THE CURE GM1 FOUNDATION

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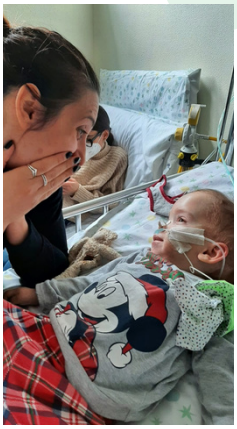
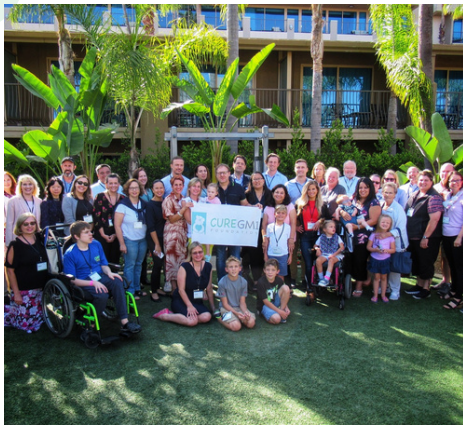
ABOUT CURE GM1

Cure GM1 is a 501(c)(3) nonprofit dedicated to GM1 gangliosidosis research, drug development, awareness, and advocacy. Our core mission is to support the development of effective, FDA-approved treatments and therapies that will improve the quality of life of and prognosis for those living with GM1. Cure GM1 has the largest number of contacts with GM1 of any organization worldwide and international reach with attendees of our conference from 20 countries.





WHAT IS GM1 GANGLIOSIDOSIS?

GM1 Gangliosidosis is a rare inherited disease that primarily affects children. It has many debilitating ramifications, some of which are developmental regression, seizures, visual impairment, and neurodegeneration. The thousands of diagnosed children and adults who suffer from GM1 Gangliosidosis face the progressive loss of skills and abilities until death. This is a fatal disease that currently has no cure or effective treatment.

OUR FOCUS AND WORK

Our primary focus is to catalyze progress towards treatment through seed funding and advocacy. To do this effectively, networking and collaboration are key. Such work may include conversations with the FDA to help impact change to support approval of treatments, as well as engagement and connection with key stakeholders in the GM1 landscape to work collaboratively at problems we are all facing. Broader awareness and community also build momentum. Our two annual signature events are GM1 Awareness Day and the Cure GM1 Conference.

ACCOMPLISHMENTS AND HIGHLIGHTS:

- Cure GM1 has contributed over $1.5M to various research projects in the following areas: enzyme replacement therapy, gene therapy, animal models, newborn screening assays, and drug repurposing.
- Since Cure GM1 was founded there have been four clinical trials as opposed to none in all the years prior to Cure GM1's existence.
- Cure GM1 has contributed to multiple peer-reviewed publications and published the first-ever caregiver preferences study.
- The GM1 Externally-Led Patient-Focused Drug Development Meeting with the FDA was held in October 2022.
- Community conferences that support connection, learning, and awareness are held each year.
- GM1 Awareness Day is observed every year on May 23rd, and helps us build momentum for our cause.

CRITICAL MILESTONES AHEAD:

- Continued collaboration with biotechs and researchers regarding preclinical research, as well as current and upcoming trials.
- Multi-stakeholder initiatives to expand collaboration on GM1 research and drug development.
- Extension of newborn screening assay development in collaboration with University of Washington.
- And much more! The potential work outstrips the current funding. Therefore, support is always needed.

Cure GM1 exists to build community and hope for all those impacted by GM1 gangliosidosis. We work each day so that someday, those impacted will no longer experience debilitating cognitive and physical regression; so that parents of GM1 children don't have to explain to their child why they can no longer walk, talk, or eat; so that families of those with GM1 won't have to shoulder the immense financial and caregiving burden the disease demands; and so that families of those with GM1 won't have to anticipate their loved one's eventual death.

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