



# GM2 Externally-Led Patient-Focused Drug Development Meeting

February 15, 2024 — 9:30am – 3:00pm ET

[www.ntsad.org/gm2pfdd](http://www.ntsad.org/gm2pfdd)

## AGENDA

- 9:30-9:35am**      **Welcome Remarks**  
Kathleen Flynn, CEO  
National Tay-Sachs & Allied Diseases Association
- 9:35-9:45am**      **FDA Opening Remarks**  
Jacqueline Karp, MD  
Clinical Team Lead in the Division of Rare Diseases and Medical  
Genetics  
U.S. Food and Drug Administration
- 9:45-10:00am**      **Meeting Overview and Demographic Polling**  
James Valentine, JD, MHS  
Hyman, Phelps, & McNamara, PC

### *Living with Infantile/Juvenile GM2 (Tay-Sachs and Sandhoff Diseases): Symptoms and Impacts on Daily Life*

- 10:00-10:10am**      **Clinical Overview, Infantile/Juvenile GM2**  
Florian Eichler, MD  
Director, Center for Rare Neurological Diseases  
Massachusetts General Hospital  
Boston, Massachusetts
- 10:10-10:30am**      **Caregiver Panel**  
Jennifer, Crystal, Kate, Kelly
- 10:30-11:20am**      **Audience Polling and Moderated Discussion with Live  
Panelists (TBD)**  
James Valentine and Kathleen Flynn

AGENDA, Continued

***Living with Late Onset GM2 (Tay-Sachs and Sandhoff Diseases):  
Symptoms and Impacts on Daily Life***

- 11:20-11:30am**      **Clinical Overview, Late Onset GM2**  
Cynthia Tifft, MD, PhD  
Deputy Clinical Director, National Human Genome  
Research Institute  
National Institutes of Health  
Bethesda, Maryland
- 11:30-11:45am**      **Patient and Caregiver Panel**  
Rachel, Michael, Sophia
- 11:45am-12:30pm**      **Audience Polling and Moderated Discussion with Live  
Panelists (TBD)**  
James Valentine and Kathleen Flynn
- 12:30-1:00pm**      **Lunch Break**
- Current and Future Treatments for GM2***
- 1:00-1:25pm**      **Patient and Caregiver Testimonials**  
Mandy, Carla, Ken, Allie, Reita
- 1:25-2:40pm**      **Audience Polling and Moderated Discussion with Live  
Panelists (TBD)**  
James Valentine and Kathleen Flynn
- 2:40-2:55pm**      **Meeting Summary**  
Larry Bauer, RN, MA  
Hyman, Phelps, & McNamara, PC
- 2:55-3:00pm**      **Closing Remarks**  
Kathleen Flynn

*Note: Although very effort will be made to stay on schedule, the times above may vary slightly.*