

## Research & Awareness

- Many GM1 caregivers participated in the study, “Humanistic burden of neurodegenerative lysosomal disorders in the US: insights from caregivers of patients living with GM1 and GM2 gangliosidoses”, which highlighted caregiver and patient burdens and will help in future research.
- The Voice of the Patient report from the Externally-Led Patient-Focused Drug Development Meeting (EL-PFDD) was published on the FDA website, shedding light on patient experiences and most important symptoms to treat.
- TEGA received a Small Business Innovation Research (SBIR) grant based on seed funding provided by Cure GM1 to continue their Enzyme Replacement Therapy study.



## Initiatives and Research Support

- Cure GM1 collected blood and urine samples to support newborn screening research at the Gelb Laboratory at the University of Washington. These samples will be used to further refine newborn screening research and assays for GM1.
- Cure GM1 joined CombinedBrain and initialized a patient-led GM1 bio-repository to collect and store samples from GM1 patients to be used in research.
- Cure GM1 and xCures initiated a collaboration to start a natural history study pilot.



## Advocacy

A huge number of people in our community showed up in person and online to support us in our community-building efforts this year, including:

- Rare Disease Day
- GM1 Day (for which families got proclamations from 11 more states!)
- Move for GM1, during which hundreds of people logged miles for Cure GM1
- The 2023 International GM1 Community Conference, our first in-person & hybrid event since the pandemic

Christine Waggoner, Founder and President of Cure GM1 and mother of Iris, also won the 2023 WORLD Patient Advocacy Leadership award!



## Notable Fundraisers

We give our heartfelt thanks to everyone who put in the work to host fundraising events for Cure GM1 this year, which generated critical funds for continued progress. We are so grateful for your efforts.

- Face off for Marley Charity Hockey Game & Marley's Miracle Mile
- Cure Kinley & Kennedy Annual Golf Tournament
- Mount Snowden Hike
- Dorney Lake Run
- A Cure for Violet
- Giving Tuesday, where we raised over \$40k for GM1

## The Future

We have multiple projects underway behind the scenes, which we are excited to announce in the near future when they are ready. We can't say much yet, but they are giving us a lot of hope right now.

Our continued progress depends on you. Thank you for your continued support.

