AskBio™

The AAVenger™

ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

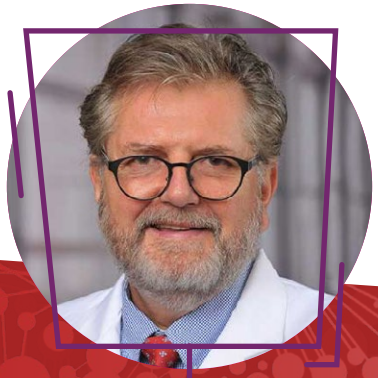
FALL 2024 | VOL.11

ASKBIO RECEIVES FDA FAST TRACK AND MHRA INNOVATION PASSPORT DESIGNATIONS FOR AB-1005 INVESTIGATIONAL GDNF GENE THERAPY FOR PARKINSON'S DISEASE

- AB-1005 (formerly known as AAV2-GDNF) is being studied for the treatment of patients with moderate Parkinson's disease
- AskBio is currently enrolling patients in its phase II REGENERATE-PD trial in the United States
- European Union and United Kingdom clinical trial sites planned to open later in 2024

Bayer AG and Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the United States (U.S.) Food and Drug Administration (FDA) has granted Fast Track Designation for AB-1005, which is being developed for moderate Parkinson's disease. AB-1005 has also been awarded the Innovation Passport, the United Kingdom Medicines and Healthcare products Regulatory Agency (UK MHRA) innovative medicine designation, for the treatment of Parkinson's disease.

CLICK HERE TO READ THE FULL ANNOUNCEMENT



"These designations clearly underscore the importance of developing innovative therapies for those living with Parkinson's disease, where a significant unmet need still exists. They further highlight the willingness of key regulatory bodies to support the accelerated development of AB-1005 with a focus on the potential benefit to patients."

KRYSTOF BANKIEWICZ, MD, PHD | SCIENTIFIC CHAIR, PARKINSON'S AND MSA | ASKBIO

Have You Been Diagnosed with **Parkinson's Disease (PD)?** Do You Know or Care for Someone With PD?

Contact Us

Thank you for your interest in the investigational REGENERATE-PD study. Your participation in this study may help scientists and physicians better understand PD and how to improve treatment. Your participation may contribute to the medical field and may make a positive impact on the lives of people affected by PD. **Learn more here>>**

For more information, please contact AskFirst@askbio.com or scan the QR code below:

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HOW PATIENT ADVOCACY IS STRENGTHENING CONNECTIONS AND IMPACT

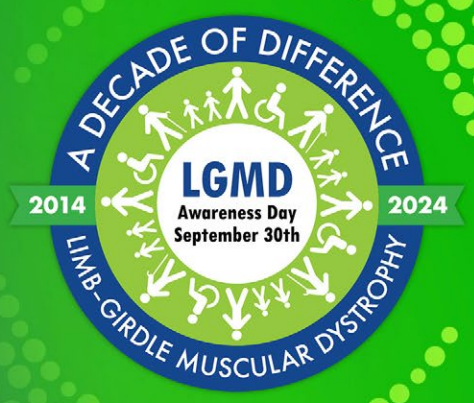
Key Initiatives Boosting AskBio's Community Engagement:

AskBio's Patient Advocacy team had a busy and exciting first half of 2024!

The team engaged externally with 25 patient advocacy groups (PAGs) across AskBio's therapeutic areas, including Cardiovascular, Parkinson's disease (PD), multiple system atrophy (MSA), limb-girdle muscular dystrophy (LGMD), and Pompe disease. "Our advocacy partners are some of our greatest collaborators and are crucial in helping to prioritize clinical candidates, establish research protocols, and advance technological innovation," said Margaret Heim, Medical Affairs. In addition, the team also sponsored or participated in nearly 20 face-to-face patient events highlighting our gene therapy educational activities and our latest clinical trial information. "We support the community by connecting with patients and advocacy groups in meaningful ways," Kara Witcoff, Patient Advocacy. "One way we do this is through education and by supporting organizations focused on increasing awareness of these diseases and research to develop potential treatments." One example of education and awareness is through our Employee Engagement Committee events, like our Rare Disease Day All Hands in February where patients, advocates, and caregivers shared their experience living with Pompe disease with our colleagues. We are humbled by and grateful for the support of the many organizations and individuals who support us in working to advance gene therapy research and clinical outcomes to impact people's lives.

CONGRATULATIONS TO THE LGMD COMMUNITY ON CELEBRATING A DECADE OF DIFFERENCE AS THE 10TH ANNUAL LGMD AWARENESS DAY IS ON SEPTEMBER 30TH.

AskBio has recognized this day for the past few years and this year we are featuring an LGMD community family and a Patient Advocacy Group, the LGMD Awareness Foundation. In honor of this month, we are also proud to support the Speak Foundation's Congressional Hill Day and the CureLGMD2i Foundation's annual Connecting for a Cure event.



The Norton Family

A LIMB-GIRDLE MUSCULAR DYSTROPHY (LGMD) COMMUNITY INTERVIEW WITH CRAIG, JODI, BRODY AND CJ NORTON FROM FAIRFAX, VA

When meeting Brody, 18 and CJ, 16, one immediately recognizes young men eager to fit in and excel at life. They are smart, well-mannered teenagers who approach the world with a positive outlook and a strong support system of friends and a loving family. The brothers' bond runs deeper than most siblings; they carry the burden of an ultra-rare genetic disorder, Limb-Girdle Muscular Dystrophy (LGMD) 2I/R9.

Craig and Jodi, when did you first notice something was off?

"When Brody was 15 months old, he had a virus that wouldn't go away. Each day he became weaker. When we noticed he had a floppy neck, we took him to our local children's hospital. We saw a neurologist who determined that he had elevated CPK levels and asked if muscular dystrophy ran in our family; it does not. Brody spent the next four nights in the ICU where they took a muscle biopsy, which came back with the diagnosis of LGMD2I." Jodi was pregnant with CJ at the time but could not fathom they would have two sons with the same ultra-rare condition. The risk for two carrier parents to pass the gene and, therefore, have an affected child, is 25% with each pregnancy.

How has LGMD personally affected your family?

"Life has continued to become more challenging for Brody and CJ as muscle deterioration takes place in their shoulders, pelvis, and upper arms and legs. Despite their struggle to walk, participate in sports or perform normal activities as their teenaged peers do, Brody and CJ have a group of loyal friends who never shy away from lending a helping hand and filling in as secondary caretakers. On a daily basis, we worry about what's next with each and every step, and the anxiety that comes with that is overwhelming. They've handled it with a lot of grace, but it's difficult. It was hard enough being an able-bodied teenager without LGMD."

What do you wish people knew about living with LGMD?

"At a rather young age, Brody and CJ noticed they could not run fast, and as time passed, their symptoms have progressed to the point where running is no longer possible, and both just hope to make it from class to class each school day. CJ often uses a scooter to get around while Brody still manages to walk on his own, though impaired. The unfortunate solution is mobility enhancement – scooters and wheelchairs. Because of LGMD, they are not capable of doing things like getting out of bed, stepping up a curb or making it up a flight of stairs. Anything to intercede in their progression is all we can hope and pray for."

How important is it to find new treatments for LGMD?

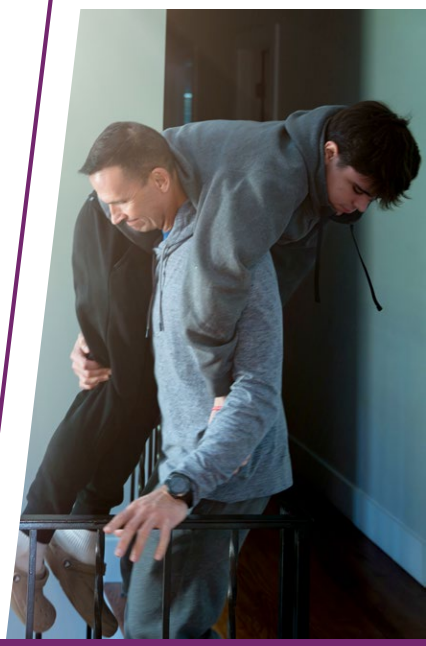
Brody and CJ could not be more transparent about the challenges that LGMD has brought to their lives. Brody says with remorse, "Living with LGMD is challenging, to say the least. I watch all my friends do the things I can't, and it makes me wonder why not me?" CJ adds, "I wish I could wrestle or do one sport. I feel I'm missing out. I want to be able to go somewhere without having to worry what I'll do if I fall." Yet even as Brody and CJ face the daunting task of getting out of bed on their own each day without a future treatment, let alone a cure, they hold their heads high with hope and optimism.

What are your hopes for the future?

"We know the path ahead will not be easy. As parents, we must endure watching the deterioration of the boys' physical abilities unless a treatment is discovered. The opportunities for their lives are closing when, at this stage, they should be widening. We put a lot of eggs in this basket of hope. To those working on a treatment for LGMD, time is of the essence. We have been waiting for years. We keep hearing something is on the horizon, but we are desperate for anything that might improve their daily lives. Given the level of anxiety we feel and experience each day, I would never sleep through the night without the hope that a cure is around the corner!"

For more information about LGMD, visit the **CureLGMD2iFoundation**, the **LGMD Awareness Foundation**, or the **SPEAK Foundation**.

To learn more, please visit **AskBio.com**, email **AskFirst@AskBio.com**, scan the QR code or go to **https://clinicaltrials.gov/study/NCT05230459**.



Do You or Someone You Know Suffer From **Congestive Heart Failure (CHF)?**

Contact Us

Your participation in this study may help scientists and physicians better understand CHF and how to improve its treatment, potentially with AB-1002 gene therapy. Your participation may contribute to the medical field and make a positive impact on the lives of people affected by CHF.

GenePHIT

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ASKBIO IS A PROUD SPONSOR OF THE FOLLOWING CONFERENCES!

ESC
August 30–September 2, 2024 | London, UK

Defeat MSA Alliance Annual Patient & Family Conference
September 7–8, 2024 | Virtual

LGMD Congressional Hill Day
September 18, 2024 | Washington, DC

Michael J. Fox Foundation PDIQ & You
September 21, 2024 | Carlsbad, CA

CureLGMD2i Foundation Connecting for a Cure
September 21, 2024 | Philadelphia, PA

International Congress of Parkinson's Disease and Movement Disorders
September 27–October 1, 2024 | Philadelphia, PA

HFSA Annual Scientific Meeting
September 27–30, 2024 | Atlanta, GA

Mission MSA Path to a Cure
October 3–6, 2024 | Virtual

Michael J. Fox Foundation PDIQ & You
October 19, 2024 | Charlotte, NC

ESGCT
October 22, 2024 | Rome, Italy

MDA-Engage Community Education Symposia
November 2, 2024 | Palo Alto, CA

AHA Heart Walk
November 2, 2024 | Philadelphia, PA

Parkinson's Foundation Moving Day NC Triangle
November 2, 2024 | Raleigh, NC

AHA Scientific Sessions
November 16–18, 2024 | Chicago, IL

CLINICAL TRIALS

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

NEWSLETTER

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