National Tay-Sachs &
Allied Diseases Association

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National Tay-Sachs & Allied Diseases Association
Hosts First of Its Kind Drug Development Meeting for GM2

Boston, Mass. January 31, 2024—The National Tay-Sachs & Allied Diseases Association (NTSAD), leader in the worldwide fight to treat and cure Tay-Sachs, Canavan, GM1 and Sandhoff diseases, is hosting the first-ever, Externally-Led Patient-Focused Drug Development Meeting for GM2 gangliosidoses (Tay-Sachs and Sandhoff diseases) on Thursday, February 15, 2024.

Externally-led Patient-Focused Drug Development (EL-PFDD) meetings give the U.S. Food and Drug Administration (FDA) and other key stakeholders, including medical product developers, health care providers, regulatory agencies, an important opportunity to hear directly from patients, their families, caregivers, and patient advocates about the symptoms that matter most to them, the impact the disease has on patients' daily lives, and patients' experiences with currently available treatments. This input can inform FDA's decisions and oversight both during drug development and during their review of a marketing application.

GM2 can affect anyone no matter race, gender, ethnicity or country of origin. NTSAD alone serves hundreds of families in the U.S. and globally. To date more than 150 people including patients, researchers, clinicians, and members of industry have registered for the GM2 EL-PFDD meeting. This virtual meeting is livestreamed via YouTube, open to the public, and free to attend. However, participants must register in advance at www.ntsad.org/gm2pfdd. The meeting begins at 9:30 a.m. Eastern on February 15, 2024, and is scheduled to end at 3 p.m. Eastern.

Nearly 30 individuals, either adults or parents of children affected by infantile, juvenile, and late onset Tay-Sachs and Sandhoff diseases will share their stories and experiences. Patients, parents, caregivers and bereaved family members will share their perspectives and participate.

"Sharing my daughter's story and continuing to be her voice to advance research in the hopes of a treatment and cure for GM2, even now, twelve years after her death, is imperative so that no other family has to know this searing pain of loss, said Becky Benson."

Other presenters include Jacqueline Karp, MD, Clinical Team Lead in the Division of Rare Diseases and Medical Genetics, U.S. FDA; Florian Eichler, MD, Massachusetts General Hospital; Cynthia Tifft, MD, PhD, National Institutes of Health; and Kathleen M. Flynn, NTSAD Chief Executive Officer. There also will be call in opportunities and live polling of all meeting participants. In addition, people can submit comments in advance, during, and 30 days following the meeting.

"NTSAD was founded by families. They are the experts on living with these devastating rare, genetic diseases, and their voices should be heard, said NTSAD Chief Executive Officer Kathleen M. Flynn. "It is an honor to provide families with this unique opportunity to share their stories and perspectives to accelerate the development of treatments and make a difference, if not for themselves, but so other families will not have to experience grief and enduring heartache."

Sponsors of the GM2 EL-PFDD meeting include Sanofi, Cure Tay-Sachs Foundation, New York Area Fund at NTSAD, Mathew Forbes Romer Foundation, Azafaros, and JCR Pharmaceuticals.

To learn more about the GM2 EL-PFDD meeting and to submit comments visit www.ntsad or watch an informational webinar at https://www.youtube.com/watch?v=tuyd26kn9wU&feature=youtu.be. There will be a Voice of the Patient Summary Report made available this summer.

About NTSAD

NTSAD is among the first and most respected patient advocacy groups and was a pioneer in advancing carrier screening to prevent rare genetic diseases. The organization supports families and individuals around the world through one-on-one professional support, mentoring and peer groups, information and resources, and its Annual Family Conference. As part of its mission, NTSAD also advances research. Since 2002, the organization has awarded more than $4.7 million in grants that have been leveraged to more than $30 million of investments in research. These initiatives have led to the development of potential therapies for Tay-Sachs, Canavan, GM1, and Sandhoff diseases. For more information, please visit NTSAD.org.

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