IMPACT DECK

CureLGMD2i Foundation

AGENDA

- ★ Our Vision
- ★ What is LGMD2I/R9?
- ★ Mission Statement
- ★ About Us
  - ○ Our Story
  - ○ Our Team
  - ○ Our Core Values
- ★ Our 3 Pillars
  - ○ Advocacy
  - ○ Awareness
  - ○ Advancing Science
- ★ Impact to Date
- ★ Funding Opportunities
- ★ Ways to Get Involved

CURELGMD2i
Limb Girdle Muscular Dystrophy

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.**

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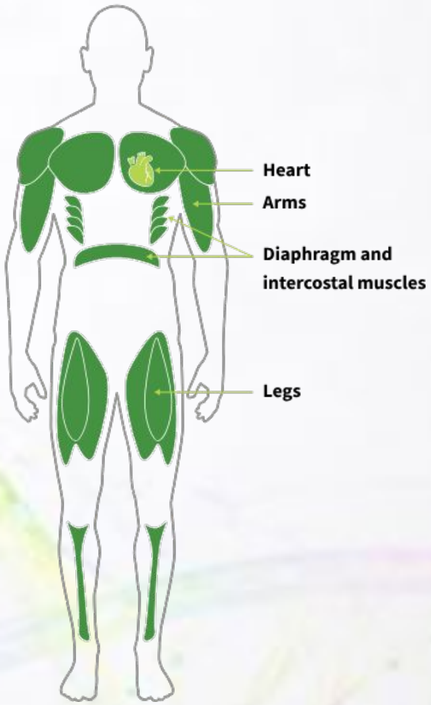
WHAT IS LIMB GIRDLE MUSCULAR DYSTROPHY TYPE 2I/R9 (LGMD2I/R9)?

- LGMD2I/R9 is a rare, recessive genetic disorder caused by mutations in the FKRP (Fukutin-Related Protein) gene

  The FKRP gene encodes an enzyme that is essential for glycosylation processes that stabilize muscle fibers and facilitate proper cell function

- In people with LGMD2I/R9, FKRP does not function at full capacity, causing instability and injury of muscle cells. ***This leads to progressively debilitating muscle damage.***
- Muscle wasting and weakness occurs mainly in the proximal limbs and shoulder and hip areas. At later stages, cardiovascular and respiratory challenges occur, and functional independence deteriorates.
- **There is no known cure or FDA-approved treatments for LGMD2I/R9**

Muscles affected by LGMD2I/R9



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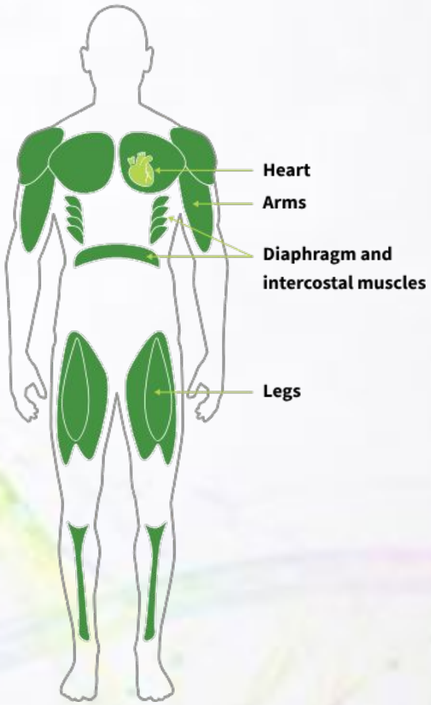
WHAT IS LIMB GIRDLE MUSCULAR DYSTROPHY TYPE 2I/R9 (LGMD2I/R9)?

- LGMD2I/R9 is recognized as an orphan disease
  - *According to the NIH, fewer than 5,000 people in the U.S. are affected*
- Patients and families face many challenges:
  - Because this is such a rare disease, those living with LGMD2I/R9 often feel isolated and alone
  - Most drug companies lack financial incentive to develop treatments for small patient populations
  - Doctors often have limited knowledge, since they see so few cases
  - Diagnosis delay, difficulty finding a medical expert and lack of access to treatment or ancillary services commonly occur
  - Patients and families affected by LGMD2I/R9 bear the burden of raising public awareness to support research for effective treatments and a cure

Muscles affected by LGMD2I/R9



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MISSION

Providing Advocacy, Spreading Awareness & Supporting Scientific Research and Drug Development to Find a Cure for Limb Girdle Muscular Dystrophy 2I/R9

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Limb Girdle Muscular Dystrophy

OUR STORY

- The CureLGMD2i Foundation was created in 2011 by the Brazzo Family when their daughter, Samantha, was diagnosed with Limb Girdle Muscular Dystrophy Type 2I/R9 at the age of two.
- They created this 501(c)3 Nonprofit Organization (formerly known as The Samantha J. Brazzo Foundation) with a mission to make a difference for those living with LGMD2I/R9.

The Brazzo Family
(Marina, Kelly, Sammy, Brandon & Keith)

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OUR TEAM

The Executive Board
(Kristen, Dan, Kelly, John & Kaitlyn)

Kristen, Dan, John, Kaitlyn & Kelly's daughter, Sammy, live with LGMD2I/R9

Kelly Brazzo
Co-Founder, President/CEO

Dan Pope
Vice President/Advocacy Director

Kristen Olsen
Secretary/Treasurer

Kaitlyn Neroladakis
Officer/Communications Director

John Spencer
Officer/Creative Director

Lacey Woods
Community Engagement Coordinator

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OUR CORE VALUES

PATIENT-FOCUSED

We regularly engage with and listen to our patient and caregiver community to represent the voice of the patients as we connect with industry partners and regulatory bodies

MOTIVATION

We are a driven team of LGMD2I/R9 patients and caregivers, committed to responsibly utilizing funds to support cutting edge research, patient conferences, scientific meetings, and advocacy programs focused on accelerated drug development for LGMD2I/R9

COLLABORATION

We work closely with drug development stakeholders, regulators and other advocacy organizations to expedite the approval process for a future treatment for LGMD2I/R9

ENTHUSIASM

We are passionate, creative, and courageous in expressing and responding to the unmet needs of our patient community, and determined to continue our mission in facilitating the approval of an accessible and effective treatment for those affected LGMD2I/R9

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OUR 3 PILLARS

Advocacy

Representing the patient voice to partners and regulatory bodies.

Awareness

Spreading knowledge and fostering community engagement globally.

Advancing Science

Supporting cutting-edge research and accelerated drug development.

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ADVOCACY

- Events:
  - Rare Disease Day on the Hill
  - MDA Neuromuscular Advocacy Collaborative
  - Rare Across America
  - LGMD Day on the Hill
  - EveryLife Foundation Community Congress
- Presentations:
  - Facilitating the Development and Review of Cell and Gene Therapies to FDA CBER
  - ASGCT Empowering Patients 2025: A Cell and Gene Therapies Summit
  - Rare Disease Congressional Caucus
  - Advocacy Power Hour Webinar

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ADVOCACY

- Leader of Patient Focus Groups
- Obtained our ICD-10 Code (G71.036)
- Member of Advocacy Coalitions:
  - Save Rare Treatments Task Force
  - Genomic Answers for Children's Health Alliance
  - Pediatric Inclusion Alliance
  - EveryLife Foundation Community Congress
  - Member of Critical Path Institute LGMD Task Force

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AWARENESS

- Host Annual Connecting for a Cure Fundraising Events
- Launched Campaigns & Documentaries:
  - Rare Disease Day Ambassador Campaign
  - “Growing up with LGMD” Documentary
  - 15th Anniversary of CureLGMD2i
- Conference Participation:
  - MDA Conference
  - Iowa Wellstone Dystroglycanopathy Conference
  - International LGMD Conference
  - 2nd European LGMD2I/R9 Conference

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ADVANCING SCIENCE

- Sponsorships:
  - International LGMD Conference
  - Iowa Wellstone Dystroglycanopathy Conference
  - European LGMD2I/R9 Conference
- Industry Investments:
  - Cure Rare Disease for the development of Myo-AAV for LGMD2I/R9
- Patient Registry:
  - Global FKRP Registry – Newcastle University

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ADVANCING SCIENCE

- We are always looking to build our portfolio with new grants and relationships with researchers. Grant seekers can apply for funding via our website: curelgmd2i.org

- Research Grants Funded by CureLGMD2i
  - Dr. Emma Rybalka, Victoria University – Preclinical screening of adenylosuccinic acid (ASA-001) in a mouse model of LGMDR9
  - MDA – Developing a Core Muscular Dystrophy Clinical Trial Research Network
  - Dr. Peter Currie, Monash University – Examining the clinical significance of FKRP's regulation of Fibronectin Sialyation
  - Drs. Emerson & Wolf, UMass – FKRP gene correction via prime editing (CRISPR)
  - Dr. Isabel Richard, Genethon – Circulating micro-RNAs as biomarkers for LGMDR9
  - Dr. Paul Martin, Nationwide Children's, – Novel gene therapy

    *Many of these programs co-funded with our partners: LGMD2i Fund

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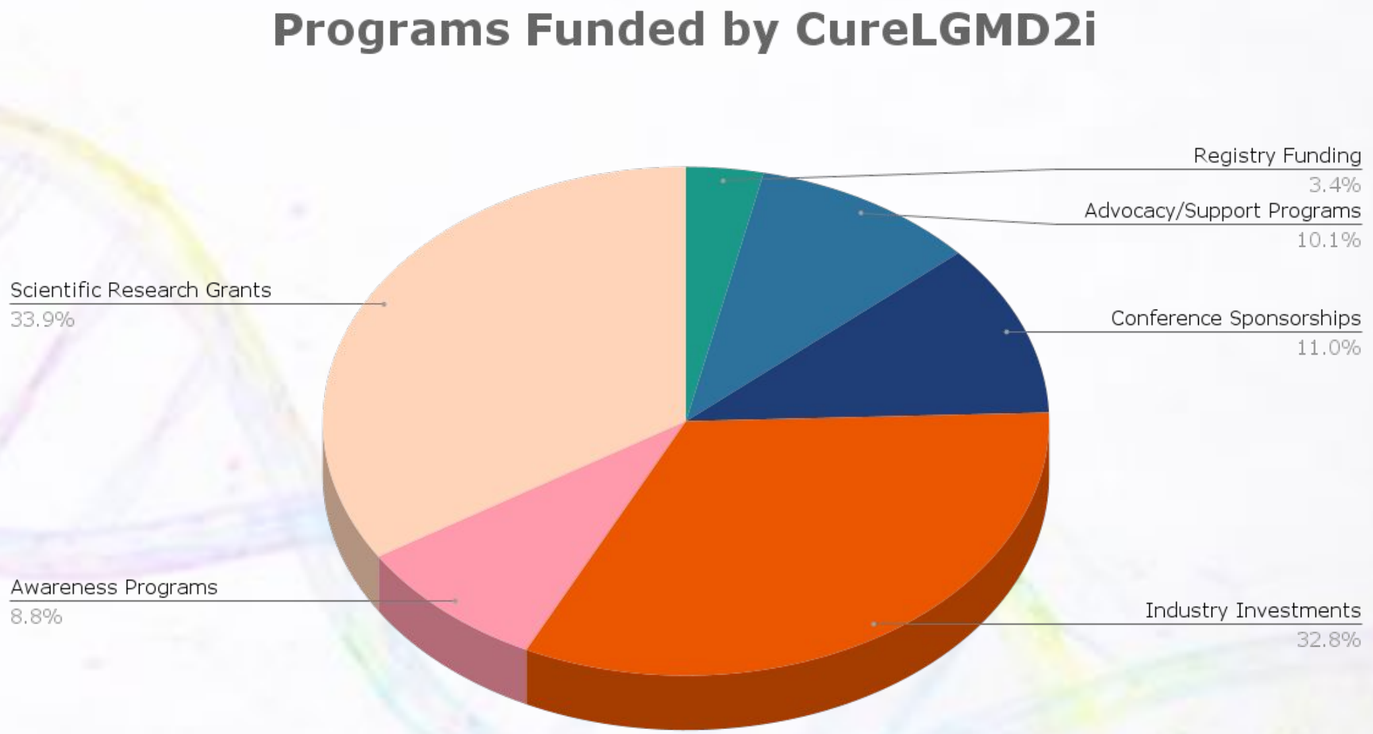
IMPACT TO DATE

15
years in operation

>$1,500,000
committed

21
scientific grants funded



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Turn hope into progress.

Join us in accelerating research and bringing hope to families.

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“Each day is a reminder of the urgent need for research, investment, and hope that one day there will be treatment that makes a difference. CureLGMD2i gives me the opportunity to connect with the community, create positive change, and advocate for a cure for those like me who dream of a healthier future.”

Kaitlyn Neroladakis

Executive Board Member & Communications Director, living with LGMD2I/R9

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“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 LGMD2I/R9

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"The CureLGMD2i Foundation is shining light and bringing hope into the lives of patients and families living with this condition, and gives meaning and purpose to me knowing that the work we are doing today is building a brighter tomorrow for others."

Dan Pope

Executive Board Member & Vice President, living with LGMD2I/R9

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“For me, the CureLGMD2i Foundation has turned my fear into action and isolation into connection. Because of this foundation, our community has hope, a voice, and a future we’re actively building together."

Lacey Woods
Lacey
Community Engagement Coordinator, living with LGMD2I/R9

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HOW YOU CAN MAKE AN IMPACT

- CureLGMD2i relies on the support of generous donors like you
- Please consider making a tax-deductible donation

  Tax ID: 80-0680447
- For ongoing updates, please follow us @curelgmd2i on Facebook, Instagram and LinkedIn, or visit our website: curelgmd2i.org

$500
Covers 1 Travel Stipend for LGMD2i Conference

$1,000
Office and overhead expenses for one month

$5,000
Creation of an LGMD2I/R9 Awareness Campaign

$10,000
One year International FKRP Registry Grant

$25,000
Provides a new LGMD2I/R9 Scientific Research Grant

$100,000
Investment in New Biotech Startup

$200,000
Covers the Cost of Dosing 1 Patient with Gene Therapy + 5 Year Follow Up

Learn More / Donate

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Thank you!

CureLGMD2i Foundation

curelgmd2i.org

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FUNDING PROGRESS & FOSTERING COMMUNITY
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