
Written By Melina Garza

# Becoming One Voice for Change: LGMD Day on the Hill 2024

**On September 18, 2024, I joined 18 other families and individuals impacted by limb-girdle muscular dystrophy (LGMD) in Washington, D.C., to make our voices heard.**

Our group consisted of patients and caregivers with LGMD R1/2A, R2/2B, R3/2D, R5/2C, R9/2i, R10/2J, and 1D/DNAJB6. Kathryn and James Knudson, Kelly Brazzo, Rachel and Josh DeConti and family, Joe Dion, Michell Clayton and family, Carol and Tim Abraham, Chris and Joy Carroll, Victoria Nedza, Kemi Robertson and family, Faran and Justin Day and family, Doug and Heather Wright and family, James Garner, Jennifer Levy, Kelly McCormick, John Faver, Amy Koran, Heath Gunter and myself—Melina Garza—joined together. Constituents of 12 states had the opportunity to address their elected officials with one shared goal: to advocate for legislative actions that could support those affected by this progressive disease and to remind lawmakers that we—the patients and caregivers—are central to shaping solutions.

## The Training: Learning the Legislative Process

The evening before, Thorn Run Partners equipped us with essential training, offering insights into the structure of Congressional Offices, the roles of staff, and a deeper understanding of the regulatory process for treatments for ultrarare diseases. We learned about the FDA's role and the Accelerated Approval Pathway, which can expedite treatments through surrogate endpoints. However, the FDA often underutilizes these tools, creating an urgency for us to advocate for more modern regulatory approaches.

## Presenting Critical Legislative Priorities

Equipped with an understanding of our mission, we set out the next day with a clear agenda. Our primary focus was on three key legislative bills that directly impact the LGMD community:

1. **BENEFIT Act** (H.R. 1092)
   This bill aims to ensure that patient experience data is included in the FDA's review process. We emphasized that patients, the ones directly experiencing the risks and challenges, must have their perspectives put front and center in regulatory decisions.

Melina Garza
(mom of Brooklyn Garza, age 17, LGMD R1/2A) and Kathryn Bryant Knudson

### 2 Creating Hope Reauthorization Act (H.R. 7384)

The Rare Pediatric Priority Review Voucher (PRV) Program incentivizes the development of treatments for rare pediatric diseases, with half of rare disease patients being children. Time was critical, as this program was set to expire on September 30, and we passionately voiced its importance to representatives. To our joy, we received word that the vote to reauthorize had passed that very day!

### 3 The Protecting Health Care for All Patients Act (H.R. 485)

This bill challenges the use of Quality-Adjusted Life Years (QALYs) in determining treatment value—a metric that almost inevitably undervalues the lives of those with disabilities. Extending this policy across federal programs would prevent discriminatory valuation and expand access to vital health care for individuals with conditions like LGMD.


APPLICATIONS AVAILABLE: JANUARY 1 – FEBRUARY 15, 2025

**Are You Struggling with Daily Tasks to Get Ready for Work?**

**The Personal Care Attendant Stipend Program** will offer 10 supplemental grants to employed U.S. individuals living with LGMD who need personal care assistance readying for their work days. Qualified applicants will receive a one-time, $3,000 grant per family. Do you qualify? Please visit **TheSpeakFoundation.com/grant-programs** to access the application.

**Get the Help You Need—*Apply Today!***

The SPEAK Foundation

THIS GENEROUS GRANT WAS PROVIDED BY A PRIVATE DONOR TO HELP INDIVIDUALS LIVING WITH LGMD.

**(Far Left):** Melina Garza and LGMD Awareness Foundation's Carol Abraham; **(Left):** Dr. Nicholas Johnson and Melina Garza

We also highlighted the importance of access to reliable home care. **H.R. 8110** touches on the issue but does not go far enough. Many LGMD patients face a difficult choice between employment or Medicaid access to personal care attendants, with income limits often standing in the way of essential support.

**LGMD Day on the Hill Panel**
**(L to R):** Kathryn Bryant Knudson, Dr. Nicholas Johnson, Kelly Brazzo, and Annie Kennedy

## A Sense of Purpose and Community

At midday, we attended a Senate briefing where advocate, mom, and founder of CureLGMD2i Kelly Brazzo, leading neurologist Dr. Nicholas Johnson, founder and CEO of The Speak Foundation Kathryn Bryant Knudson, and rare disease expert Annie Kennedy of the EveryLife Foundation shed light on LGMD's drug development challenges and opportunities. Hearing these passionate voices affirmed our mission. By day's end, I felt a renewed sense of purpose, camaraderie, and determination that our efforts could indeed create lasting change.

Being part of this experience underscored that every voice matters and that, together, we can make a difference. Please consider joining us at future LGMD Days on the Hill. This is just the beginning—together, we are stronger. ▪

## Participant's Feedback

*I successfully advocated for the community in an official capacity, and I am proud that 2J was one of the many subtypes in attendance that day. A win for one is a win for all of us, and we all just moved one more step (or ramp!) closer to meaningful outcomes and real hope for treatments in the near future. As I reflected on the experience, I felt like the LGMD community as a whole would be proud of us.*

**John Faver**
Patient living with LGMD R10/2J

*Elizabeth and I began our journey this past June following our middle son Henry's 2i diagnosis. The strength and unity we see in the LGMD community are both inspiring and encouraging to us. It was a privilege to advocate alongside patients and caregivers for the entire LGMD community across the country. The senators and representatives we met with were receptive and engaged in constructive discussions. I pray each day that the time and energy spent on Capitol Hill will lead to meaningful advancements in care and treatment for our LGMD community.*

**Heath Gunther**
Father of Henry Gunther, LGMD R9/2i

*We were so grateful for the opportunity to share my story on Capitol Hill, along with our group of incredible Pennsylvania constituents! To get the chance to be vulnerable and put a face to this disease, in front of the very people who have the ability to pass these legislations and gain the attention of the FDA, is an experience that we will never forget!*

**Christopher Carroll**
Patient living with LGMD R3/2D

*Having 3 children with LGMD, we jumped at the chance to advocate for them and the community on Capitol Hill. The work is just beginning, but we know we had a positive impact on the staffers, and we will continue to push for a cure. Our kids left with a different perspective on their place in all of this, and I think we have at least two rare disease lobbyists in the making.*

**Heather Wright**
Mom of Walker, Reagan and Rex, LGMD R1/2A

**LGMD Day on the Hill** is a program that gives individuals and families living in the United States the opportunity to advocate in person with congressional representatives in Washington, D.C. You can also be a delegate in the 2025 LGMD Day on the Hill event. Our selection process occurs via an online application. Please see our upcoming 2025 Spring issue of *LGMD News* for more information.