

# 2024 CURE GM1 IMPACT REPORT



Cure GM1 remains deeply committed to our mission and fight for life. We are hopeful for 2025 with the announcement of GEMMABio in 2024, the upcoming Phase 3 Azafaros small molecule clinical trial, and the ongoing intravenous AAV9 trial at the National Institutes of Health.

## Research & Awareness

- Cure GM1 is investigating the development of new research programs. Our current focus is the investigation of an enzyme replacement therapy (ERT). While there have been gene therapy and small molecule trials, there have been no trials for ERT.
- Cure GM1 has been hard at work applying for grants to work towards diversifying our sources of funding.
- Cure GM1 will present two posters and abstracts at the WORLD Symposium in 2025. The subjects of the posters are real-world data on GM1 and data sharing to support natural history and to deepen the understanding of GM1.



## Advocacy, Events, and Networking

Our community showed up in person and online to support greater awareness and to drive meaningful change.

- Cure GM1 attended the WORLD Symposium to meet with biotech companies and researchers and will attend this year.
- GM1 Day was a success with families who obtained proclamations and spread awareness.
- This year, for Move for GM1, families around the world logged miles and spread awareness.
- Our International GM1 Community Conference informed and educated.
- Christine Waggoner spoke at the Federal Rare Disease Congressional Caucus and the FDA's Rare Disease Innovation Hub kickoff.



## The Future

Cure GM1 is constantly assessing the landscape and there are more potential projects than funds available. Our work and advocacy is critical to continuing to push for possible treatments and to build more progress.

**Will you join us in our fight? Your ongoing support is critical.**



**xCures**

## Initiatives and Drug Development

- Cure GM1 is investigating the necessary steps for data generated from the Lysogene video natural history study to be utilized in new research projects.
- Cure GM1 continues its membership of CombinedBrain to support our biorepository.
- Cure GM1 is working hard to complete the real-world evidence xCures natural history study pilot. We hope that the results of the pilot will lead to the expansion of the study.
- Cure GM1 maintains contact with biotechs and key opinion leaders around the world to keep abreast of possible new projects and the overall landscape.



## 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.

- Iris's Sweet 16
- Cure Kinley & Kennedy Annual Golf Tournament
- Move for GM1
- Marley's Miracle Mile
- Giving Tuesday



Your Support  
is Critical  
[curegm1.org/donate](https://curegm1.org/donate)

