Winter 2026 • Vol. 6 / Issue 1

LGMD News

Uniting the Limb-Girdle Muscular Dystrophy Community

HONORING DONAVON DECKER
A Legacy of Advocacy, Hope, and Faith

LGMD DAY ON THE HILL
Together We Amplified the Voice of the Community

GRASP-LGMD
The Promise of AAV Gene Therapy for LGMDs

CURELGMD2T
My Diagnosis, My Mission: Living with LGMD R19/2T and Fighting for a Cure

United for Strength and a Cure

Mission to Cure Part 2: The Global Movement Transforming Sarcoglycanopathies

EVERY VOICE SPEAKS

KAT BRYANT KNUDSON
FOUNDER, SPEAK FOUNDATION
LIVING WITH LGMD

PATRICK MOESCHEN
MUSCULAR DYSTROPHY PATIENT ADVOCATE
LIVING WITH LGMD

WHERE EVERY VOICE SHAPES THE FUTURE OF MUSCULAR DYSTROPHY.

Every Voice Speaks **is a new podcast dedicated to uniting and empowering the muscular dystrophy community! Through honest conversations, education, and shared experiences, our podcast elevates the patient voice, strengthens advocacy, and fosters collaboration to advance research, drug development, and better futures for all of us living and working in the muscular dystrophy space.**

Each episode will blend real patient and caregiver stories with accessible discussions on emerging therapies, clinical trials, and the research process. Listeners will hear directly from patients, advocates, researchers, clinicians, regulators, and industry leaders, gaining clear insights into drug development while learning how their voices can shape trials, policy, and care.

With community spotlights, deep dives into treatment science, expert interviews, and practical guidance on advocacy and research participation, *Every Voice Speaks* ensures that lived experience remains at the center of progress.

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The Speak Foundation
Uniting the entire LGMD community to make a difference together in future treatments for this rare disease.

The origin of The Speak Foundation's name comes from Proverbs 31:8. It is: "Speak up for those who have no voice." Living with a rare disease means many of us wait years to have a voice in areas that impact our daily lives personally. The Speak Foundation helps our voices to be heard.

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Remembering Donavon Decker, an LGMD Pioneer

We highlight perspectives from advocacy organizations working tirelessly to advance treatments for sarcoglycanopathies—efforts that mirror Donavon's belief in collaboration, family engagement, and the power of shared purpose.

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Dear LGMD Community,

In this issue of *LGMD News*, we honor the life and legacy of Donavon Decker—a pioneer in LGMD advocacy whose impact continues to shape our community. Donavon lived with LGMD R3/2D and made history as the first recipient of gene therapy for any form of muscular dystrophy. Throughout his life, he exemplified resilience, courage, and an unwavering commitment to advancing research and amplifying the patient voice.

The stories featured in this issue reflect the very principles Donavon championed. We highlight perspectives from advocacy organizations working tirelessly to advance treatments for sarcoglycanopathies—efforts that mirror Donavon's belief in collaboration, family engagement, and the power of shared purpose.

Luke Garner's article further illustrates this legacy in action. His journey with LGMD R19/2T and the founding of CureLGMD2T embody the same determination to ensure that every LGMD subtype is seen, heard, and supported—an approach that aligns closely with The Speak Foundation's mission to serve all affected by LGMD, from the most common to the most rare.

This spirit of advocacy also comes to life in our coverage of LGMD Day on the Hill, shared by participant Bliss Sheets Welch. The gathering brought together patients, families, and advocates united by a shared goal: advancing understanding, care, and treatment options for the LGMD community.

Looking ahead, we are also excited to announce the upcoming LGMD Scientific Summit in 2026, which will further advance research and collaboration across the LGMD landscape. More details will be shared in the Spring edition.

We hope this edition of *LGMD News* not only informs you about scientific progress, but also honors the enduring legacy of advocacy, resilience, and hope that Donavon Decker exemplified. Thank you for your continued dedication to improving the lives of those affected by LGMD. Together, we carry this work forward. ▪

Kat Bryant Knudson

Kathryn Bryant Knudson
Editor In Chief
Founder & CEO, The Speak Foundation

In the News

The Speak Foundation joins the Muscular Dystrophy Public Health Consortium representing Limb Girdle Muscular Dystrophy.

International Consortium of LGMD Organizations

United States

The Speak Foundation
Uniting the entire LGMD community
TheSpeakFoundation.com

Beyond Labels & Limitations
Funding research for LGMD R1/2A and educating on its disease course
BeyondLabelsLimitations.com

Breathe with MD
Educating and raising awareness about breathing muscle weakness in neuromuscular disease
BreatheWithMD.org

CamronsCure
Funding research for LGMD R18/2S
Facebook.com/LGMDCC

Coalition to Cure Calpain 3
Funding research for LGMD R1/2A
CureCalpain3.org

CureLGMD2D Research Foundation
Funding research for LGMD R3/2D
sujivasu01@gmail.com

CureLGMD2i
Funding research for LGMD R9/2I
CureLGMD2i.org

CureLGMD2T
Raising awareness for LGMD R19/2T and GMPPB-related Congenital Muscular Dystrophies
CureLGMD2T.org

Dion Foundation
Funding research for LGMD R5/2C
TheDionFund.org

Kurt + Peter Foundation
Funding research for LGMD R5/2C
KurtPeterFoundation.org

LGMD Awareness Foundation
Raising awareness of and advocating for the LGMD community
LGMD-Info.org

LGMD-1D DNAJB6 Foundation
Representing LGMD D1/1D and DNAJB6 subgroup
LGMD1D.org

LGMD2D Foundation
Funding research for LGMD R3/2D and educating patients and physicians
LGMD2D.org

LGMD2i Research Fund
Funding research for LGMD R9/2I and educating the patient community
LGMD2iFund.org

LGMD2L Foundation
Representing the LGMD R12/2L Anoctamin5-related community
LGMD2L-Foundation.org

Team Titin
Strengthening the titin community: LGMD R10/2J
TitinMyopathy.com

The Jain Foundation
Funding research for LGMD R2/2B and educating the patient community
Jain-Foundation.org

Argentina

ADM Argentina Muscular Dystrophy LGMD Group
Funding research for neuromuscular diseases
ADM.org.ar

Australia

Daniel Ferguson LGMD2A Foundation
Funding research for LGMD R1/2A and educating the patient community
DFFoundation.com.au

France

"GI LGMD"/LGMD Patient Group of AFM-Telethon
Focusing on all subtypes of LGMD, supporting research and educating the patient community
LGMD.AFM-Telethon.fr

Italy

Conquistando Escalones Association
Funding research for LGMD D2/1F
ConquistandoEscalones.org

"GFB ONLUS"/Family Group of Beta-Sarcoglycanopathy
Representing the LGMD R5/2C Gamma Sarcoglycan-related, LGMD R3/2D Alpha Sarcoglycan-related, LGMD R4/2E Bèta-Sarcoglycan-related, and LGMD R6/2F Delta-Sarcoglycan-related communities
Beta-Sarcoglicanopathy.org

UILDM – Unione Italiana Lotta alla Distrofia Muscolare
Focusing on all subtypes of LGMD, raising awareness and providing support for the entire Italian community
UILDM.org

Italian Association Calpain 3
Funding research for the LGMD R1/2A Calpain 3-related community
AICA3.org

Japan

Patients' Association for Dysferlinopathy Japan
Representing the Japanese and International LGMD R2/2B Dysferlin-related and Miyoshi Muscular Dystrophy 1 (MMD) communities
PADJ.jp/index.html

Netherlands

Stichting Spierkracht
Raising awareness and supporting the LGMD R3/2D Alpha Sarcoglycan-related community
StichtingSpierkracht.com

South Korea

Korean Dysferlinopathy Patients Association
Providing patients with LGMD R2/2B information and research updates
Cafe.Naver.com/UniteDsyferlinopathy

Spain

Conquistando Escalones Association
Funding research for LGMD D2/1F
ConquistandoEscalones.org

Proyecto Alpha
Funding research for LGMD R5/2C Gamma Sarcoglycan-related, LGMD R3/2D Alpha Sarcoglycan-related, LGMD R4/2E Bèta-Sarcoglycan-related, and LGMD R6/2F Delta-Sarcoglycan-related communities
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For additional information about the International Consortium of LGMD Organizations: **ContactUs@TheSpeakFoundation.com**

Doris G. Leung, MD, PhD

Director, Center for Genetic Muscle Disorders at Kennedy Krieger Institute

Meet the Expert

Doris G. Leung, MD, PhD is the director of the Center for Genetic Muscle Disorders at Kennedy Krieger Institute and an associate professor of neurology at the Johns Hopkins University School of Medicine. She obtained her undergraduate degree in biochemical sciences from Harvard University and her medical degree from Duke University School of Medicine. She completed her neurology training at the Stanford University Medical Center. Dr. Leung joined the Center for Genetic Muscle Disorders as a translational research fellow in 2010. Since then, she has earned a PhD from the Graduate Training Program in Clinical Investigation at the Johns Hopkins Bloomberg School of Public Health. Dr. Leung's multidisciplinary clinic focuses on the diagnosis and management of hereditary muscle diseases, and her research centers on the development of biomarkers for muscle disease. She has also served as site principal investigator for multiple clinical trials and natural history studies in muscular dystrophy.

Q

I have a confirmed diagnosis of LGMD R1/2A. What key outcome measures or biomarkers do you see as most promising for inclusion in clinical trials, and how might MRI be applied not only for visualizing disease progression but also for tracking therapeutic response?

A

When we talk about whether an intervention is effective from an FDA perspective, it means that it has a positive impact on how a patient "feels, functions, or survives." In general, clinical outcome assessments are supposed to directly measure how patients feel, function, or survive, and these will always be a crucial component of clinical trials. In muscular dystrophy trials, these will include muscle strength and function tests. However, there is also growing interest in using biomarkers as surrogate endpoints for trials. Biomarkers may not directly measure function, but if they can be shown to be reliable predictors of function, they could help researchers overcome some of the challenges we encounter with strength and function testing. I anticipate that many future trials in LGMD will include a combination of outcome assessments and surrogate endpoints.

There has been a lot of interest recently in using MRI-based measurements as biomarkers. MRI scans can be used to measure different features of skeletal muscle, including how much intact muscle tissue there is and how much has been damaged owing to muscular dystrophy. There are also practical advantages to using MRI to evaluate muscle; unlike muscle biopsy, for instance, you can measure the same region of muscle over and over again, and unlike many function tests, they are less impacted by the effects of fatigue. It may also be possible to detect smaller amounts of change on MRI than you would with strength and function testing alone.

Another class of biomarkers that are being evaluated in trials now come from wearable activity trackers. A number of measurements can be made from these trackers, but one that has gained a lot of interest recently is the distribution of walking speed. As a person goes about their day, they will walk faster or slower at different times. As their LGMD progresses, the range of walking speeds becomes more narrow, and this can be monitored using these wearable devices. These devices also provide a look into how a patient interacts with their environment outside the clinics where clinical trial measurements typically take place.

Q

Other than Calcium and Vitamin D for bones, are there any other supplements you recommend for individuals with neuromuscular diseases?

A

A lot of my patients take a variety of supplements that they learn about from various sources. In general, I don't actively recommend taking any particular supplements for muscular dystrophy. I don't think there's anything inherently wrong with taking them; however, patients rely on physicians to make recommendations that are based in science, and nutritional supplements have not been researched extensively enough in muscular dystrophy for me to give scientifically-sound advice about them. The exception would be if a patient has a proven deficiency of a vitamin, in which case it makes sense to take supplements to reach a normal level. However, taking supplements to an above-normal level or taking them without being able to measure the level in one's system at all is difficult to justify.

Q

I am 21 years old living with LGMD R9/2I. I am currently walking, and I would like to know how I can keep walking as long as possible.

A

The rate of disease progression in LGMD R9/2I is affected by multiple factors. The specific pathogenic variant in FKRP is one of those factors. Multiple studies in LGMD R9/2I have shown that people who have a founder variant common in European populations (c.826C>A) on both copies of FKRP will tend to lose ambulation at older ages than people who have other pathogenic variants. Unfortunately, this is not something we can change at this time.

In coming up with ways to potentially prevent loss of ambulation that we can control, I start by thinking about patients who may have lost ambulation earlier than I would have expected based on their muscle strength. Here are some of my thoughts:

- **Maintaining range of motion and preventing contractures.** When muscles start to become weak, the tendons and ligaments around them can start to get tight. Losing flexibility and range of motion really impairs our ability to correct our posture and stop ourselves from falling—which leads to my next point.
- **Preventing falls.** Some patients gradually lose the ability to walk, transitioning to longer and longer periods in a scooter or wheelchair. Others have a single catastrophic fall that causes a major injury. Patients don't always recover the ability to walk after this type of injury, so preventing this type of fall (and reducing the number of falls in general) would be an important goal in maintaining ambulation as long as possible.
- **Optimizing management of other health problems.** I'll use diabetes as my example, since it's a common disease. Patients with muscular dystrophy can find ways to compensate for loss of muscle strength for a long time. However, if there is a second medical problem that remains untreated, like diabetic neuropathy, the combination of loss of strength from muscular dystrophy and loss of sensation from the diabetic neuropathy can make walking even more dangerous. I encourage patients to work on getting their non-muscular dystrophy health conditions under good control as much as possible.

In general, I don't actively recommend taking any particular supplements for muscular dystrophy. I don't think there's anything inherently wrong with taking them; however, patients rely on physicians to make recommendations that are based in science, and nutritional supplements have not been researched extensively enough in muscular dystrophy for me to give scientifically-sound advice about them.

Question

I personally have always been in favor of knowing as much as possible, and I'm pretty sure I would get tested if I were at risk, but not everyone is going to feel the same way. Their reasons for not wanting to get tested might seem insufficient, but there really is no magical argument that will convince all your family members to get tested.

Q

We have a mutated MYOT gene in our family which is the source of a late onset myotilinopathy in several older members of the family, including me. It's autosomal dominant, so there are many children, grandchildren, and a recent great-grandchild presently at risk of inheriting the faulty gene. Can you suggest strategies for encouraging family members to be tested for a possible gene mutation?

A

In my experience, there are many people who are at risk for an inherited disease and choose not to get tested. I personally have always been in favor of knowing as much as possible, and I'm pretty sure I would get tested if I were at risk, but not everyone is going to feel the same way. Their reasons for not wanting to get tested might seem insufficient, but there really is no magical argument that will convince all your family members to get tested. Furthermore, pushing too hard with people who are not ready to face this decision often generates even more resistance to the idea. When I speak with relatives, I explain that they may have inherited a genetic condition, that testing is available, and that the results may guide important health decisions. What they choose to do with that information is up to them.

Now, if there were a treatment available in the pre-symptomatic stage that would improve the outcome down the line, the conversation changes a lot. This is especially true of the pathogenic variants that are known to cause heart disease. In these cases, there is a practical, potentially life-saving reason for getting tested, as people who have the pathogenic variant can undergo cardiac surveillance that allows us to identify heart disease and treat it before life-threatening complications happen. In these situations, I do encourage patients more strongly to get tested, and if they cannot, to undergo cardiac surveillance even if there is no confirmed diagnosis.

Q

What do you recommend for individuals with tight Achilles tendons? Are there nighttime braces or specific exercises that are recommended?

A

There are many exercises and devices available that are designed to stretch the Achilles tendons. I do not think there is a consensus on which one is best. They all work, though, so picking a specific approach generally comes down to what the patient can do consistently. The duration of the stretch matters a lot. Using night splints to stretch the ankle all night provides a much longer stretch than doing active stretches for a few minutes a day. However, if someone finds it too uncomfortable to wear the night splints, then that few minutes a day is better than not stretching at all. One of the favorites in our clinic is the standing wedge. There are different types, but it's basically a platform with an inclined surface that you stand on so that heels are a bit below the level of the toes. Ask your physical therapist before buying one, though. Depending on how tight the tendons are, the incline may need to be adjusted.

Accepting Applications Beginning January 5, 2026

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We Offer HOPE.

Are you an individual living with limb-girdle muscular dystrophy who needs assistance covering the costs of mobility and durable medical equipment (DME)? The SPEAK Foundation is here to help.

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For additional information or to submit an application from January 5, 2026 through February 5, 2026, please visit **TheSpeakFoundation.com/grant-programs**.

The HOPE Project

A Program of The SPEAK Foundation

TheSpeakFoundation.com/grant-programs

Accepting applications from January 5, 2026 through February 5, 2026.
Available to US residents only.

You're not alone on your LGMD journey.

LGMD-Info.org is a one-stop shop designed for **Patients, Families** and **Advocates** seeking trustworthy information and community support.

LGMD AWARENESS FOUNDATION

Access advocacy resources, event listings, grant opportunities, genetic testing, and patient registries, plus inspiring Spotlight Interviews from others living with LGMD.

Together, we can advocate, educate, and celebrate!

JOIN OUR MAILING LIST:

The LGMD Awareness Foundation, Inc. is a 501(c)(3) non-profit advocacy organization committed to raising global awareness about limb-girdle muscular dystrophy (LGMD), a group of rare and often debilitating neuromuscular diseases. Help support our efforts at **lgmd-info.org/donate** to raise awareness of LGMD and advocate for individuals living with these rare conditions — so they can more easily access diagnosis, care, and treatment.

Q

Will exon skipping therapy work for LGMDs?

A

Exon skipping is a technology that is currently used in several drugs that are designed to treat Duchenne muscular dystrophy (DMD). The reason that exon skipping can be applied to DMD relies on the fact that most cases of DMD are caused by large deletions in the dystrophin gene. These large deletions usually shift the DNA sequence so that a stop signal ends up in the middle of the gene. The stop signal tells the muscle's copying machinery to stop reading the DNA at that point, so the full gene doesn't get copied. Exon skipping overcomes this problem by preventing a piece of the code from being incorporated into the final product. This results in making the deletion even bigger, but it also shifts the sequence again so that there isn't a stop signal in the wrong place anymore. The dystrophin protein that gets produced has a piece missing in the middle, but because the gene is very large, it can still partially function without that piece.

Now the genes that cause LGMD are a lot smaller than the dystrophin gene, so deleting a whole exon out of one of these genes will probably have a more damaging impact on the function of the gene. So my guess is that exon skipping is not going to play a major role in treating LGMD. However, the type of drug that is used to skip exons (antisense oligonucleotides) can be designed to do things other than exon skipping, and those could be applied to the LGMDs in the future. In animal experiments, oligonucleotides can be used to suppress or enhance the expression of other genes. They can also modify the structure of genes to alter their function. All of these types of actions could theoretically be applied to treat different forms of LGMD.

Q

I have low bone density, and my endocrinologist and team are always conflicted about whether prescribing treatments like bisphosphonate is risky for someone with LGMD. What considerations should I keep in mind?

A

It's difficult for me to know what specific risks may be present in your case, but if it helps put this into perspective at all, our endocrinology clinic team prescribes bisphosphonates to patients with all types of muscular dystrophy. When to initiate treatment, how long to continue treatment, and what treatment goals should be are much harder questions to answer and are generally considered case-by-case. However, the bisphosphonates remain the main class of drugs used to treat osteoporosis, and they can and are safely used in people with muscular dystrophy to prevent loss of bone density. There are now emerging therapies that promote the building of new bone, rather than just preventing the loss of existing bone. While these have not been extensively studied in muscular dystrophy, it is exciting to see potential new options for treating osteoporosis, which can be a major cause of medical complications in our patients. ■

"However, the bisphosphonates remain the main class of drugs used to treat osteoporosis, and they can and are safely used in people with muscular dystrophy to prevent loss of bone density."

Have a Question for Our Experts?

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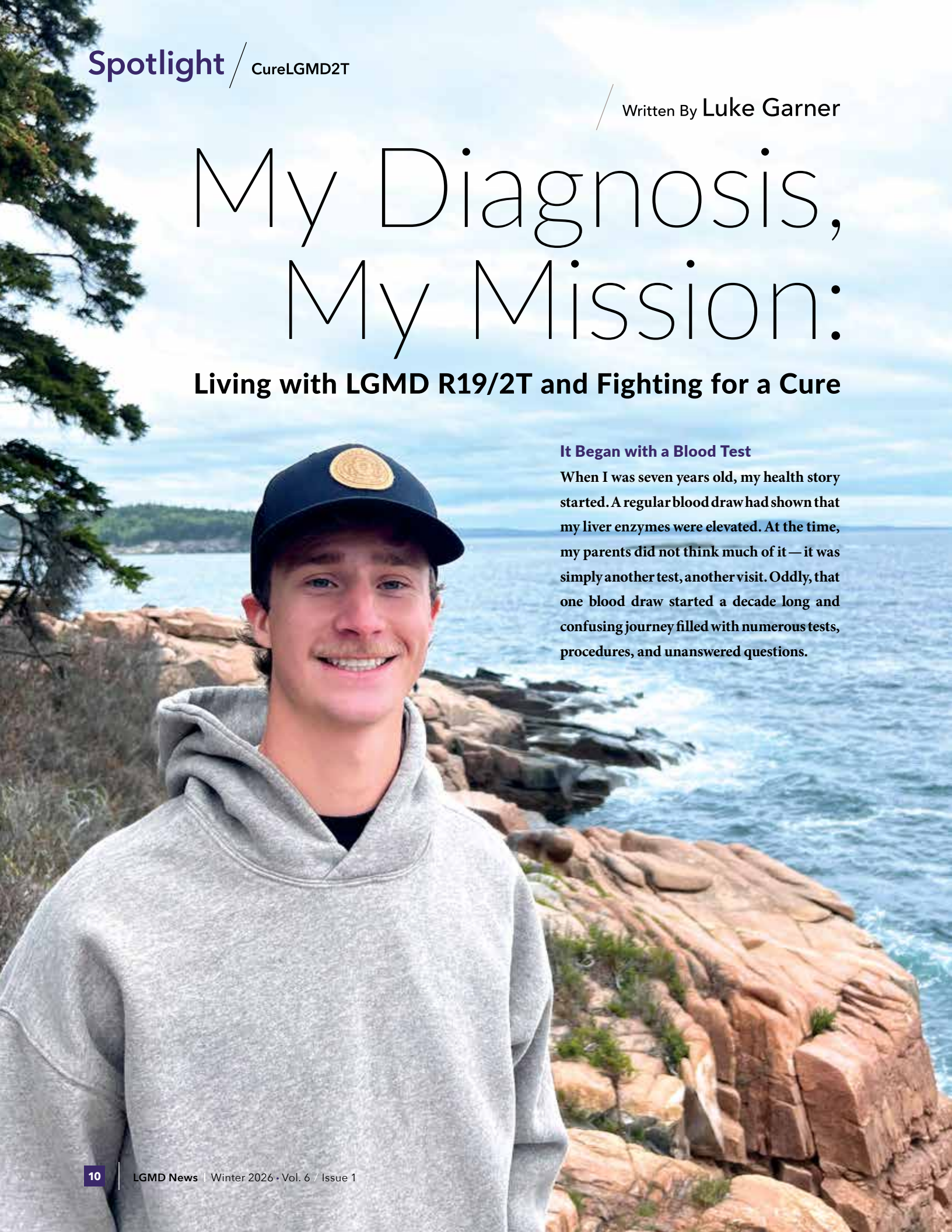
Written By Luke Garner

My Diagnosis, My Mission:

Living with LGMD R19/2T and Fighting for a Cure



It Began with a Blood Test

When I was seven years old, my health story started. A regular blood draw had shown that my liver enzymes were elevated. At the time, my parents did not think much of it — it was simply another test, another visit. Oddly, that one blood draw started a decade long and confusing journey filled with numerous tests, procedures, and unanswered questions.

Eventually, doctors discovered I had an elevated CK (creatine kinase) level, a sign that something might be affecting my muscles. A muscle biopsy was ordered — but the results were normal. We went through genetic testing for Duchenne and Becker muscular dystrophies, but those tests were negative, too.

Without a clear answer, I was eventually given a diagnosis of *idiopathic hyperCKemia*, which basically meant, "Your CK levels are high, and we don't know why."

Living with the Unknown

For the next ten years, my family and I continued with life as best we could. But in the back of our minds, the uncertainty lingered. We didn't know what was really going on with my body or what my future might look like.

When I turned 17, we decided it was time for a second opinion. That decision changed everything.

A new round of genetic testing finally gave us the answer we'd been searching for: I was diagnosed with limb-girdle muscular dystrophy, type 2T (LGMD R19/2T), caused by mutations in a gene called GMPPB. The diagnosis was later confirmed by Dr. Steven Moore, a renowned expert in neuromuscular pathology.

It was overwhelming. After ten years of questions, we finally had a name for what I was living with — but it wasn't the answer we had hoped for. There is no cure, no treatment. Just a name and a new reality.

Turning Diagnosis into Purpose

Once we knew what we were facing, my dad and I knew we couldn't sit still. He immediately began learning everything he could about LGMD R19/2T. He attended rare disease conferences and even participated in LGMD Day on the Hill in Washington, D.C., advocating for increased awareness and research.

Those experiences lit a fire in both of us.

Above: Luke Garner and his father James together founded CureLGMD2T, a nonprofit dedicated to supporting patients and families affected by LGMD R19/2T and GMPPB-related congenital myopathy.

Together, we founded CureLGMD2T, a 501(c)(3) nonprofit dedicated to supporting patients and families affected by LGMD R19/2T and GMPPB-related congenital myopathy. Our mission is to raise awareness, build community, develop a patient registry, and push forward the research that could someday lead to a treatment — or even a cure.

We want to give this rare condition a voice, because too often, people with LGMDs are overlooked or misunderstood.

"Our mission is to raise awareness, build community, develop a patient registry, and push forward the research that could someday lead to a treatment — or even a cure."

Above: Friends Isaac Redman (Left) and Luke Garner (Right), both living with LGMD R19/2T, working together at the recent 2025 International Limb Girdle Muscular Dystrophy Conference.

Finding Community and Connection (Start of the 2T Team)

One of the most meaningful parts of this journey has been meeting others who are living with the same diagnosis. That's how I met Isaac Redman. He's my age and was also diagnosed with LGMD R19/2T. We connected instantly — not just because of the shared condition, but because each of us really understood what the other was going through.

I'm currently a fourth-year nursing student, and Isaac is in his fourth year studying marketing communications. With Isaac and his mom Becky joining our team, we were able to expand our outreach, build a social media presence, and strengthen our community connections.

It's been amazing to see how much stronger we are when we work together.

The Power of the Patient Registry

A major focus of CureLGMD2T is developing our patient registry. It's one of the most powerful tools we have — not just to connect with others who have LGMD R19/2T, but to help researchers better understand this condition.

If you or someone you know has LGMD R19/2T or GMPPB-related congenital myopathy, I strongly encourage you to register. Every entry helps:

- **Raise awareness**
- **Strengthen the LGMD R19/2T community**
- **Provide valuable data for future research and clinical trials**

The registry gives us a collective voice — the more people who participate, the louder and more impactful that voice becomes.

How You Can Help

Starting CureLGMD2T has been a journey of hope, determination, and resilience. But we can't do it alone. We rely on donations, grants, and community support to fund research, support clinical studies, connect patients, and keep moving forward. Every contribution — big or small — brings us closer to answers and helps shine a light on this rare condition.

If you're able to donate, spread the word, or get involved, please visit our website at **CureLGMD2T.org**. Together, we can build something bigger than ourselves. Something powerful. Something that matters.

> **"Getting diagnosed with LGMD R19/2T wasn't the end of my story — it was the beginning of a new chapter. One filled with challenges, yes, but also filled with community, compassion, and purpose."**

Moving Forward with Purpose

Getting diagnosed with LGMD R19/2T wasn't the end of my story — it was the beginning of a new chapter. One filled with challenges, yes, but also filled with community, compassion, and purpose.

Through CureLGMD2T, I've learned that rare doesn't have to mean invisible. Even in the face of uncertainty, we can choose action. Sometimes, the best way to cope with a diagnosis is to fight for the people who share it.

We're not giving up. Not now. Not ever. ▪

Connect with Us

Info@CureLGMD2T.org

CureLGMD2T.org

Facebook.com/CureLGMD2T

Instagram.com/CureLGMD2T

Bliss Sheets Welch

LGMD Day on the Hill

Policies should begin with the people they affect most. On the morning of September 9, 2025, eighty-four LGMD advocates—speaking with one unified voice—dispersed across Capitol Hill in Washington, D.C. Our mission was to ensure the stories, challenges, and hopes of the LGMD community were heard by those who shape policy. It was incredible to experience LGMD Day on the Hill with my daughter, Annabelle. I am so proud of her for speaking up in several meetings to advocate for me and the entire LGMD community. We were both grateful for the opportunity to make sure the voices of the LGMD community were heard.

Above: Eighty-four LGMD advocates dispersed across Capitol Hill in Washington, D.C. to ensure the stories, challenges, and hopes of the LGMD community were heard by those who shape policy.

From Preparation to Action

The evening before, LGMD advocates, family, and caregivers had gathered at the offices of Thorn Run Partners, a DC firm, which partnered with the Speak Foundation to organize LGMD Day on the Hill, for an overview of what to expect during our fast-paced day on Capitol Hill. Each of us joined a team with advocates from other states, and we attended the scheduled meetings together. Our LGMD community knows that together we are stronger!

In our orientation, we were given an overview of current policies that are directly impacting our community. Our assignments were clearly laid out, and we were provided

one-page summaries for the following legislative priorities:

- **PRV (Pediatric Priority Review Voucher) Protections**
  Renewing this vital incentive to drive innovation and accelerate treatments for rare diseases like LGMD.
- **Regulatory Flexibility for Rare Diseases**
  Encouraging federal agencies to adapt traditional clinical trial frameworks for smaller patient populations.
- **NIH Research Funding**
  Continuing funding NIH grants for essential LGMD research.
- **BENEFIT Act**
  Requiring FDA to prioritize patient-focused drug development.
- **Scientific EXPERT Act**
  Establishing a process for the FDA to participate in externally led, science-focused drug development meetings to address challenges impacting the development of drugs for rare diseases.
- **Home-Based Care Access**
  Advocating for expanded skilled home care services to support independence and quality of life for those living with muscular dystrophy.

Over the course of the day, LGMD constituents from 20 states attended a total of 54 congressional meetings. We are confident that our advocacy efforts moved us one step closer to continued research funding, treatment development, and access to much needed Personal Care Assistant services.

Behind the Scenes

The photographs on these pages are a powerful reminder that our voices were heard, but they only tell part of the story. They don't show the worry and fear of the individual who hoped their power wheelchair was still fully functional when their plane landed in DC. They don't show the dehydration of the individual who limited their liquid intake because they didn't know if they could safely navigate a public restroom. They don't show the caregiver who was on duty the entire trip, ready to assist at any moment. The photos don't show the individual that slept in their power wheelchair for the entire trip because it was more comfortable than a hotel bed. They don't show the sheer exhaustion of the ambulatory individual who wasn't sure their legs would carry them one step farther. These are the unseen realities we carry with us, and they motivate us to advocate on behalf of our community.

Our successful day was concluded with a reception hosted by the Speak Foundation to honor a true champion of the LGMD community, Congressman Dr. Neal Dunn. The Speak Foundation's founder Kathryn Bryant Knudson presented an award to Rep. Dunn for his work on behalf of the LGMD and rare disease communities. We are fortunate to have Congressman Dunn in our corner!

Above: Kathryn Bryant Knudson (Right) honoring Congressman Dr. Neal Dunn for his work on behalf of the LGMD and rare disease communities.

> *It's not too late for your voice to be heard. Together we are stronger!*

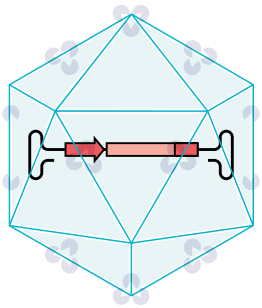
Together We Can Amplify the Voice of the LGMD Community!

Advocacy doesn't just happen in person. Your voice can be heard from the comfort of your own home. Every call and email a congressional office receives is logged and tracked to inform members of Congress about constituent priorities. If you were unable to attend LGMD Day on the Hill, you can still participate by scanning the QR code to take part virtually. It's not too late for your voice to be heard. Together we are stronger! ■

BECOME A VIRTUAL

DELEGATE TODAY!

The Promise of AAV Gene Therapy for LGMDs



Above: A capsid (teal icosahedron) with peptide modifications (purple), and a therapeutic gene inside (red).

Through participation in networks like the GRASP-LGMD Consortium, patients help us define a natural course of disease—a course we rely on in designing clinical trials to evaluate the efficacy of novel drugs.

The various subtypes of limb-girdle muscular dystrophy (LGMD) are caused by mutations in over 30 different genes, leading to missing or defective skeletal muscle proteins. In circumstances where the protein is lost or has reduced function, gene replacement strategies are a promising therapeutic option, targeting the root cause of the disease rather than only treating the resulting symptoms.

Adeno-associated viruses (AAVs) are naturally occurring viruses not associated with any known human disease. AAVs are able to deliver DNA to cells they infect and represent an ideal vehicle for delivering gene therapies. This is especially true for recessively inherited LGMDs, as the coding sequences of most of the associated genes can fit within the AAV packaging limit, enabling production of the full protein sequence to maximize potential benefit.

Which cells the AAV targets is largely dictated by the capsid, or outer shell of the virus. Early clinical trials for muscular dystrophy used AAV vectors with naturally occurring capsids that target many cell types in the body. These types of AAVs especially target the liver, which has led to fatalities in clinical trials from liver toxicity. Over more than two decades, researchers have been developing AAV capsids that better target tissues of interest (muscle in the case of LGMDs), with the goal of improving safety and enhancing gene delivery. Companies like Latus Bio, Solid Biosciences, AskBio, Evolyra Therapeutics, and others are testing modified capsids for various indications, and early clinical trial results support this approach.

Our opportunity to translate these advances relies on the generosity of patients and their families. Through participation in networks like the GRASP-LGMD Consortium, patients help us define a natural course of disease—a course we rely on in designing clinical trials to evaluate the efficacy of novel drugs. Further, patient bio-samples allow us to better define the mutation spectra and characterize pathology associated with individual LGMD subtypes. This valuable information is used to generate genetically and phenotypically accurate models, allowing us to assess safety and fine-tune therapeutic design prior to clinical trials. In this way, future treatments can be developed that will be safe and effective at targeting the root causes of LGMDs. ■

Written by Ellie Carrell, PhD
Assistant Professor in the Department of Neurology, Virginia Commonwealth University

GRASP-LGMD Natural History Study Locations

UPDATED: JANUARY 1, 2026

Active GRASP-LGMD Natural History Study

Recruiting:

Trial Readiness and Endpoint Assessment in LGMD R1/2A (GRASP-01-003)

Inclusion Criteria

- Age between 12 – 50 at enrollment
- Clinically affected (defined as weakness on bedside evaluation in a pattern consistent with LGMD R1/2A)
- Genetic confirmation of LGMD R1/2A (presence of homozygous or compound heterozygous pathogenic mutations in CAPN3)
- Must be able to provide written informed consent and be willing and able to comply with all study requirements

Exclusion Criteria

- Have contraindications to MRI or MRS (e.g., non-MR compatible implanted medical devices or severe claustrophobia)
- Non-ambulatory, defined as unable to safely walk 10 meters without assistive devices (ankle foot orthotics allowed)
- Positive pregnancy test at any timepoint during trial
- Have dominantly inherited CAPN3 mutations (i.e. LGMD D4)
- Any other illness that would interfere with the ability to undergo safe testing or would interfere with interpretation of the results in the opinion of the site investigator

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Spotlight: Dr. Ellie Carrell

Virginia Commonwealth University

Dr. Ellie Carrell is an Assistant Professor in the Department of Neurology at Virginia Commonwealth University. Over her 19-year career, Dr. Carrell's research has consistently focused on skeletal muscle, both in states of disease and normal physiology. She earned her undergraduate degree in Biomedical Engineering from the University of Iowa and completed her graduate and early postdoctoral training at the University of Rochester. As a postdoctoral fellow at the Children's Hospital of Philadelphia, she acquired expertise in adeno-associated virus (AAV) development with a focus on improved safety through intelligent transgene design. Working alongside clinicians, her current research aims to better understand mechanisms of disease in LGMDs and myotonic dystrophy to advance the development of effective therapies.

In It for the Long Haul

Why Retention in Clinical Trials Matters

By Andrew Huy Cao Nguyen, MD
Director, Medical Writing, BridgeBio

Clinical trials play a crucial role in evaluating the safety and efficacy of an investigational therapy. For a trial to produce sufficient high-quality data and results that meet trial goals and regulatory requirements, it requires enough participants—and those participants must remain involved for the duration of the trial. For rare diseases such as limb-girdle muscular dystrophy (LGMD), where the pool of potential participants is very small, retention becomes especially essential.

Why Participants Leave Early

There are many reasons why participants may leave a trial early: unclear participant expectations, financial and travel burdens, side effects, or complex trial designs. Each of these factors can influence someone's decision to continue his or her involvement in a trial.

When participants drop out, the consequences can be far-reaching. The necessary strength and quality of the trial data can be affected, leading to less reliable results or insufficient data to perform effective statistical analysis of trial findings. Timelines can be delayed, and particularly in the case of rare diseases, the loss of even a few participants can slow progress substantially. For people with LGMD, this can mean longer waits for potentially life changing therapies that might improve quality of life.

What Helps Participants Stay

Researchers and trial centers recognize that participant retention is just as important as recruitment. Retention efforts often focus on three areas: communication and trust, practical support, and recognition.

Communication and Trust

Communication between the trial team and the participant must be clear, transparent, and continuous. Making trial materials available in plain language, providing regular updates, and offering opportunities to ask questions, all help participants understand what is expected of them and how the trial is progressing. Informed participants experience greater comfort and less anxiety surrounding their trial involvement. When participants feel heard and respected, they are more likely to remain engaged.

Practical Support

Participation often requires significant adjustments to daily life. Travel to trial sites, time away from work, school and family responsibilities can all make continued trial participation difficult, especially for lengthy trials. Assistance with transportation, reimbursement for trial-related travel costs, flexibility with appointment and visit scheduling, offering remote visit options, and provision of childcare support can ease these burdens and make it easier for participants to stay involved. From a researcher and trial sponsor standpoint, designing a trial to minimize protocol complexity and participant burden can also help make participant engagement easier.

Recognition and Care

Recognizing participants' involvement and contributions helps ensure that they feel valued. Prompt trial team response to adverse events reassures participants that their safety is a top priority. Involving participants' caregivers and families in updates keeps everyone aligned with regard to trial involvement and to trial progress and goals. Simple gestures—such as thank-you notes, tokens of appreciation, or personalized health updates—signal that the trial team respects and appreciates the time and effort participants contribute and makes them feel valued.

Conclusion

Participant retention in clinical trials directly affects the quality, consistency, and efficiency of the research conducted. Strong retention means more comprehensive data, fewer delays, and clearer answers about the efficacy and safety of a treatment. For rare conditions such as LGMD, each participant represents an essential part of the trial. Their continued involvement determines how quickly and effectively research can move forward toward new treatment options that can potentially improve quality of life or even be life-changing.

Retention in Clinical Trials: Key Points

In rare diseases like LGMD, every participant represents an essential part of the trial population.

Participants may withdraw from a study early because of unclear communication, financial or travel demands, side effects, or lengthy and complex trial schedules.

Retention, or continuous participant involvement, supports more comprehensive data, consistent results, and fewer delays in determining whether a treatment may be effective and safe.

Strategies that support retention include:

Clear communication

Easy-to-understand materials, regular updates, and encouraging dialogue between participants and the trial team.

Practical support

Travel help, cost reimbursement, flexible scheduling, and simpler trial design.

Recognition and care

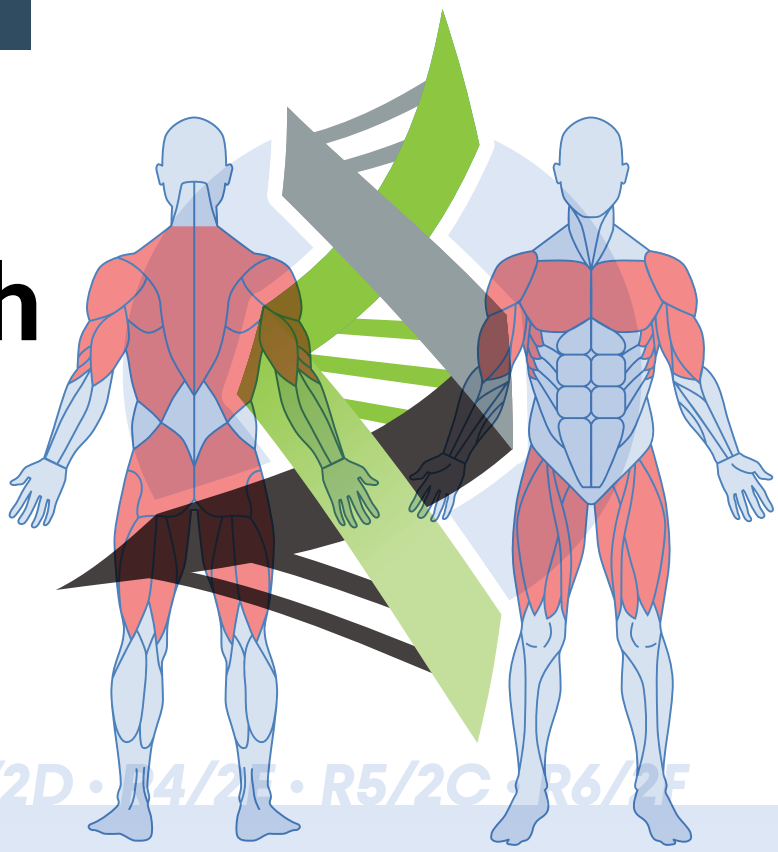
Prompt attention to safety concerns and acknowledgment of participant contributions.

Higher participant retention supports collection of high-quality trial data, efficient conduct of trials, and thorough evaluation of treatment efficacy and safety, helping drive research progress towards potential new therapies.

For more information visit bridgebio.com/lgmd2i-r9/

United for Strength and a Cure:

Mission to Cure Part 2: The Global Movement Transforming Sarcoglycanopathies



Around the world, thousands of patients are diagnosed with a genetic disorder so rare that many families spend years searching for answers—and even longer searching for hope.

Sarcoglycanopathies are four subtypes of LGMD: LGMD R3/2D, R4/2E, R5/2C, and R6/2F, which collectively represent up to 25% of all LGMD cases and are among the most severe and rapidly progressing forms of the disease. Symptoms often begin before age 10, with children who once ran freely gradually losing the ability to climb stairs, lift their arms, and eventually walk. Many require a wheelchair in adolescence, along with specialized respiratory and cardiac care, as muscles throughout the body weaken over time.

Sarcoglycanopathies are caused by pathogenic variants in one of four genes—SGCA, SGCB, SGCG, or SGCD—each responsible for producing a sarcoglycan protein. The four sarcoglycans are joined together in a bundle that helps protect muscle cells from damage during movement. These conditions are autosomal recessive: to develop the disease, a child must inherit a mutated copy of the same gene from both parents, each of whom is typically a healthy carrier.

Although these diseases are ultra-rare, they are present worldwide, with higher reported frequencies in Japan, the Netherlands, Europe, and across Brazil and India, where clusters of cases have been identified through genetic studies. Despite multiple patient registries, the exact numbers for each sarcoglycanopathy subtype remain unclear. Many individuals go undiagnosed or misdiagnosed, suggesting a global footprint far greater than what has been recorded. Clinically, sarcoglycanopathies share similarities with Duchenne muscular dystrophy—including rapid functional decline during childhood and adolescence—underscoring the urgency for early intervention.

Yet despite these challenges, families are no longer fighting alone. A growing network of dedicated sarcoglycanopathy advocacy organizations worldwide—led by fiercely determined parents and patients—is accelerating research, funding natural history studies, advancing clinical trial readiness, and pushing for earlier and more accurate diagnosis. Their unified message is clear: time lost is muscle lost, and every year without treatment matters.

Today, the tide is turning. Gene therapy programs—once only a distant possibility—are now in clinical development. Researchers are also exploring small-molecule therapies designed to preserve muscle, extend mobility, and maintain treatment readiness while gene therapies advance. For the first time, the community has real momentum, real partnerships, and real reasons to believe that transformative treatments are within reach.

This is the story of a global movement driven by a community that refuses to be defined by a rare disease. A story of science catching up to urgency. A story of patients who deserve hope for tomorrow and the families and foundations determined to deliver it.

Written By Caroline Barber and Rachel DeConti

LGMD2D Foundation

LGMD R3/2D

Founded in 2013 by Bryan and Caroline Barber, the LGMD2D Foundation has become a vital force in the LGMD community. Together with patients, families, researchers, and partners worldwide, the Foundation works tirelessly to advance research, accelerate treatments, and bring hope to those affected by this rare disease.

Our Mission Remains Steadfast:
To expedite the development of treatments or a cure for limb-girdle muscular dystrophy, type R3/2D alpha-sarcoglycan related (LGMD2D).

The Foundation's Ongoing Work Includes:

- Maintaining the only international patient registry for LGMD R3/2D
- Funding and monitoring promising research and clinical trial development
- Providing financial support to help accelerate therapeutic progress
- Fostering collaboration between scientists, clinicians, and advocacy groups
- Partnering across the LGMD community to amplify awareness and advocacy

How did we get here? Here are the stories of two families and their journeys to LGMD advocacy.

The Barber Family

"When I grow up, I want to be a pitcher for the San Francisco Giants!" Max proudly declared at his preschool presentation. Baseball was his world – always tossing a ball with friends, family, or anyone willing to play catch. His first T-ball season felt like the start of a dream. But that's when we noticed something different: Max struggled to run.

His pediatrician initially believed physical therapy would prepare him for kindergarten and the ballfield. Yet, after six months with little improvement, concern began to grow. Quietly, our physical therapist suggested further testing. At a routine visit, our pediatrician handed me a lab slip. "Just a precaution," he said, "to rule out a few things, one of them being muscular dystrophy." He reassured me that he was 99% certain it wasn't that.

I didn't even know what muscular dystrophy was – but my gut told me I was about to find out. I called my husband, Bryan, at work. He tried to calm me, saying this was just worst-case protocol. But minutes later, he walked into the hospital lab. "I just need to be here," he said.

Below: LGMD2D Foundation's founders Bryan and Caroline Barber, with Max and Reese Barber, both Directors, Patient Advocacy

The next morning, the phone rang. It was our doctor. His voice was heavy. "Max does, in fact, have muscular dystrophy."

That was 2012. At the time, information was scarce. To understand Max's specific diagnosis, we endured painful muscle biopsies, eventually learning he had limb-girdle muscular dystrophy, type 2D (LGMD R3/2D), an ultra-rare sarcoglycanopathy.

There was no online community. No local patients. No treatments. No specialists. Just silence – and us. We felt completely alone. And that's when we decided no family should ever feel that way again.

In September 2013, after a year of relentless research and countless calls, we founded the LGMD2D Foundation.

Our mission was simple but urgent: to create a place of hope. A place where newly-diagnosed families could find answers, connect with others, and see the progress being made toward treatments and, one day, a cure.

In the early years, we focused on driving research. We connected with Dr. Jerry Mendell, a pioneer in gene therapy, who was preparing to begin human trials for LGMD R3/2D. Through the foundation's efforts and fundraising, Max became the first person in the world to receive lower-body gene therapy—a historic moment that happened, fittingly, as a replay of the San Francisco Giants 2014 World Series win blared in the operating room.

Our mission was simple but urgent: to create a place of hope. A place where newly-diagnosed families could find answers, connect with others, and see the progress being made toward treatments and, one day, a cure.

Twelve years later, the LGMD2D Foundation has grown into a beacon for patients and families worldwide.

We built the first international LGMD2D patient registry, connecting people across the globe, accelerating research, and strengthening our community. That registry has connected us with other incredible families and advocates like the DeConti Family. Rachel DeConti, Executive Director of the LGMD2D Foundation, has carried the torch forward with unwavering passion and commitment since 2022.

Left: The Barber family

Above: Dr. Jerry Mendell with Jacob DeConti

The DeConti Family

Like many new parents, my husband, Josh, and I followed every recommendation during my pregnancy – prenatal screenings, healthy habits, and regular checkups. Everything was perfect. On August 2, 2016, we welcomed our firstborn son, Jacob, into the world – a healthy, precious 8 lb, 6 oz baby boy.

Jacob hit every milestone right on time, even walking at just 11 months old. He was curious, strong, and full of life. We had no reason to think anything could be wrong.

The weekend before Jacob's fifth birthday, life as we knew it would change forever. He was outside playing with friends when he fell off his scooter. It seemed minor – he cried, brushed it off, and went right back to playing.

Later that night, as we were getting ready for his bath, Jacob went to the bathroom, and we immediately noticed his urine was dark red. Alarmed, we rushed him to urgent care, and soon after, to the ER.

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©Nikki Nicole Photography

Far Left: Jacob DeConti

Left: LGMD2D Foundation's Executive Director Rachel DeConti and family

After a night of bloodwork and monitoring, doctors told us that Jacob's CK levels were extremely high. The combination of high CK and dark red urine were indicative of rhabdomyolysis – a term we had never heard before. He was admitted to the hospital, where he spent three days on IV hydration and was discharged on his fifth birthday.

When a follow-up blood test showed another spike in CK, we were back in the hospital again. This time, doctors ordered a genetic test to "rule out" muscular dystrophy. A few weeks later, our greatest fear was confirmed: Jacob had muscular dystrophy – limb-girdle muscular dystrophy (LGMD).

We were devastated, confused, heartbroken, and desperate for answers. A month later, we met with a local neurologist who explained that LGMD isn't one single condition, but a group of subtypes. Jacob's results showed he had LGMD, type R3/2D.

That diagnosis led us on a journey to learn everything we could. We watched the 2021 International LGMD Conference, connected with the wonderful Speak Foundation, and, most importantly, met Bryan and Caroline Barber, the founders of the LGMD2D Foundation. That connection changed our family's path entirely.

We immediately got involved – offering to support the LGMD2D Foundation in any way possible. The idea that there was no treatment to slow or stop this disease was simply unacceptable to us.

Jacob was soon enrolled in Sarepta Therapeutics' Journey Natural History Study at Nationwide Children's Hospital in Columbus, Ohio, under the care of Dr. Jerry Mendell. Knowing he was part of a study that could one day help bring treatments to children like him – and that he was under the guidance of one of the world's leading LGMD experts – brought us both hope and purpose.

At first, we told Jacob the study was "Avengers Training Camp," helping him earn a place alongside his favorite superheroes – especially Spider-Man. Now that he's older, he understands more about his condition and why his participation matters. He prays daily for his "medicine" to come very soon, just as we do.

Today, Jacob is a thriving nine-year-old. He's energetic, funny, and always moving—riding his bike, climbing, and playing with his younger brother, cousins, and friends. His only restriction is avoiding competitive team sports that could strain his muscles, but that doesn't stop him from playing casually with friends. He's also part of the PGA First Tee golf program and dreams of being a pro or joining a soccer or baseball team one day.

As his mom – and as Executive Director of the LGMD2D Foundation – I'm inspired by his resilience every day. Our family channels that energy into advocacy, awareness, and pushing for treatments for LGMD R3/2D and all forms of LGMD.

Looking Ahead with Hope

Every day, Max and Jacob remind us why we fight – for them, and for every child, patient, and family affected by LGMD R3/2D. While there's still no approved treatment, incredible scientific progress is underway. With continued research, collaboration, and support, we believe that a future with treatments for our children is closer than ever. It needs to be.

Until then, we won't stop pushing forward—with hope, determination, and love leading the way.

LGMD2D Foundation
Limb-Girdle Muscular Dystrophy

LGMD2D.org

Written By Beatrice Vola
President of the Foundation GFB

The GFB Foundation

LGMD R3/2D, R4/2E, and R5/2C

The **GFB** (Gruppo Familiari Beta-sarcoglicanopatie) Foundation was established in Italy by Beatrice Vola and Marco Perlini, who have two children living with LGMD R4/2E.

Left: GFB's musical course allows people with mobility difficulties to resume musical activities they previously enjoyed but were forced to stop due to progression.

For nearly seven years beginning in 2003, we searched everywhere for information about the disease affecting our family—through different associations, conferences, doctors, websites, and social media. Everyone talked about other neuromuscular diseases, but never about the disease affecting our family. There were no foundations specifically focused on our subtype of LGMD R4/2E, and it felt as though no one was interested in this disease. As a result, in 2010, we launched GFB's website **LGMD2E.org**, and in 2013, our nonprofit organization was legally established. At first, GFB focused exclusively on LGMD R4/2E, but later we expanded our work to also include LGMD R5/2C and LGMD R3/2D.

The Search for Other Families

We started searching for other families affected by our disease because we felt alone, and finally, six years later, we found the first family who shared our children's subtype. Over time, it has become easier to locate and connect with other families by means of the internet and various social media groups.

Support for Scientific Research

We began to support scientific research because little was being done on this disease. We have always believed that systematic, targeted study of the disease is essential to improving quality of life. Since 2012, GFB has funded the preclinical phase of a gene therapy project for LGMD R4/2E in Columbus, Ohio, with Dr. Jerry Mendell—for which Sarepta Therapeutics is now in the process of applying for FDA approval.

Clinical Studies

We began clinical studies because very little data existed on patients affected by this condition. To meet this need, GFB began a natural history study on Italian patients in 2018 with Dr. Yvan Torrente at the Milan Polyclinic in Italy. The study collected retrospective data from 26 patients with LGMD R4/2E and was published in 2021.

Italian patients are few, so access to international patient data is crucial. The imminent arrival of clinical trials for LGMDs has spurred GFB to launch a worldwide study on the quality of life of patients with LGMD R3/2D, R4/2E, and R5/2C. Associations and patients must actively engage to provide researchers, doctors, and industry partners with the data needed to advance their studies of these diseases.

For these reasons, in 2021, we began the *Quality Project*, a study on patient quality of life conducted by

GFB with the support of the Milan Polyclinic. The project began with great momentum, leading to many collaborations with foundations and clinical centers.

Over the last 20 years, the GFB registry has grown: today we are in contact with more than 800 patients worldwide across three sarcoglycanopathy subtypes: LGMD R3/2D, R4/2E, and R5/2C. We strongly believe that together, we can accomplish far more!

To date, 216 patients from 44 countries have joined the Quality Project. There are 69 patients with LGMD R3/2D, 93 with R4/2E, and 54 with R5/2C. A total of 795 questionnaires have already been completed, and 29 clinical histories have been studied, spanning from the first symptoms of disease to loss of ambulation. The project's findings are currently being prepared for publication.

Currently, the GFB Foundation collaborates with the Treat-NMD network to include Quality Project data in the global registry TGDOC (TREAT-NMD Global Data Systems Oversight Committee). Anyone interested in participating in the project can email GFB Foundation at ***presidenza@beta-sarcoglicanopatie.it***.

To further advance diagnostic research, we started a new project called *Long-read Whole Genome Sequencing (WGS) of Single Hit Sarcoglycanopathy Patients* in collaboration with John Walton Muscular Dystrophy Research Centre, Newcastle upon Tyne, England. This project was initiated because six patients worldwide have been identified who carry a single pathogenic variant ("hit") in one of the sarcoglycan genes. The goal is to perform long-read whole genome sequencing on these patients to identify the elusive second variant—whether deep intronic, regulatory, copy number, or structural in nature.

This project has a dual purpose: it holds the potential to provide long-awaited genetic diagnoses for these patients, ending their diagnostic odysseys, and it will also serve as a valuable pilot project to identify unknown mutations. The project is led by Dr. Leonela Luce at the John Walton Muscular Dystrophy Research Centre, with support from GFB Foundation. We hope this work will pave the way for future large-scale long-read WGS studies aimed at resolving unresolved neuromuscular cases.

iPSCs Project

Over the years, we have diversified and expanded our field of action and interest. Beyond studying the natural history of the disease, we have developed other research projects on iPSCs (induced pluripotent stem cells) and muscle diversity. There are currently no in vitro models for LGMD R4/2E, and only one iPSC line from a patient with LGMD due to a homozygous mutation in the SGCB gene. In 2023, GFB promoted the *IPSC Generation, Characterization and Differentiation for LGMDR4-2E Patients* project with the goal of creating new tools to study the disease and evaluate the impact of emerging therapies on patient-derived cells.

This project aims to create in vitro human models of LGMD R4/2E to test potential drugs, validate treatments, and study disease mechanisms. The iPSC lines generated are now functional and ready to be differentiated by researchers working on research projects focused on LGMD R4/2E. Anyone interested in participating can submit a proposal to: ***presidenza@beta-sarcoglicanopatie.it***.

Muscle Diversity Project

In recent years, GFB Foundation has been collaborating with Dr. Sanchez Carles Riera and La Sapienza University of Rome to delve deeper into the basic mechanisms of LGMD R4/2E. In 2020, the *Muscle Diversity* project was launched. This ongoing research aims to understand why certain muscles within the same patient resist degeneration while others deteriorate, despite sharing the same genetic mutation. Furthermore, the presence of beta-sarcoglycan in other structures and tissues adds complexity to our disease but also opens a window to new treatments and alternative approaches. Dr. Riera has published an article on the presence of sarcoglycan at the neuromuscular junction and is investigating the mechanisms of inflammation and fibrosis. The project is currently looking for financial support; interested parties can contact: ***presidenza@beta-sarcoglicanopatie.it***.

Activities

Over the past 20 years, GFB Foundation has organized a wide range of activities for patients, including sporting, recreational, cultural, informative, and fundraising initiatives. The Foundation periodically holds scientific webinars, distributes newsletters, and presents its work at numerous international scientific conferences. In 2025, the Foundation began experimenting with a musical course, which allows people with mobility difficulties to resume musical activities they previously enjoyed but were forced to stop due to progression.

Through two decades of dedication, GFB Foundation continues to unite families, advance scientific research, and foster hope for a future where effective treatments and cures for LGMD R3/2D, R4/2E, and R5/2C are within reach. We thank everyone who has taken part in our projects and helped us achieve these milestones.

Beta-Sarcoglicanopatie.it

Written By Rana Abu Khadra
Co-Founder, CureSCG

CureSCG

LGMD R3/2D, R4/2E, and R5/2C

Our daughter, Sara, was born on July 7, 2015, in the United Arab Emirates. She came into our lives with laughter, light, and a boundless spirit. From her earliest days, she filled our home with joy—dancing through her childhood, drawing colorful worlds on paper, and running across tennis courts and gym floors.

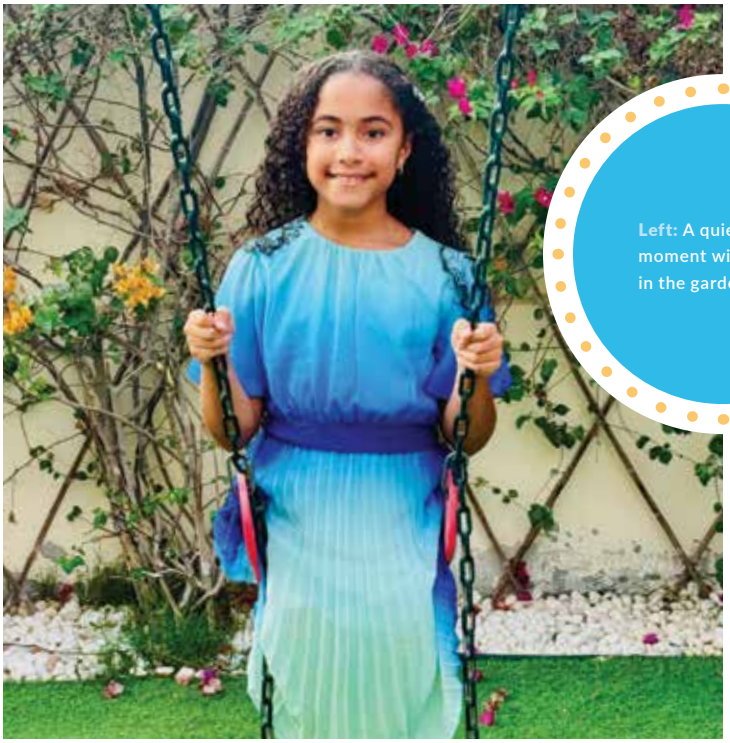
Everything changed when Sara turned eight. What began as subtle signs such as slowing down on the stairs, tripping more often, or struggling to rise from the floor soon became impossible to ignore. As parents, we knew something wasn't right. We visited one doctor after another, each reassuring us that she was simply "growing differently." But deep down, we knew there was more to her story.

Right: Sara with her parents, Rana and Harun, and her little sister Maya.

One night, everything shifted. A doctor called urgently after reviewing Sara's bloodwork, as her CK levels were alarmingly high. He asked us to see a pediatric neurologist immediately. That neurologist spoke the words no parent is ever prepared to hear, "Your daughter has muscular dystrophy."

A follow up genetic test confirmed Sara's condition as limb-girdle muscular dystrophy, type 2C (LGMD R5/2C). In that moment, our world stopped. But only for a moment. Then, instinct took over. We decided that despair would not be Sara's story, action would.

In our search for answers, we learned about the International LGMD Conference being held by the Speak Foundation in Washington, D.C. It was thousands of miles away from our home in the United



Left: A quiet, joyful moment with Sara in the garden.

Arab Emirates (UAE), but something in our hearts told us we needed to be there. We didn't know anyone. We didn't understand the medical jargon. But we went, hoping to find guidance, maybe even a glimmer of hope.

What we found was so much more. We discovered a global community bound by resilience and love, scientists dedicating their lives to rare disease research, families standing together through heartbreak and hope, and patients determined to fight for their futures. In their strength, we found our purpose.

That conference became the turning point. Out of that experience, Cure Sarcoglycanopathy (CureSCG) was born, a foundation built from one family's love, driven by one child's courage, and inspired by a community of fighters who refuse to give up.

Founded in 2024, CureSCG is dedicated to accelerating research and treatment for sarcoglycanopathies, a group of rare forms of limb-girdle muscular dystrophy.

What began as a personal mission has grown into an international effort to unite families, clinicians, and researchers across borders. We have since built collaborations with world-renowned medical institutions and genetic specialists in both the United States and the UAE, working to advance gene therapy research and support the journey toward clinical trials.

In partnership with local health authorities, we have helped coordinate the first ever LGMD clinic in the Middle East, a milestone for patients who, until recently, had nowhere to turn. Every step, every email, meeting, and late-night call is fueled by the same vision: to bring treatments and hope to those living with LGMD before time runs out. For children like Sara, every day matters.

We are still a young organization, but our determination runs deep. We are raising awareness, connecting researchers, and building bridges between patient communities around the world. Our goal is not only to find a cure but also to ensure that no family ever feels alone in this journey.

Our story is one of transforming heartbreak into hope—of parents who refused to stand still in the face of an incurable disease. Through CureSCG, we believe that the impossible can become possible when compassion meets science and when families stand together across borders and backgrounds.

Through CureSCG, we believe that the impossible can become possible when compassion meets science and when families stand together across borders and backgrounds.

Sara's laughter still fills our home. She continues to draw and paint, creating vibrant worlds that remind us why we fight so hard. Sara is an artist—a painter who inspires with every brushstroke, transforming her challenges into color, courage, and hope.

Her art has become a symbol of our mission, which is to turn pain into purpose and to help the world see that rare is powerful.

CureSCG stands for more than one family's fight. It stands for a future where every child with sarcoglycanopathy has access to hope, to treatment, and to life.

CureSCG
Cure Sarcoglycanopathy

CureSCG.org

Written By Evangelina Garcia Bava
Coordinator and founder of the LGMD Argentina Group,
Member of the Executive Committee
of the Argentine Muscular Dystrophy Association

LGMD Argentina Group

Sarcoglycans and Other LGMD Subtypes

My name is Evangelina García Bava, and I have two sons with LGMD R3/2D: Juan Andrés, age 20 and Nicolás, age 23.

In 2009, Nicolás, who had just turned seven, began to show weakness when climbing stairs and getting up from the floor. I remember the day and the time when his pediatrician told us "This could be serious." On August 21, 2009, at 6:50 p.m., our lives would change forever.

We then began the difficult journey to diagnosis. The uncertainty, the frustration, and the lack of medical knowledge about the non-specific symptoms, the need for multiple medical consultations, and the emotional impact were very stressful—both for us, the parents, and for the entire family. Our situation was further complicated by the possibility that his brother had the same condition, as his CK levels were also very high, even though he was asymptomatic at the time.

After almost three years, following genetic studies carried out in France, both of my children were diagnosed with LGMD R3/2D alpha-sarcoglycan related muscular dystrophy.

The information available at the time was more limited than that of today. I spent whole nights searching the internet for experts on the condition or trying to find other affected individuals. The journey was very long, but finally, I located the name of Dr. Jerry Mendell. I did not hesitate to write to him, and he very kindly replied to me several times. Perhaps he didn't know it, but here at the end of the world in Argentina, he was giving a desperate mother hope because I realized that there was someone doing something about this disease. This understanding marked a new beginning, and I was determined to locate more affected people in my country.

Left to Right:
The Garcîa Bava family: Alberto, Evangelina, Nicolás, and Juan Andrés.

As a result, the LGMD Argentina Group emerged in 2017, with a mission and vision aligned with the Muscular Dystrophy Association of Argentina (ADM), a non-profit civil association. Our mission is to contribute to improving the quality of life for the community of people with these diseases by providing visibility, assistance, and guidance, and promoting the development of national resources for their diagnosis, care, and treatment. Furthermore, we aim to ensure they

have guaranteed rights and access to precise and clear information for the adequate management of their disease.

Today, the LGMD Argentina Group has a presence across Latin America; we are the voice of affected individuals and their families.

Our mission is to contribute to improving the quality of life for the community of people with these diseases by providing visibility, assistance, and guidance, and promoting the development of national resources for their diagnosis, care, and treatment.

We are aware that the path to diagnosis is still very difficult, which is why we have spent the last two years developing the *Genetic Study Program* to accelerate diagnosis and maintain a reliable registry of those affected. Currently, we have a registry of over 60 people with various sarcoglycanopathies, as well as other LGMD subtypes. We know that compiling a list of these individuals is crucial for understanding the natural history of the disease, and ultimately for conducting clinical trials.

We are highly active on social media and through our website. All our programs focus on awareness, visibility, diagnosis, and support. We provide information, emotional assistance, and guidance on disability resources available within Argentina.

The Group focuses on affected individuals and their families/caregivers, running programs where we strengthen the *Help Network* and *Caring for the Caregiver*.

We have cultivated a strong community, providing trust and hope, knowing that the timing of science is not our own, but in the meantime, we must keep going, as our commitment extends beyond science.

Today we feel that the LGMD Argentina Group is thriving. We continue to work and connect with other Spanish-speaking communities, engaging in collaborative projects.

Our message is that you are not alone, that we are looking for you, and that together we are stronger. For further information or to connect with our community, please contact **lgmd@adm.org.ar**. For the present, our organization, like so many others, will continue collaborating with the scientific community to find a cure.

ADM.org.ar

Written By Scott Frewing

Kurt + Peter Foundation

LGMD R5/2C

In 2009 my wife noticed that our oldest son, Kurt, then three years old, had delayed physical abilities. After a long diagnostic path that included many false turns, doctors diagnosed Kurt with limb-girdle muscular dystrophy, type 2C (LGMD R5/2C, also called gamma-sarcoglycanopathy). Like most people, we were unfamiliar with LGMD, much less this specific form. A year later, we tested our younger son, Peter, and learned he also had LGMD R5/2C.

Above: The Kurt+Peter Foundation was formed by Kurt's and Peter's family to take affirmative action to apply promising research to LGMD R5/2C.

When doctors diagnosed Kurt and Peter, there were no available treatments, and no companies or laboratories in the United States were even researching or developing them. Although researchers had identified and described the disease in published papers, and Genethon* in France was running a small, localized gene therapy trial (that merely tested a single injection in one arm as proof of concept, with no prospect of treating the disease), no one in the United States was advancing the research toward clinical-stage development. Even some of the basic building blocks needed for drug development were not in place; for instance, the mouse model that existed at the time could not be used for testing some treatment strategies. In other words, the scientific community had done some foundational science work, but no one was pursuing translational work to create an approvable treatment.

*Editor's Note: A Spotlight article on Genethon's drug development programs appeared in the Fall 2024 Issue (Vol. 4 / Issue 4) of *LGMD News*.

We formed the Kurt+Peter Foundation in 2010, primarily to fund this necessary translational research. We wanted to encourage researchers to take multiple "shots on goal" for treatments for LGMD R5/2C. We focused on funding researchers who were willing try to apply to LGMD R5/2C approaches that were showing promise for DMD. We

We formed the Kurt+Peter Foundation in 2010, primarily to fund this necessary translational research. We wanted to encourage researchers to take multiple "shots on goal" for treatments for LGMD R5/2C.

funded development of an improved LGMD R5/2C mouse model developed by Elizabeth McNally, then at the University of Chicago. We also funded several studies demonstrating that, despite the short gamma sarcoglycan gene,


Kurt Frewing


Peter Frewing

exon skipping could deliver a functional protein in the mouse model. In addition, we supported pre-clinical gene therapy work conducted by Dr. Louise Rodino-Klapac at Nationwide Children's Hospital in Columbus, Ohio.

Building on that progress and initiatives from the LGMD program from Nationwide Children's Hospital, we joined with other LGMD families and investors to establish Myonexus Therapeutics, which was later acquired by Sarepta Therapeutics. We were also grateful to collaborate with other LGMD foundations, including projects led by Emory University and the Imperial College London, to help fund studies aimed at identifying patients.

As we funded projects, we knew the scientific and clinical development processes would be difficult. We did not anticipate, however, the magnitude of the challenges that would arise. Although exon skipping seemed promising in the laboratory, manufacturing multiple oligonucleotides at clinical scale proved prohibitively expensive to pursue as a small foundation. Moreover, continuing to pursue exon skipping became harder to justify as gene therapy appeared to gain momentum. After the gene therapy program was acquired by Sarepta,** delays and the company's recent decision to discontinue the program just as clinical trials were beginning have been deeply disappointing.

***Editor's Note:**
The previous issue of *LGMD News* (Fall 2025; Vol. 5 / Issue 4) includes an Opinion article covering the Sarepta news and its implications.

Today, Kurt and Peter are living with LGMD R5/2C as a college and high school student, respectively. The Kurt+Peter Foundation continues to seek opportunities to help fund a treatment. We are currently discussing collaborations with other foundations and institutions to pursue safe and effective next-generation gene therapies, and we are exploring opportunities to fund treatments for muscle fibrosis (replacement of muscle by connective tissue). The journey to treat LGMD R5/2C patients remains unfinished, and we look forward to working with others to complete it.

kurt+peter FOUNDATION

KurtPeterFoundation.org

Written By Joe & Courtney Dion

The Dion Foundation

LGMD R5/2C

Meet the Dion Family of Marshfield, Massachusetts: Courtney and Joe Dion, and their three children—Peter (13), Luke (11), and Maggie (9). For years, the Dions lived what seemed like a perfectly ordinary family life. Peter and Maggie hit every milestone, walking before their first birthdays and playing just like other children.

Left to Right: The Dions: Joe, Luke (11), Maggie (9), Peter (13), and Courtney

Left: Peter and Maggie were diagnosed with LGMD R5/2C at the ages of 9 and 6 respectively.

That sense of normalcy changed abruptly in the late summer of 2022. During the family's annual boating trip, Courtney noticed subtle but alarming differences in Peter's strength. "He was having trouble climbing the ladder onto the dock – something he'd done effortlessly the year before," Courtney recalls. "That night, I couldn't sleep. Something hit me: this could be muscular dystrophy."

Her instincts were heartbreakingly accurate. When a pediatric exam revealed a sky-high CK level, followed by genetic testing, Peter was diagnosed with limb-girdle muscular dystrophy, type 2C (LGMD R5/2C) – a severe, childhood-onset form of muscular dystrophy caused by mutations in the gamma-sarcoglycan gene. Months later, Maggie was diagnosed as well. Doctors at leading Boston hospitals offered little hope. "They told us, 'There's nothing we can do. See you in six months,'" Joe recalls.

A Foundation Built from Determination

After months of grieving and reflection, the Dions chose action over despair. In January 2023, they founded The Dion Foundation for Children with Rare Diseases, Inc., a nonprofit dedicated to advancing awareness, advocacy, and treatment development for ultra-rare pediatric neuromuscular diseases like LGMD R5/2C.

Through community events, partnerships, and tireless fundraising, the Foundation raised over $1 million in its first two years, supporting critical preclinical research and next-generation gene therapy studies. Yet, as Courtney explains, "We felt the weight of time every day. Starting from scratch wasn't a luxury our children had."

"We felt the weight of time every day. Starting from scratch wasn't a luxury our children had."

From Boston to Paris: Building a Bridge to Treatment

Determined to find a faster path, the Dions connected with scientists at Genethon in France and learned of promising LGMD R5/2C gene therapy work led by Dr. Isabelle Richard. That research had spun out into Atamyo Therapeutics, a Paris-based biotech nearing its first-in-human trial—but only for European patients.

The Dions didn't hesitate. "We boarded a red-eye to Paris and sat down with the Atamyo team," Courtney recalls. "Our plea was simple: there are U.S. children waiting – how can we help?"

After months of discussions and dedicated fundraising, a breakthrough came. In September 2024, The Dion Foundation and Atamyo Therapeutics announced a landmark partnership to expand Atamyo's clinical trial (ATA-200) into the United States – the first ever for children with LGMD R5/2C.

ATA-200 is an investigational, single-dose gene replacement therapy designed to restore gamma-sarcoglycan expression and improve muscle function. The ongoing Phase 1b/2 dose-escalation study evaluates the therapy's safety, pharmacodynamics, and efficacy in pediatric patients.

"We are so grateful for the opportunity to establish a partnership with Atamyo to bring this groundbreaking research to a U.S. clinical site for the first time," said Courtney Dion, Co-Founder and President of The Dion Foundation. "This is a monumental step forward for the entire LGMD community."

A Landmark Milestone in 2025

In June 2025, Atamyo announced that the first two children in the world had received ATA-200 at the Powell Gene Therapy Center at the University of Florida, under the direction of Dr. Barry Byrne, Associate Chair of Pediatrics and Director of the Powell Center.

"We are delighted to share in the success of The Dion Foundation and Atamyo's study to complete dosing of the first two participants," said Dr. Byrne. "We look forward to

evaluating the potential benefit of ATA-200 in these first children."

Atamyo's Chief Medical Officer, Dr. Sophie Olivier, added: "LGMD R5/2C is among the most severe forms of limb-girdle muscular dystrophy. We are thrilled to offer a potentially disease-modifying therapy to these young patients."

In July 2025, The Dion Foundation announced its continued commitment to fund a third U.S. patient, with Fall 2025 dosing representing the final step in completing the study's first cohort.

Advocacy Beyond the Lab: The Power of Policy

Alongside its research partnerships, The Dion Foundation has become a leading voice for pediatric rare disease policy in the U.S. When the Pediatric Priority Review Voucher (PPRV) program expired in December 2024, the Dions helped rally national support for its reauthorization. Their advocacy reached Congress – inspiring remarks from Former Congresswoman Cathy McMorris Rodgers and Representative Gus Bilirakis, who each cited the Dion family's story as a call to action.

Working in collaboration with the Biotechnology Innovation Organization (BIO), MassBio, and other advocacy organizations, the Foundation continues to emphasize the urgent need for incentives that make rare pediatric drug development possible. Maggie's heartfelt video messages – shared with lawmakers and even sent to President Trump – have become a symbol of that advocacy's humanity.

©Julie Ryan Photography

Right: Peter and Maggie at the **Par For A Cure** annual charity fundraiser.

Expanding a Movement: No Child Left Behind

In September 2025, The Dion Foundation announced its expansion, welcoming two new families united by shared purpose and determination:

- **The Bailey Family–** Brian, Chrissy, and their children Colton (10), Kennedy (8), and Berkeley (6)–of New Hampshire's Seacoast region. Following the girls' recent LGMD R5/2C diagnoses, the Baileys immediately joined the Foundation's board to raise awareness and accelerate research.
- **The Colella Family–** Grant, Alexa, and their daughter Charlotte (11) of Champaign, Illinois– became advocates after Charlotte's diagnosis, transforming their heartbreak into a mission of hope and action.

With these families onboard, The Dion Foundation's impact continues to grow – building a powerful coalition of parents, clinicians, and researchers driven by one shared vision: "No child left behind." The work is far from done. Too many children remain without access to treatments, and too many families still sit in exam rooms and hear, *"There's nothing we can do."* Together, we are working to make sure that day never comes again.

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A Conversation with Kirsten Decker

Honoring Donavon Decker

A Legacy of Advocacy, Hope, and Faith

Earlier this year, the LGMD community lost one of its most dedicated champions. Donavon Decker, who lived with LGMD R3/2D, was a pioneer in gene therapy advocacy and research, a devoted Christian, and a loving husband. His tireless commitment to raising awareness and advancing treatment progress for those living with LGMD left an indelible mark on all who knew him.

This issue of *LGMD News*—focused on sarcoglycanopathies—is dedicated to Donavon's memory and the impact of his work. As a steadfast supporter and voice for the LGMD community, Donavon inspired countless families, researchers, and advocates to keep moving the mission forward.

To honor his life and legacy, we spoke with his wife, Kirsten Decker, about Donavon's journey—from becoming the first person in the world with muscular dystrophy to receive gene therapy, to his vision for advancing treatment options and empowering the patient voice. Her reflections capture the heart of who Donavon was and the lasting difference he made.

***LGMD News:* When Donavon became the first person in the world with any form of muscular dystrophy to receive gene therapy in 1999, what did that moment mean to him and to his loved ones? How did he see his role in paving the way for others who would come after him?**

Kirsten: For Donavon and his family, 1999 marked the birth of real hope for a cure. His sisters who were also diagnosed with LGMD remember that he felt a responsibility to undergo the therapy for their sakes. Despite the risks, he saw it as an opportunity to contribute to research—something he had prayed about throughout the previous year.

He saw his role as an important and exciting step in beginning to understand the impact gene therapy could have on LGMDs and providing a foundation for others to build on. He never imagined that this moment would lead to so many opportunities for him to bring awareness about this disease and to advocate for further advancements towards a cure.

***LGMD News:* Donavon was working on creating a company, Angle Therapeutics. Can you tell us more about his vision and what he hoped it would achieve?**

Kirsten: He wanted to leverage his experience and connections to pursue a different gene therapy delivery system for LGMD to address the challenge of redosing. His decision to create Angle was shaped by the loss of friends and family to the disease and observing promising science shelved by companies, all the while experiencing the loss of his own physical strength—especially the strength to breathe.

While Donavon was a "true believer" in gene therapy as a path to restore the strength in the arms and legs, he also recognized that strengthening respiratory muscles would be a major win for improving quality of life. He was very adamant that he did not create the company for it to be lost in the shuffle of big-dollar buyouts and mergers. He believed that helping those with rare and ultra-rare diseases requires a different approach from a business standpoint—a different angle, if you will.

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***LGMD News:* What aspects of Donavon's character helped him face the challenges of LGMD and inspire those around him?**

Kirsten: Donavon persevered like nobody else I've known and did not dwell on setbacks. Instead, he'd always be looking for various means and places to advocate and move the research forward, and he did it with grace and gratitude. He was absolutely fearless. He had no qualms about asking for help of any kind in pursuit of a cure. He had a gift for creating relationships with people from all walks of life, ages, and geographies. Often, he was able to bring those people together and make bridges of support and synergy. I think he found a lot of joy in making connections with people, not only because it enriched his life, but he also saw working with others as the key to achieving meaningful progress.

He had compassion for others experiencing what he had lived through and never hesitated to reach out and help in any way he could. One day, he'd be talking with a family who was newly diagnosed, sharing his hope and advising them of resources. Another day, he'd be encouraging a friend facing the transition to a wheelchair or mourning alongside someone who had lost a family member to the disease.

Above all, he trusted Jesus with his life. This faith was the light on his path and from where he drew strength and hope on a daily basis. It was clear that Donavon was absolutely created to be a part of research. He was living his purpose every day, never wavering from where he was headed.

***LGMD News:* What do you feel is the most important legacy Donavon leaves for individuals and families living with LGMD and for the broader community of researchers and patients/advocates? In what ways would you like people to remember and honor Donavon's life and the impact he made?**

Kirsten: Donavon's legacy is perseverance. He took one step at a time, steadfastly doing what he could each day to move forward.

He quickly understood the value of including the patient voice at every point in the process because it had the power to drive positive change. This was evident in his advocacy work with the Muscular Dystrophy Coordinating Committee (MDCC), National Institutes of Health (NIH), Food and Drug Administration (FDA), and United States Department of Transportation (USDOT), as well as when he passionately shared his story with audiences both large and small, near and far. I believe he would urge others to continue educating people about what it's like to live with this disease, integrate what matters to the patient into research outcomes and care models, and ensure that decision-makers grasp the urgency.

Everybody in this community has something to bring to the table. Although these defects translate into physical weakness in a relatively small population, the combined voices and contributions of the LGMD community are incredibly strong.

To everyone in and connected to the LGMD community — scientists, clinicians, patients and their families, advocates, legislators, industry, foundations, investors, regulators, policymakers, fundraisers, encouragers, bridge-builders, whatever you've been given to do — carry the light with the same perseverance of Donavon. Work together, and make sure the voice of the patient is woven into everything you do. ▪

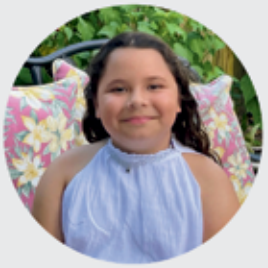
> **Consider it pure joy, my brothers and sisters, whenever you face trials of many kinds, because you know that the testing of your faith produces perseverance. Let perseverance finish its work so that you may be mature and complete, not lacking anything.** —James 1:2-4

LGMD2D Foundation

Limb-Girdle Muscular Dystrophy

These LGMD2D patients — and so many more — are in critical need of treatment.

Every day without progress means lost muscle, lost independence — and lost time.



Together, We Can Change That.

The **LGMD2D Foundation** was built from love — by families living with Limb-Girdle Muscular Dystrophy type 2D / R3 (LGMD2D) who refuse to give up hope.

Since 2013, we've been on a mission to accelerate research and clinical trials that will bring real treatments and, one day, a cure for this rare disease. Every connection and every story brings us closer to the breakthroughs our patients and families desperately need.

Be Part of the Progress Towards a Treatment.

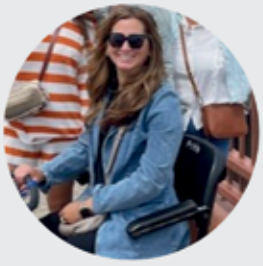
The International LGMD2D Patient Registry* is the *only* global database dedicated exclusively to those affected by LGMD2D/R3 — and it's free to join. This important database helps:

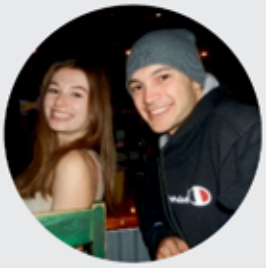
- Connect patients with clinical studies, trials, and researchers
- Give scientists the data they need to move treatments forward
- Build awareness and unite families across the world

Participation doesn't just add to our registry — it adds to hope. Together, our determination for treatments will make the difference.

*Your privacy is protected. No personal information is ever shared without your consent.

Connect with us today: lgmd2d.org





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