CUREGM1 FOUNDATION

IMPACT REPORT

Driving Progress in 2022 and Beyond

First Ever Publication of its Kind: GM1 Caregiver Preferences Study

The voices of both the patient and caregiver are essential for drug development. Impacted families are the true experts of GM1 gangliosidosis. This study characterizes what caregivers consider to be the most critical symptoms to treat for GM1. This study makes plain the excruciating daily experiences and relentless onslaught of symptoms. The caregiver stories underscore the most important symptoms to treat–among them pain, quality of life, and regression.

"For me, taking care of my child was not a bad thing, losing them was the bad thing. And so I think for me that was my determining factor. Like, how can I get more time? Even if I'm having to take care of him and he really can't do much, which treatments are gonna give me more time?" - GM1 Parent

The first ever International GM1 Gangliosidosis Awareness Day

GM1 Day was a hugely successful and impactful day of recognition for those living with GM1. Iowa, Michigan, Arizona, and Colorado issued proclamations making the day an official day of observance. The mighty GM1 community showed up to share their stories and advocate in press, events, and on social media. Many many people in our community posted online holding up the number one and spreading awareness and support. Join in this year to continue the momentum!

International Virtual Community GM1 Conference

The latest research was presented with progress towards the development of treatments. Talks were also given on support for GM1 families and the relationship and daily life dynamics involved in caring for someone with GM1 gangliosidosis. This was a well-attended event that inspired hope in our community for what may come in the future.

The Externally-Led Patient Focused Drug Development Meeting with the FDA

This critical and touching meeting officially recorded statements from family and caregivers firsthand. It will be referenced by researchers, policy makers, advocates, and families for years to come, and will be influential in the creation of treatments and therapies for GM1 gangliosidosis. This was a critical foundational milestone needed to help inform the FDA and the world of the true impact of GM1. The GM1 Voice of the Patient Report, a comprehensive summary of the meeting, is available on the Cure GM1 and FDA websites.

"These kids are so resilient and teach us so much every day to be strong, to be happy in spite of all their trials and pain." - Ruth

Daily Engagement, Assistance, Correspondence, and Support

Correspondence with dozens of newly diagnosed families, researchers, and clinicians globally to educate and answer questions about GM1, offer support, and connect families to each other. We continue to disseminate the latest information on clinical trials and preclinical research.

Notable Activities in 2022

- Work continued on the TEGA Therapeutics project to research Enzyme Replacement Therapy. ERT is a known treatment modality that has been largely neglected for GM1. This research demonstrates the viability of this approach, even though venture funding is still needed to progress the project further. TEGA presented at the WORLD Symposium in February 2023, bringing more visibility to GM1 and the possible treatment approach.
- Formation of a collaborative working group with biotechs and key opinion leaders to identify and advance pre-competitive areas of collaboration such as natural history data sharing. This group meets monthly and is working to formalize and navigate the complexities of aggregation of natural history data.
- Notable fundraisers: Dean's Fundraiser for Cure GM1, A Cure for Violet, Cure Kinley and Kennedy, and A Cure for Marley. Thank you!

Upcoming Activities in 2023

- Formalization of multi-stakeholder natural history data sharing collaborative
- 2nd International GM1 Gangliosidosis Awareness Day on 5/23
- Fall In-person conference for the first time since the pandemic
- Extension of newborn screening assay development
- Continued collaboration with biotechs and researchers regarding preclinical research, current and upcoming trials
- And more! New projects and requests arise regularly and there is still much work to do.

"If I had to pick, I wish she could find peace and contentment, I wish she no longer had a pained look on her face much of the day. I want her to be happy. She deserves such a better life." - Douglas

You are Key to Building a Hopeful Future

The vast majority of rare diseases do not have a disease-specific patient advocacy group. By supporting our work at Cure GM1, you can greatly increase the visibility and likelihood that biotech companies and researchers will choose to work on GM1. Without a community and without advocacy, GM1 would be lost amongst the now 10,000+ known rare diseases.

The actions we take today will determine the outcomes for everyone with GM1. We can choose to do nothing and continue to watch our loved ones suffer from this wretched disease, or we can choose to act. We can advocate, participate in studies, share our stories, and most importantly - raise money and donate. The stark truth is there is no progress without funding.

Your continued support is vital to ensuring these endeavors continue to yield progress and hope. Would you consider making a donation today to help make 2023 as successful as 2022?