



Meet the Panelists

By Carol Abraham and Michele Wrubel
Coalition to Cure Calpain 3

The LGMD Coalition is grateful to the EL-PFDD panelists for sharing their stories. We hope they inspire you to reflect upon the issues you would like to share about living with LGMD and encourage you to participate. By writing in with comments (before or during the meeting), responding to live polling, and/or phoning in during the September 23rd livestream event, our united voices will make a difference!

SESSION 1



Rania

Age: 35 ▪ LGMD2E/R4
Orlando, Florida

"It's a remarkable feeling to have the reach and influence to speak with a governing body that could drastically change the quality of my life."

- **First Symptoms:** I waddled when walking, had difficulty climbing steps, tired easily, and needed a chair to lean on to get up from the floor.
- **If I Were Cured Tomorrow...** I would pack my suitcase, go to the airport, and take the next available, international flight.
- **Fun Fact:** I have been on a hot air balloon ride!



Kurt

Age: 16 ▪ LGMD2C/R5
Menlo Park, California

"I am excited to participate in the EL-PFDD to provide insight about what it's like living with LGMD2C."

- **First Symptoms:** I would army crawl (age 2).
- **If I Were Cured Tomorrow...** I would get a job working in a professional kitchen.
- **Fun Fact:** I am obsessed with cooking.



Peter

Age: 14 ▪ LGMD2C/R5
Menlo Park, California

"I am excited about the opportunity

to share my experience with LGMD2C with other patients and the FDA."

- **First Symptoms:** I tired more easily than my classmates.
- **If I Were Cured Tomorrow...** I would go to Disneyland!
- **Fun Fact:** I like to code and build giant LEGOs.



Elizabeth

Age: 42 ▪ LGMD2C/R5
Holland, Michigan

"If sharing my story of living with a rare neuromuscular disease helps in future research that may lead to finding treatment or a cure, I am happy to take part."

- **First Symptoms:** I was a toe-walker, had difficulty getting up from the floor, and easily fatigued (age 7).
- **If I Were Cured Tomorrow...** I would love to be able to live independently!
- **Fun Fact:** I love crafting, coffee, and books!



Donavon

Age: 59 ▪ LGMD2D/R3
Andover, Kansas

"I want to tell the FDA my family's story."

- **First Symptoms:** I was not able to run very fast (age 10).
- **If I Were Cured Tomorrow...** I would drive.
- **Fun Fact:** I was the first person in the world to undergo gene therapy for any form of MD.

SESSION 2



Dan

Age: 57 ▪ LGMD2I/R9
Longmont, California

"There is an urgent and unmet need to find effective treatments and therapies for LGMD."

- **First Symptoms:** I was a toe-walker (toddler).
- **If I Were Cured Tomorrow...** I would hike into my old, secret fishing spot and fish all day long.
- **Fun Fact:** I learned to fly sailplanes when I was 15.



Jane

Age: 25 ▪ LGMD2I/R9
Charlotte, North Carolina

"This is something all patients should be excited about, as it is a huge step for the LGMD community. I am honored to have been chosen to represent our close-knit community in front of the FDA and am hopeful for our future."

- **First Symptoms:** I had trouble keeping up with friends on the playground and consistently used the same leg to climb stairs instead of alternating (age 4-5).
- **If I Were Cured Tomorrow...** I would start by taking my dog for a run. Then, I would go out with friends without stressing about accessibility or the daunting task of transferring out of my chair to use the restroom.
- **Fun Fact:** My eyes are two different colors.

9/23/22

LIVE
EVENT

10:00AM (EDT)



DON'T MISS YOUR CHANCE!

You must pre-register in order to participate by signing up at:

www.lgmdpfd.com



Brooklyn

Age: 15 • LGMD2A/R1
Pflugerville, Texas

"I'm very excited to share my story and hope it will make a difference."

- **First Symptoms:** I could not support my weight with my arms and kept falling while trying to learn to do a cartwheel (age 9).
- **If I Were Cured Tomorrow...** I would go on a hike at Yellowstone National Park.
- **Fun Fact:** I LOVE listening to music and going to concerts!



Carol

Age: 61 • LGMD2A/R1
Twin Lakes, Wisconsin

"I am grateful to have this opportunity to share my LGMD journey with the FDA and other key stakeholders. No one knows the effect of how these progressive neuromuscular diseases impact our lives better than those who live with these conditions."

- **First Symptoms:** I walked on my toes and had difficulty keeping up with my peers (age 5).
- **If I Were Cured Tomorrow...** I would run up to my husband, wrap my arms tightly around him, and give him a huge hug and kiss! Then, I would go running on a sandy beach.
- **Fun Fact:** In 2001, I received the "Female Waterskier of the Year" Award from Rehabilitation Institute of Chicago!

SESSION 3



Patrick

Age: 49 • LGMD2E/R4
Salem, New Hampshire

"I am happy and excited to share my story and meet others!"

- **First Symptoms:** I displayed weakness while running in gym class, ran on my toes, and lost balance while jumping and running (age 11).
- **If I Were Cured Tomorrow...** I would play my drums again.
- **Fun Fact:** I have driven across the U.S. twice, visiting 38 different states. My bucket list is to visit all 50.



Paul

Age: 65 • LGMD2C/R5
Napa, California

"Once I realized God has my back, I was no longer depressed about having this disease."

- **First Symptoms:** I had symptoms at birth, like choking and paralysis. My parents thought I was going to die as an infant.
- **If I Were Cured Tomorrow...** I would RUN!
- **Fun Fact:** I love cooking. I'm a big foodie!



Jacob

Age: 6 • LGMD2D/R3
Newington, Connecticut

Jacob's mom (Rachel):

"As a caregiver, I am honored to be a part of the LGMD Coalition and this meeting for the LGMD community. My hope is with events like these, more treatment options will be available for patients like my son, sooner."

- **First Symptoms:** Jacob was diagnosed after a case of rhabdomyolysis (which causes dark urine when overly active) and his legs hurt and are sore (age 5).
- **If I Were Cured Tomorrow...** We would let Jacob be as active as he wanted – running, jumping, climbing – for as long as he wanted, with a little less worry.
- **Fun Fact:** Jacob LOVES Spider-Man! For his Kindergarten graduation, he said that he wants to be him when he is older!



Samantha

Age: 14 • LGMD2I/R9
Lancaster, Pennsylvania

Sammy's mom (Kelly):

"It is important for us to show the FDA how our lives have been affected on so many levels by this progressive disease. I am excited for the FDA to see the strength of our amazing LGMD community, and I pray that this will help to successfully bring treatments to the market more efficiently."

- **First Symptoms:** Sammy had trouble keeping up with peers, fell often, had difficulty getting up from the ground, and she had a waddling gait/hip sway (age 2).
- **If I Were Cured Tomorrow...** I would want to go to the beach, run through the sand and into the ocean, jump in the waves, and not worry about falling over or getting sea legs when I get out. That would be amazing.
- **Fun Fact:** I really love watching basketball (my dad is a coach), and I love cooking all kinds of savory foods and sweets.



Kathryn

Age: 49 • LGMD2I/R9
Tallahassee, Florida

"Be joyful in hope, patient in affliction, and faithful in prayer." – Romans 12:12

- **First Symptoms:** I had rhabdomyolysis starting at six years of age.
- **If I Were Cured Tomorrow...** I would go to the beach and spend all day at the ocean. Then I would go to Italy and hike everywhere!
- **Fun Fact:** I competed as "Miss Chesterfield County" in the Miss Virginia pageant!

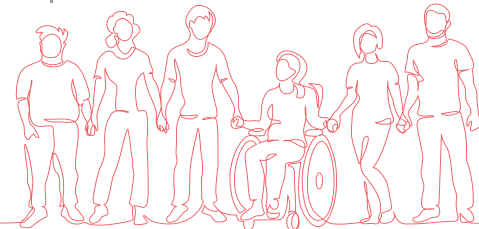


Andrew

Age: 41 • LGMD2A/R1
Canterbury, U.K.

"It's a real honor to be involved in this project."

- **First Symptoms:** I struggled to get up from the floor (age 19).
- **If I Were Cured Tomorrow...** I would walk with my son on the beach.
- **Fun Fact:** My tongue has grown quite large because of my muscle disease. Try sharing an ice cream with me now!



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