AUGUST 2025 | ISSUE 2

THE AAVENGER

Dear AAVenger Community,

As we welcome the summer season, we're energized by the growing reach of our mission and the deepening of our connections across the globe. At AskBio, our commitment to patient-centered gene therapy continues to evolve through meaningful partnerships and an unwavering focus on empowerment.

This issue reflects that progress. We're proud to highlight our ongoing commitment to patient communities, including a spotlight on Melissa Grove, an LGMD2i patient and advocate whose voice and experience continue to inspire.

From clinical updates to expert insights and myth-busting education, this issue of *The AAVenger* brings together stories, science, and shared purpose. Thank you for being part of this journey. We're honored to walk it with you.

Pooja Merchant, MD, MPH
Vice President, Medical Affairs, Global Head of External Engagement & Public Affairs

FRESH FROM THE FRONTLINES OF SCIENCE: LATEST NEWS

AskBio Presents Complete Results of Phase 1 Trial of AB-1002 Gene Therapy in Participants with Congestive Heart Failure at European Society of Cardiology Heart Failure Meeting

AskBio Announces Publication of Complete Results of Phase 1b Trial of AB-1005 Gene Therapy in Participants with Parkinson's Disease in Movement Disorders

BREAKING NEW GROUND IN HEART FAILURE

At AskBio, we're harnessing the power of AAV gene therapy to fundamentally change how cardiovascular disease is treated—by addressing the underlying cause, rather than just managing symptoms. Our investigational therapies deliver functional genes directly to heart tissue, with a focus on areas of high unmet need, including congestive heart failure (CHF). In partnership with WomenHeart, we're helping to raise awareness about the potential of AAV gene therapy in cardiovascular care through educational efforts, including a recent webinar that explored both the science and patient experience. Watch a replay of the webinar here!

AskBio's AskFirst™ is a collaborative program for advocacy groups, patients, and their families who may benefit from the latest advances in gene therapy research and potential curative therapies. It's part of AskBio's commitment to patient-first care and increasing trial accessibility.

SCIENCE, SIMPLIFIED: AN EDUCATIONAL SERIES ON AAV GENE THERAPY

This Issue: Debunking 3 Common Myths About AAV Gene Therapy

Let's set the record straight on some common misconceptions.

Gene therapy is one of the most exciting frontiers in medicine today—but with innovation comes questions, confusion, and sometimes myths. At AskBio, we believe in empowering patients and families with clear, honest information. Here's a closer look at what AAV gene therapy really is—and what it *isn't*.

❌ **Myth 1: Gene therapy changes your DNA permanently.**
✅ **Truth:** AskBio's AAV gene therapy does **not** permanently alter your DNA. Unlike some other types of gene editing technologies, AAV vectors usually remain outside the cell's chromosomes. That means they can deliver lasting benefits without making permanent changes to your genetic code.

❌ **Myth 2: If I get the placebo in a clinical trial, I'm out of luck.**
✅ **Truth:** Some gene therapy trials include the option for participants in the control group to receive treatment after a certain period. That means more people may eventually access the therapy, even if they were randomized to the control group first.

❌ **Myth 3: Cell therapy and gene therapy are basically the same.**
✅ **Truth:** While they can overlap, cell therapy and gene therapy are distinct. Cell therapy gives a person new, healthy cells - either from themselves or a donor - to replace or support damaged ones, while gene therapy fixes or replaces faulty instructions (genes) inside a person's cells to help them work properly.

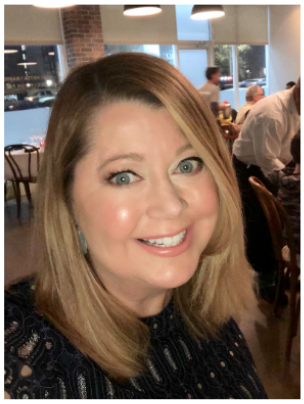
At AskBio, we're committed to scientific transparency and patient-first innovation. If you have questions about gene therapy, clinical trials, or our AAV platform, email us at *askfirst@askbio.com*, we're here to help!

IN THEIR WORDS: VOICES FROM THE COMMUNITY

Finding Strength in Community: Melissa Grove on Life with LGMD2i

How has your personal journey with LGMD influenced your advocacy or professional work?

I was diagnosed at 18, but didn't meet anyone else with LGMD2i for 25 years. For those 25 years, my neurology appointments were always the same—they'd just say, "We don't know, there's nothing we can do for you." At a certain point, they told me about a conference for my subtype at the University of Iowa, the Iowa Wellstone Dystroglycanopathies Patient and Family Conference. That was a powerful moment—being in the same room with other people who had the same issue I did. Finding my community changed everything. I'd spent my career in HIV and mental health, so I knew the power of group support—but it was different experiencing it for myself. With my background and experiences, I knew I had talents that could be useful—as a therapist, and as the executive director of a large healthcare nonprofit. Now I speak at conferences and advocate on Capitol Hill, bringing attention to both science and the psychosocial side of rare disease.



Patient Spotlight: Melissa Grove, Former Executive Director of Legacy Counseling Center, Inc.

What kind of progress or change would you most like to see in the LGMD2i community?

I would love to see more clinical trials and potential treatment options, as well as more clarification on treatment guidelines—because there's not an official treatment guideline. I know CureLGMD2i and the Speak Foundation are working on it, but so many people with LGMD2i don't know about the cardiac and pulmonary issues they should be looking into and paying attention to. It's just not commonly known, sometimes even among experts in neurology.

What advice or encouragement would you offer to someone newly diagnosed with LGMD2i?

Don't panic—progress is happening. Join Facebook groups, connect with others, and don't be afraid to reach out privately. Some of the best support I've received came from people living it—not doctors. So many questions my doctors couldn't answer for me were answered by people with lived experience. Sometimes doctors say, "Don't confuse your Google search with my medical degree." To which I say, "Well, don't confuse your medical degree with my 57 years of lived experience." There are things only those with lived experience can truly understand, from assistive devices to relationships and caregiver planning. Community makes all the difference. We're all there to support each other in good times and bad, and that's incredibly powerful.

What's one thing you wish more people understood about living with LGMD2i?

Muscular dystrophies are not one-size-fits-all, LGMD alone has 30 subtypes. They each have their own specific problems and concerns. For example, with LGMD2i, there are cardiac and pulmonary issues that differ from other types of LGMD. We need more awareness of these distinctions.

What keeps you hopeful or motivated on the harder days?

A sense of humor and community. I always say—if you were training for the Olympics, you'd work out every day. Living with a rare disease requires that same mental training. Resilience can be built through therapy, support groups, education, and balance. When I was first diagnosed, I didn't tell anyone—I needed time to process. But over time, I found strength in connecting with others and using my experience to help. There are things you can do to build your resilience and your knowledge base—and learn how to cope. Put your energy into that. When you're newly diagnosed, you may feel like you don't want to tell anyone. I didn't want to tell my family. I knew I needed to figure it out myself before I could deal with others' emotions. But as you gain experience living with it, you realize there are other people you can be supporting—and you can build a tribe of people who can support you in turn.

SUPPORTING PATIENTS EVERY STEP OF THE WAY: EXPANDING NAVIGATOR SERVICES ACROSS ASKBIO CLINICAL TRIALS

Navigating clinical trials can feel overwhelming—especially for patients and families facing progressive or rare diseases. That's why AskBio offers a dedicated Patient Navigator service to help individuals explore potential eligibility, understand what to expect, and feel supported throughout the clinical trial process.

Launched in 2023, the Patient Navigator program initially focused on clinical trials for Multiple System Atrophy (MSA) and Limb-Girdle Muscular Dystrophy (LGMD2I/R9). In recent years, it has grown to include Parkinson's disease, with more to come as our pipeline grows.

Trained Patient Navigators provide personalized, one-on-one guidance for patients and caregivers, answering questions, offering education, and helping individuals make informed decisions about participation.

Powered by our trusted external partner, myTomorrows, our AskFirst™ Patient Navigator initiative allows patients to engage directly through AskBio's trial pages and Patient Navigator access points. The goal is simple: to make clinical research more approachable, more human, and more accessible to those who need it most.

Whether you're considering a trial for yourself or supporting a loved one, our Patient Navigator service is here to help guide you. This service is available via our clinical trial websites and below through the "Clinical Trials Corner" section of the newsletter, where QR codes and direct links connect patients to the appropriate support channels.

"We are making gene therapy trials more accessible. In partnership with AskBio, we are working to bring clinical trials closer to the people who need them most. At myTomorrows, supporting patients is at the heart of everything we do. Our dedicated Patient Navigators listen with empathy, build trust, and guide patients and their families—offering clarity, support, and partnership every step of the way."

- Madeleine Carrier, Senior Patient Navigator

CLINICAL TRIALS CORNER

The Clinical Trials Corner provides a direct link to the latest AskBio clinical updates for each disease, including trial phase and status, recruitment updates, key milestones, and resources to learn more. Scan the QR codes below to explore each program further or click on the icon.

LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2I/R9

CONGESTIVE HEART FAILURE

MULTIPLE SYSTEM ATROPHY

PARKINSON'S DISEASE

LATE-ONSET POMPE DISEASE

Not currently recruiting

Learn more about our AAV gene therapy clinical trials.

DID YOU KNOW?

AAV gene therapy has the potential to produce long-lasting effects from just a single dose—especially in tissues like muscle and brain where cells don't divide frequently. That's why it's being studied for diseases like Pompe, Parkinson's, and LGMD.

MEMORIALS

In Memory of Tiffany House

It is with heavy hearts and profound sadness that we share the passing of Tiffany House.

Tiffany was a force of love, resilience, and advocacy. Her strength was rooted in her deep love—for her family, her friends, and the Pompe community, to which she dedicated so much of herself. That same love was returned by many who were touched by her spirit, her kindness, and her fierce determination.

In Memory of Maryze Schoneveld van der Linde

It is with deep sorrow that we share the passing of Maryze, who left us peacefully on Wednesday, May 14. Maryze was a remarkable individual whose impact on the Pompe Community reached across the globe. Tirelessly devoted to advocating for patients, she gave of herself selflessly—often putting the needs of others ahead of her own. Her legacy is one of compassion, strength, and relentless commitment to making life better for others.

RECENT & UPCOMING CONFERENCES

AskBio is a proud partner and exhibitor of these conferences. Please stop by and say hello!

ADVOCACY

Iowa Wellstone Patient and Family Conference
June 20-21, 2025 · Iowa City, IA

Speak Foundation International LGMD Conference
July 18-21, 2025 · Orlando, FL

Davis Phinney Victory Summit
August 12-13, 2025 · Golden, CO

Michael J. Fox Foundation Parkinson's Disease IQ & You · September 6, 2025 · Nashville, TN

AHA Heart Walk
September 27, 2025 · Kansas City, MO

SCIENTIFIC

European Academy of Neurology (EAN)
June 21-24, 2025 · Helsinki, Finland

Advanced Therapeutics in Movement & Related Disorders Congress (ATMRD) · June 27-30, 2025
Washington, DC

European Society of Cardiology - (ESC)
August 29 - September 1, 2025 · Madrid, Spain

Heart Failure Society of America (HFSA)
September 27-29, 2025 · Minneapolis, MN

ATMRD Advanced Therapeutics For Movement & Related Disorders™ Congress

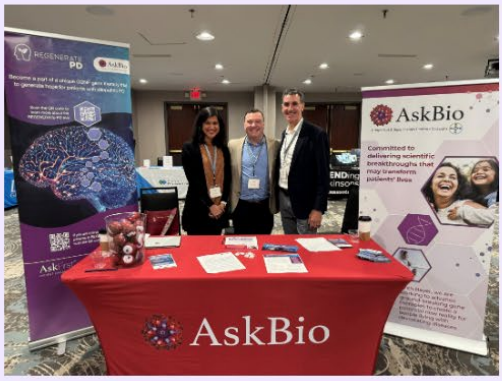
DAVIS PHINNEY Foundation For Parkinson's every victory counts®

HFSA HEART FAILURE SOCIETY OF AMERICA

The SPEAK Foundation

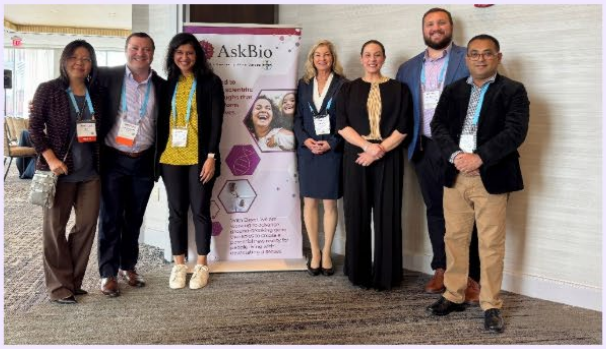
COMMUNITY CONNECTIONS

Michael J. Fox Foundation Parkinson's Disease IQ & You



Highlighting AAV gene therapy for the treatment of CHF at ESC-HF Congress

AskBio Team Puts Patients First at the Mission MSA International Conference



RESOURCES & SUPPORT

CLINICAL TRIALS
For more information
please visit
www.askbio.com/gene-therapy-clinical-trials

NEWSLETTER
Sign up to get our latest
patient news at
www.askbio.com/patient-advocacy

LET'S TALK
Email us at *askfirst@askbio.com*
if you would like to share your story or have a topic you'd like to learn more about.
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www.askbio.com/privacy

AskBio

A Bayer Cell & Gene Therapy Platform Company