



A New Team, A New Focus on Clinical Trials

On July 10th, members of NTSAD's Scientific Advisory Committee, clinicians from industry involved in rare diseases, researchers, a systems expert, and NTSAD representatives gathered at a **GM2 Clinical Research Network** meeting in Boston at Massachusetts General Hospital. While the meeting focused on GM2, the experts gathered are knowledgeable about Tay-Sachs, Sandhoff, Canavan, and GM1, so the discussion applied to all of our diseases. All are interested in working together to expedite clinical trials for GM2 and related diseases.



In attendance were (from left to right in photo):
Back Row: Gerry Cox, MD, PhD, Sue Kahn, Cynthia Tiftt, MD, Alex Sherman, Staci Kallish, DO, Swati Swathe, MD, Heather Lau, MD, Mike Gladstone, Julie Kissell, MS, Inna Tsvang
Front Row: Florian Eichler, MD, Ed Kolodny, MD
Missing in photo: Miguel Sena Esteves, PhD, Barbara Shapiro, MD, Gustavo Maegawa, MD, PhD

What data is important to gather and why?

- Standardizing the data collected from patients so all physicians seeing these patients are recording and sharing the same information (with patient or family consent, of course!) This will help us learn more about these diseases and create enough data to find meaningful clinical endpoints for trials.
- Identify the ideal patient population for trials (whether infantile, juvenile, or late onset patients; whether to study one disease or both Tay Sachs and Sandhoff together).
- Finding biomarkers that respond to treatment faster than

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Clinical Research Network

A Clinical Research Network is a global, collaborative network of healthcare professionals and researchers that supports clinical and translational research and are working together to find a cure for a disease or group of diseases.

The network can accelerate research into our diseases by promoting inter-institutional and international collaboration, and resource and information sharing.

Million Dollar Bike Ride RESULTS!

On May 3rd, Team NTSAD rode in the Million Dollar Bike Ride for 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 if you wish to receive a copy of the RFA.

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

clinical response. This means finding surrogate markers in blood, other body fluids, or imaging studies that can be correlated with disease progression or stabilization.

Collectively, this information will help us reach our goal of **Clinical Trial Readiness**.

What was discussed during the meeting?

- The group reviewed the body of knowledge we have about the natural history and biomarker studies for the gangliosides.
- The group discussed systems to collect data and resources that can be shared. It is crucial to combine as much clinical data as possible to be able to learn about the natural history of these diseases. This system should also allow researchers to share samples from patients, study these samples for possible biomarkers, and correlate the biomarker findings to clinical information.

What are the next steps that the group is working on:

- Identifying which clinical information should be collected and shared for each disease.
- Reviewing the current data about these diseases to recommend outcome measures and biomarkers for future clinical trials.
- It was emphasized throughout the meeting that these steps in determining data to be collected, clinical outcome measures, and biomarkers can be applied to other diseases. A data system to collect information can also be useful in other diseases. One of the clinicians at the meeting expressed an interest in setting up a similar network for Canavan disease.

This meeting was just a first step to define and explore what is needed to achieve our goal of Clinical Trial Readiness for all of our diseases. Stay tuned for more updates on this topic and on our progress in future *Topic of the Week: Research Reviews*.

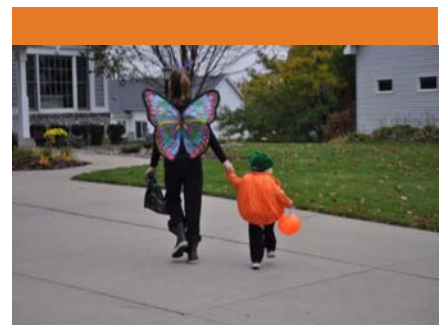
Read WHY?Y's coverage of this year's ride and see the slideshow [here](#) featuring Team Captain Ruth Feldman!



Research + Funding = Hope

Support Research with a gift [here](#).

Read more about current and past grants funded by NTSAD on our website [here](#).



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Stay Connected to NTSAD!



Progress lies not in enhancing what is, but in advancing toward what will be.
Khalil Gibran

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