD Scientific Advisory Committee Chair

Panelists:

Joe Anderson, PhD

University of California, Davis

GM2 (Tay-Sachs, Sandhoff)

Dominic Gessler

Univ. of Massachusetts Medical School

Canavan disease

Doug Martin, PhD

Auburn University

GM1 and GM2 (Tay-Sachs, Sandhoff)

Miguel Sena-Esteves, PhD

Univ. of Massachusetts Medical School

GM1 and GM2 (Tay-Sachs, Sandhoff)

The goal of this discussion is to understand the importance of natural history studies, registries and being familiar with the regulatory process.

Moderator:

Cynthia Tifft, MD, PhD

Deputy Clinical Director

National Human Genome Research Institute, NIH

Panelists:

Florian Eichler, MD

Massachusetts General Hospital

GM2

Heather Lau, MD

New York University

Canavan

Samantha Parker

Chief Patient Access Officer

Lysogene

GM1

Cordula Schwarz

Manager Regulatory Affairs

Genzyme, a Sanofi company

MAY 20, 2017 | PHILADELPHIA

MILLION DOLLAR BIKE RIDE

NTSAD Conference is for Canavan Families Too!

JOIN TEAM NTSAD &

RIDE FOR RESEARCH

If you or anyone you know likes to bike ride, consider bicycling for Team NTSAD on May 20, 2017 to raise rare awareness and funds for research! There are three routes - 12 miles, 33 miles or 73 miles - for all ages and levels. Make your plans today!

Learn more and register **here**!

The annual **WORLD Symposium** takes place next week from February 13th to 17th in San Diego where over 1,000 clinicians, researchers, companies, patients, and patient advocacy groups from around the world gather to discuss the latest research and therapy advancements in lysosomal storage diseases.

NTSAD's Executive Director, Sue Kahn, will be attending and looks forward to connecting with our NTSAD family and network there. Please get in touch with her **here** if you'd like to learn more about NTSAD's research program. A number of people from the NTSAD family and network will also be in attendance.

In addition to support sessions for families, parents and grandparents, there will be an opportunity to meet with Heather Lau, MD to learn more about the natural history study she is conducting with co-investigator, Paola Leone, PhD. She wants to meet you to discuss what they're doing and invite you to participate.

This study dovetails with the **Canavan Disease Patient Insight Network (PIN)** **"**a patient focused opportunity to build one central and international network of information on families living with Canavan. By gathering the experiences of families from around the world, we are creating a research-ready community poised to help drug developers and researchers get closer to treatments and a cure." Canavan Research Illinois and founder, Ilyce Randell, launched the PIN and collaborates now with NTSAD, Canavan Foundation, Rare Disease Foundation and Think Genetic. Learn more about the PIN **here**.

There will also be an opportunity at the conference to hear about Guangping Gao, MD and Dominic Gessler's work on gene therapy for Canavan disease. Dominic will be a part of a panel discussion focused on gene therapy during NTSAD's Research Session on Friday, March 31, 2017 in Dallas.

Register for the conference **here**.

Learn more **here** about the conference and our Helping Hand Grant Fund.

Program highlights include:

- Konrad Sandhoff, whom Sandhoff disease is named after, will be giving the keynote lecture.
- Heather Gray-Edwards from Auburn will be presenting her research results about AAV gene therapy in sheep.
- Our 2015 Million Dollar Bike Ride grantee, Marlene Jacobson, PhD, will have a poster on her research results as will NTSAD Research Initiative grantee David Radin of BioStrategies.

Stay tuned for a report about the WORLD highlights in a future **Research Review** issue.

Support Research.Make a Gift Here.

2017 NTSAD RESEARCH INITIATIVE REQUEST FOR PROPOSALS

We invited six pre-applications to submit full proposals. They range in focus from basic to translational and come from the United States, Australia and Europe. Grant awards will be made in April.

Susan Kahn, Executive Director

Brian Manning, President, NTSAD Board

Fran Platt, PhD, Chair, Scientific Advisory Committee

Veronica Huang, Science Communications Intern

2001 Beacon Street, Suite 204

Boston, MA 02135

(617) 277-4463 | info@ntsad.org

www.NTSAD.org