



Annual Report

Mission of CureLGMD2i

Providing Advocacy, Spreading Awareness, Supporting Scientific Research and Drug Development with the Goal of Finding a Cure for Limb Girdle Muscular Dystrophy 2I/R9.

Vision of CureLGMD2i

Our vision is to pioneer a future where every individual affected by Limb Girdle Muscular Dystrophy Type 2I/R9 can be treated early and live a life unencumbered by its challenges. By relentlessly pursuing innovative research, collaborative partnerships, and drug development, we envision accessible and effective treatments that slow or prevent the progression of the disease.

A Message from our Co-Founder & CEO, Kelly Brazzo



As I reflect on the progress we have made in 2024, it brings me such joy and hope for the future of our LGMD2I/R9 community. The past year has allowed us to embark on a remarkable path of advocacy, focused on accelerating the development and approval of treatments for LGMD2I/R9. We have also continued to build awareness by storytelling through documentaries and awareness campaigns and by expanding our relationships with other patient advocacy groups, industry sponsors and regulatory authorities. As we head into 2025, I am confident that we will see continued progress. Thank you for your prayers and support of our efforts. With our team of dedicated volunteers and supporters, the future for our CureLGMD2i family is bright!

Kelly Brazzo

Our Executive Team



Kristen, Dan, Kelly, John and Kaitlyn (shown above) have been working tirelessly to achieve the stated goals in CureLGMD2i's strategic plan. Read on to see all that we have accomplished throughout the past year! We are committed to continue working together to create a future that includes an approved treatment available for ALL who are affected by Limb Girdle Muscular Dystrophy, 2I/R9



Being on the executive board is my opportunity to connect with the community, create positive change, and promote advocacy to accelerate a cure for those like me who dream of a healthy future.

Kaitlyn Neroladakis
Executive Board Member &
Communications Director



**2024 Dystroglycanopathy Patient & Family Conference
University of Iowa**



Advocacy

- Rare Disease Day on the Hill
- White House Rare Disease Forum
- MDA Neuromuscular Advocacy Collaborative
- LGMD Scientific Workshop
- Rare Across America
- Proposal for an ICD-10 Code specific to LGMD21/R9
- LGMD Day on the Hill
- PA State Advocacy Day
- EveryLife Foundation Community Congress
- Presentation to FDA on Early Enrollment into Gene Therapy Clinical Trials for Rare Diseases



Advocacy, Con't

mytomorrows CURELGMD2i The SPEAK Foundation

Register for the Panel Discussion

Unlocking *LGMD2I* Clinical Trials: A Patient and Caregiver's Guide to Participating

 Moderator				
Dennis Akkaya COO at myTomorrows	Kat Bryant Knudson Speak Foundation	Kelly Brazzo CEO at CureLGMD2i	Melissa Grove LGMD2i Patient Advocate	Terri Ellsworth myTomorrows Patient Liaison

- Educating our patient community by hosting a webinar on clinical trials with My tomorrows
- Actively engaging with our industry partners to share the voice of the LGMD2I/R9 patient community
- Treat-NMD Developing Treatments in Rare Diseases Presentation
- Forging and elevating partnerships with other rare disease organizations to reach common goals

Strategic Partnerships



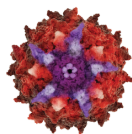


Scan here to
learn about
clinical trials
for
LGMD2I/R9:

Advocacy, Con't

Engaging with Industry Sponsors

We are thrilled to have three clinical trials ongoing for LGMD2I/R9, which have all been granted fast track designation! CureLGMD2i supports the efforts of these programs by serving on advisory committees and patient focus groups to effectively share the voice of the patient community.



AskBio



"ML Bio Solutions is privileged to partner and collaborate with CureLGMD2i Foundation. By amplifying the voice of the LGMD community, we ensure that drug development is not only scientifically sound but also deeply rooted in the lived experiences of those who need these treatments most. CureLGMD2i is an unwavering champion of the community, and we are honored to work together to driving progress and hope in the pursuit of better health."

Cybele Governour, Associate Director, Global Patient Advocacy

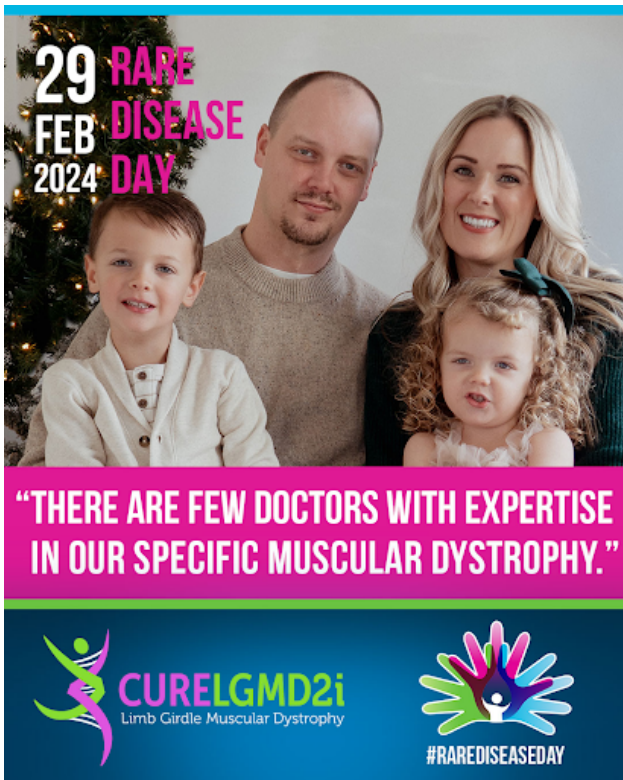
MLBio Solutions



Awareness Activities



- Gold Sponsor for 10th Annual LGMD Awareness Day & the Decade of Difference
- Rare Disease Day Ambassador Campaign
- Rare Disease Day Documentary, featuring Ryan, Jennifer, Jaxson & Chloe
- Supporting the podcast, “Unbalanced” Featuring Cerys Davage, living with LGMD21/R9
- Connecting for a Cure Documentary, “Strong Together” featuring Cerys, Cadi & Beca
- Serving on the Global FKRP Registry Steering Committee
- Contributing to the LGMD News Magazine



“The advocacy, friendship, and hope that the CureLGMD2i Foundation brings have helped me cope with so many worries about my kids' health in the future. Their hard work to find a treatment, along with the amazing relationships I've built through them, has taken away a lot of the darkness and filled those spaces with love and hope.”

Jennifer Zuchetto,

Mom of Jaxson & Chloe, two siblings living with LGMD21/R9

Advancing Science



Conference Sponsorships

- International LGMD Conference
- Iowa Wellstone Dystroglycanopathy Conference
- Inaugural European LGMD2I/R9 Conference

Industry Investments

- Ventura Life Sciences
- Atamyo Therapeutics

Research Grants

- Dr. Isabel Richard, Genethon - Circulating micro-RNAs as biomarkers for LGMDR9 and a Study of the regulation of expression of FKRP in cell culture
- Dr. Ana Topf, Newcastle University - Next generation sequencing project in Latin America
- Dr. Peter Currie, Monash University - Examining the clinical significance of FKRP's regulation of Fibronectin Sialylation
- Dr. Paul Martin, Nationwide Childrens - A novel gene therapy program for LGMD2I/R9
- Drs. Emerson & Wolf, UMass - FKRP gene correction via prime editing (CRISPR)

Patient Registry

- Newcastle University - Global FKRP Registry Support



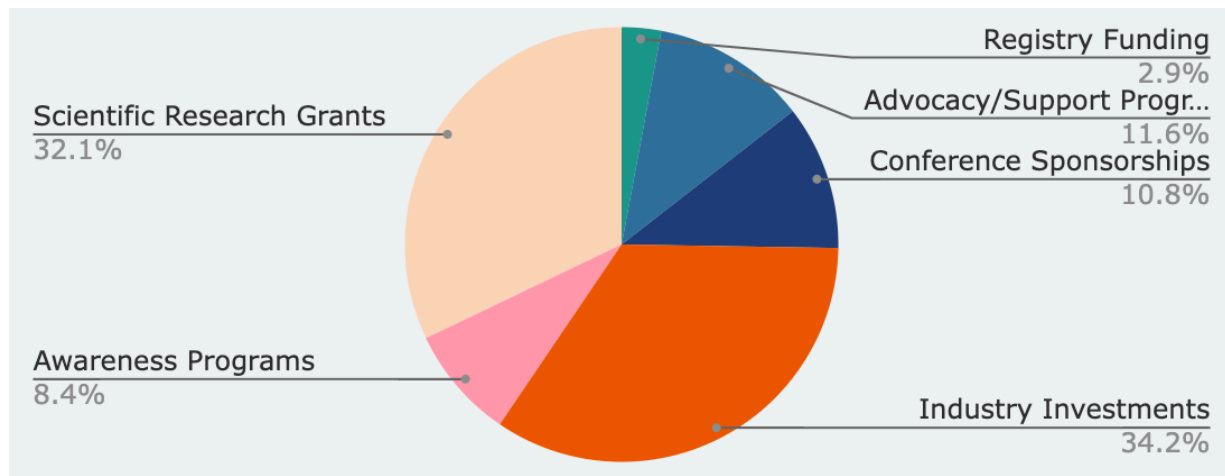
Grants & Registry Co-Funded in Partnership with:



CureLGMD2i

Since 2011, CureLGMD2i has committed to **over \$1.3 million** in funding programs for LGMD21/R9

CureLGMD2i provided over **\$355,000** in funding programs this year!



Help us to Continue Making an Impact

\$50

Monthly internet fees to connect with 2i community

\$500

Travel Stipend for LGMD2i Conference

\$5,000

Creation of LGMD21/R9 Awareness Campaign

\$10,000

International FKRP Registry Grant

\$25,000

LGMD21/R9 Scientific Research Grant

\$100,000

Investment in new biotech startup

\$200,000

One patient dosed with gene therapy + 5 year follow up

\$400,000

Opens a new Clinical Trial Site



Thank you!!

Your support matters!
To make a tax deductible donation,
please visit:

www.curelgmd2i.org

