



# Annual Report 2025



# Table of Contents

- About LGMD2D Foundation
- Impact at a Glance
- Programs & Initiatives
- Research & Clinical Progress
- Advocacy & Awareness
- Partnerships & Collaborations
- Financial Overview
- Gratitude & Donor Recognition
- 2026 Goals & Objectives
- Contact Information

# About the LGMD2D Foundation



## Committed to the awareness, advocacy, and research of LGMD 2D / R3.

Formed in September 2013, the LGMD2D Foundation is a registered 501(c) (3) non-profit organization established by families living with LGMD2D – both patients and caregivers – to support one another.

## Our mission is to expedite the development of treatments or a cure for Limb-Girdle Muscular Dystrophy, type 2D / R3 (LGMD2D).

In addition to educating patients, researchers, and physicians, the foundation:

- Maintains the only international patient registry for LGMD2D.
- Funds research and monitors progress of clinical trial development.
- Provides financial support to accelerate clinical trials.
- Encourages scientific collaboration and partnerships.
- Participates in LGMD community events

## Our Team

- Bryan Barber, Founder & CEO
- Caroline Barber, Founder & Treasurer
- Rachel DeConti, Executive Director & Board Member
- Suji Vasu, MD, Chief Scientific Advisor & Board Member
- Maxwell Barber, Director, Patient Advocacy
- Reese Barber, Director, Patient Advocacy
- Faran Day, Director, Fundraising & Patient Advocacy
- Michelle Clayton, Director, Fundraising & Patient Advocacy
- Stacey Antonino, Director, Fundraising & Patient Advocacy
- Stefanie Stanek, MS LMFT, Director, Fundraising & Patient Engagement

## Scientific Advisory Board

- Doug Albrecht, PhD
- Christopher Carroll, Patient
- Anne M. Connolly, MD
- Nicholas Johnson, MD, MSCI, FAAN
- Kemi Mascoll-Robertson, MD, FAAP



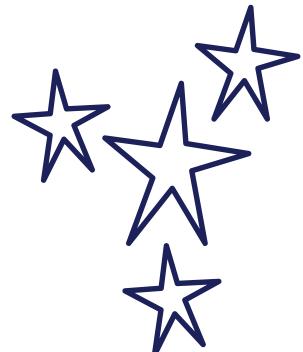
# Impact at a Glance



1

## By the Numbers

- Patients Registry Growth: **50+ new patients added**
- Social Media Reach: **53k+**
- Research Dollars Invested: **\$2k**
- Clinical Programs Supported: **6 (existing and new)**
- Total Events Hosted: **4 with 250+ total attendees**
- Awareness & Advocacy Touchpoints: **5**



2

## Highlights

- 4 new LGMD2D Foundation team members added (volunteer)
- *Friend of LGMD Sponsor* at the International LGMD Conference
- Second annual *Hope in Motion* LGMD2D Virtual 5k
- First *Fore the Cure* LGMD2D Golf Tournament
- LGMD2D awareness video released



# Programs & Initiatives



## Conferences

### MDA (2,500+ Attendees)

- Neuromuscular Advocacy Collaborative (NMAC) meeting participation
- Booth at the Patient Advocacy Pavilion
- Co-Host of LGMD Networking Event

### International LGMD (500+ Attendees)

- Conference Leadership team member
- Booth onsite
- Sarco Meet-Up Networking Event



## Events

### Hope in Motion

- 130+ attendees (22% increase from 2024)
- Over \$21k raised (174% increase from 2024)
- 3 sponsors
- 11 different states

### Fore the Cure

- 130+ attendees (60+ golfers)
- Over \$32k raised
- 26 sponsors



## LGMD Day on the Hill

This advocacy event, held in Washington, D.C., brings together patients and families living with LGMD to meet directly with their state legislative offices.

In 2025, several LGMD2D families participated, representing multiple states across the United States and amplifying the voices of our community at the national level.



# Research & Clinical Progress

1

## Natural History Studies

Following **Sarepta Therapeutics'** pause of its LGMD programs in July 2025, the multi-year Journey natural history study was discontinued. The Foundation is actively advocating to ensure that the valuable data already collected is preserved and shared to support future therapeutic development.

At the same time, research momentum continues. **GRASP-LGMD** maintains an open natural history study for LGMD2D, and the **University of Florida** has launched an MRI-focused study for LGMD2D and LGMD2C. The Foundation remains a committed partner in patient recruitment, helping ensure these critical studies move forward.

2

## Gene Therapy

In 2025, the LGMD2D community faced a profound setback with the pause of **Sarepta Therapeutics'** LGMD2D clinical trial (SRP-9004). During this challenging time, the Foundation remained steadfast—advocating for patient access to Journey study data, monitoring the FDA program hold, and engaging in discussions to support the continuation of this important work.

At the same time, we strengthened and expanded research relationships across the field with advanced next-generation AAV. Ongoing conversations with **Evolyra Therapeutics** (Richmond, VA) and the **University of Florida** efforts, led by Dr. Barry Byrne, for LGMD2D gene therapy continue into 2026, while preliminary discussions with **Genethon** may open the door to re-engaging their promising pre-clinical next-gen program.



# Research & Clinical Progress

3

## Small Molecule

In 2025, the Foundation initiated discussions with **Italfarmaco** to explore expanding the use of FDA-approved Givinostat to LGMDs. Interest remains strong, with follow-up planned for Q1 2026.

We also continue engagement with **Edgewise Therapeutics** regarding the potential expansion of Sevasemten clinical trials to include LGMD populations. For both small-molecule programs, the Foundation is advocating for umbrella trial designs that incorporate multiple LGMD subtypes.

4

## Gene Editing

Throughout the year, we had continual discussions with **Dr. Simone Spuler** and her team at **Charité Universitätsmedizin Berlin and Max Delbrück Center** in Berlin, Germany on the advancements of the gene editing research her team is working on for LGMD2D, focused on correcting disease-causing mutations at the DNA level.

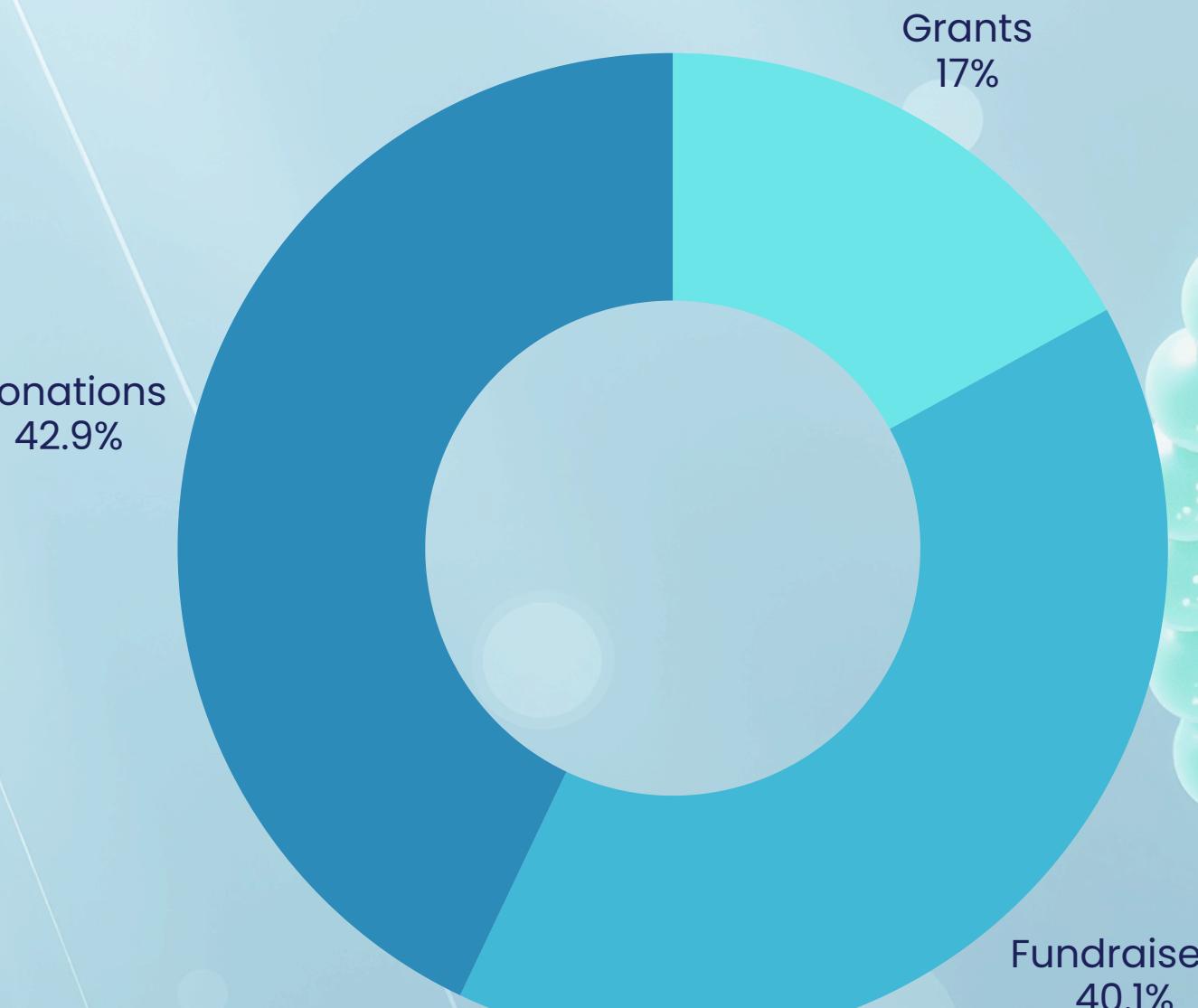
This innovative work aims to restore normal sarcoglycan function in affected muscle cells and represents a promising, long-term therapeutic approach for individuals living with LGMD2D.



# Financial Overview

**Over \$132,000  
raised.**

The majority of funds raised in 2025 came through community-led fundraisers, complemented by generous individual donations and grant support. These combined efforts enabled the Foundation to continue advancing research, advocacy, and patient support initiatives.





# Gratitude & Donor Recognition

## Hope in Motion Virtual 5K

### **Sponsors**

John Drypen Foundation  
Outstanding Orthodontics  
Old Time Hawkey

**137+ generous  
donors and  
participants**

## Fore the Cure Golf Tournament

### **Sponsors**

Cowen Tax Advisors  
Merchant Gateway  
Diane & Dennis Kral  
Verde Property Services  
Ulbrich Stainless Steel  
TREAT-NMD  
Roulston Services, LLC  
Lynn Vasquez  
Julia Vasquez  
The Maycock Family  
Carmela's Cookware Co.  
Daniel Zito & Company  
Birch Hill Landscaping  
David & Sharon Maycock

Dance-A-Lot Party Tents  
Costanzo Clothing  
Sizzle N Smoke BBQ  
Building Connections  
Counseling & Wellness  
Group  
Unique Extrusions, Inc.  
Dan Phelon & Diane  
Marchion  
Elmwood Barber Shop  
DRVN Enterprises Inc.  
Plaza Liquors  
Barker Specialty  
The Dolgert Family

**100+ generous  
donors and  
participants**

### **Grants**

Sarepta  
Therapeutics  
Eversource  
Energy

### **Donations**

Bergen House  
Taproom  
Travelers  
Champaionship  
Birdies for Charity

**120+ generous  
donations**



# 2026 Goals & Objectives

1

## **Accelerate and expand partnerships for clinical trial treatment development**

Support the initiation, expansion, and patient readiness of LGMD2D clinical trials by partnering with researchers and industry, promoting trial awareness, and reducing barriers to participation.

2

## **Advance patient-centered advocacy to support access to therapies and promote policies, including the PRV, that incentivize the development of treatments for LGMD2D.**

Engage policymakers, regulatory stakeholders, and partner organizations to elevate the LGMD patient voice, educate and encourage policy actions that accelerate rare disease therapy development.

3

## **Continue increasing awareness and understanding of LGMD2D among patients, clinicians, and the broader community.**

Expand educational outreach and visibility through targeted campaigns, provider engagement, and community partnerships to improve recognition, diagnosis, and support for individuals living with LGMD2D.

4

## **Expand and strengthen the LGMD2D patient registry to support research and clinical trial readiness.**

Continue raising awareness of patient registry to encourage enrollment and data completeness through targeted outreach, community engagement, and collaboration with clinicians.



# Contact Information

**Website:** <https://lgmd2d.org/>

**Email:** info@lgmd2d.org

**Social Media:**   