

# The AAVenger™

ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

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Paul and Carol Ehman

## A PARKINSON'S COMMUNITY INTERVIEW WITH PAUL AND CAROL EHMAN, PITTSBURGH, PA

### Paul, when did you first notice something was off?

Carol and I were driving home from Florida to our quiet town of Derry, Pennsylvania when my thumb began trembling uncontrollably as I held on to the steering wheel. It was on that drive that we knew something wasn't right, and we needed to schedule a visit to the doctor. As Carol recalls, Paul called me from the doctor's parking lot to tell me

he was diagnosed with Parkinson's disease; we just couldn't believe it. We came home and researched all the information we could find, and that led us to a neurologist, a movement specialist. After that visit in 2018, we transformed our worst fears into a plan for managing symptoms and putting energy into embracing life no differently than prior to the diagnosis. We chose to live life with the positive outlook and vibrant energy that we've always had.

### How has Parkinson's personally affected your family?

I spent 38 years as a millwright. I provided maintenance for heavy equipment at a large specialty steel operation producing airplane landing gear, helicopter rotors and jet engine components, spending my entire career working with my hands and taking on home repairs until retiring in 2009. We thought about the progressive, neurodegenerative nature of the disease that causes weakening or loss of nerve cells in the brain that control movement. My initial reaction was, would I be able to continue golfing, doing yard work – the simple day-to-day things that you take for granted? And where would I be a couple of years down the road? Above all else, I didn't want to be a burden to Carol or my kids.

### What do you wish people knew about living with Parkinson's?

That when you have Parkinson's, basic foundations to a healthy life take on new importance. At 71, I wake up each morning to begin an exercise routine that includes a boxing workout with a speed bag, time on the treadmill, and a healthy number of push-ups. I feel the rigid impact on my muscles and the tremors that continue to progress as each day passes. The pain often radiates up and down my right arm like a low voltage current, moving down to my right leg, and sometimes there's a slight migration to my jaw and lower lip. I sort of stutter with words where I used to be more proficient.

### How important is it to find new treatments for Parkinson's?

We're optimistic about innovative treatments and the potential that a gene therapy cure is on the horizon. In the meantime, I'd advise anyone not to give up on their daily routine; exercise or do whatever is necessary to find a better frame of mind, as I'm confident a cure is coming sooner than later.

### Carol, what are your hopes for the future?

I worry about what our future holds, but Paul has a great attitude and doesn't let Parkinson's get the best of him. Paul's my hero!



# 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.



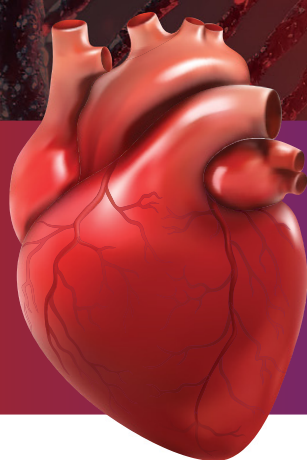
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**You or your loved one may be eligible for a clinical study evaluating a novel gene therapy to treat CHF.**



## **ASKBIO PATIENT SUPPORT & ENGAGEMENT COMMITTEE**

**Led by Jenny Siferd, Chair, Employee Engagement Committee and Heather Caro, US Lead, Patient Support and Engagement Committee**

We're thrilled to share exciting updates from the AskBio Patient Support and Engagement Committee, which officially launched on October 3, 2023. This employee-led initiative embodies our commitment to not only advancing scientific innovation but also to supporting and engaging with the communities we serve. The focus of this committee is primarily to organize and participate in advocacy activities and fundraising events devoted to supporting patients and their families. We also directly aid patients affected by rare diseases through amplifying their voices and helping them share experiences during educational and awareness events.

AskBio volunteers have already been involved in several meaningful events, such as awareness walks

supporting Huntington's Disease and caregiver-focused initiatives, with many more events planned for 2024. The aim is for the Patient Support and Engagement Committee to serve as a vital bridge, aligning closely with the therapeutic areas in AskBio's pipeline to ensure that our efforts resonate deeply with patient advocacy communities. We recognize that the true impact of AskBio extends far beyond our laboratories and scientific achievements. As we continue to grow and evolve, AskBio remains committed to strengthening our partnerships and making a meaningful difference in the lives of patients and their families.

Heather Caro, Alliance Management, Business Development



Jenny Siferd, Talent Attraction, HR

# Now Recruiting

## ASKBIO MSA PHASE 1/2 CLINICAL PROGRAM



Learn more about our actively recruiting study at [Multiple System Atrophy \(MSA\) Clinical Trial - AskBio](#), or connect with us directly at [askfirst@askbio.com](mailto:askfirst@askbio.com).

At AskBio, bringing the potential for life-changing advanced gene therapeutics to patients with diseases that have a high unmet medical need fuels our research and development pipeline.

AskBio's approach to potentially treating multiple system atrophy (MSA) uses a glial cell-line neurotrophic factor (GDNF) gene therapy that takes advantage of the brain's natural production of the GDNF protein, which is required for the development and maintenance of dopamine brain cells. These brain cells are typically lost in MSA patients. Our goal with AB-1005 is to potentially promote the survival and function of dopamine producing brain cells, which may lead to significant motor function recovery for MSA patients.

**MSA-101 is a randomized Phase 1/2 clinical trial evaluating the safety and potential effects of AB-1005 in people with multiple system atrophy-parkinsonian type (MSA-P).**

- AB-1005 is a one-time gene therapy delivered surgically into the brain to provide a continuous expression of the GDNF protein
- Eligible participants have a 2 out of 3 likelihood of receiving active treatment versus placebo
- Participants randomized to placebo will undergo minimal surgery and may be offered the gene therapy product after the main part of the study
- AskBio is only able to include US resident participants at this time

*AB-1005 is an investigational therapy and has not been approved by the U.S. Food & Drug Administration (FDA) or any other health authority.*

Have You Been Diagnosed With **Parkinson's Disease (PD)** or **Multiple System Atrophy (MSA)**? Do You Know or Care for Someone With These Diseases?

### Contact Us

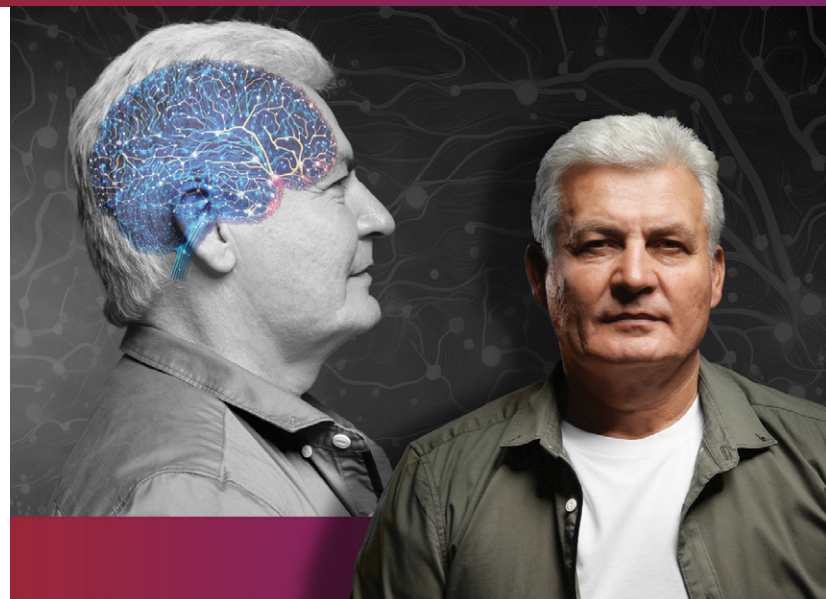
Your participation in this study will help scientists and physicians better understand PD and MSA and how to improve their treatment. Your participation will contribute to the medical field and may make a positive impact on the lives of people affected by these diseases.

- You or your loved one may be eligible for the PREGENERATE study, which will observe and prepare patients for the opportunity to be evaluated for enrollment into a future gene therapy clinical trial for PD or MSA
- The goal of the PREGENERATE study is to better understand how PD and MSA progress in patients by learning more about the symptoms and biological indicators of each disease



For more information, please contact [AskFirst@askbio.com](mailto:AskFirst@askbio.com) or scan the QR code

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# Do you or does someone you know have LGMD2I/R9?



## AskBio will be conducting a clinical study of an investigational gene therapy for individuals with a confirmed genetic diagnosis of LGMD2I/R9.

- This is a one-time intravenous infusion of gene therapy designed to produce fukutin-related protein (FKRP) in the body, primarily in muscle.
- Part 1 of the study will assess the safety of LION-101 only in adults (aged 18 to 65 years).
- This is a randomized, placebo controlled, double-blind study.
- The study is designed to investigate at least two different doses of LION-101 versus placebo.
- The initial phase of this first-in-human dose-finding study will be conducted in the US.
- Travel to study sites may be reimbursed; local and home-based testing will be used when possible.
- Information on the clinical trial can be found on [clinicaltrials.gov](https://clinicaltrials.gov).

To learn more, please visit [AskBio.com](https://AskBio.com), email [AskFirst@AskBio.com](mailto:AskFirst@AskBio.com) or go to [clinicaltrials.gov](https://clinicaltrials.gov) (NCT05230459)

## ASKBIO IS A PROUD SPONSOR AND EXHIBITOR OF THE UPCOMING CONFERENCES. PLEASE STOP BY OUR BOOTH AND SAY HELLO!

### The OhioHealth Movement Disorders Program and The Columbus Parkinson's Retreat

April 6, 2024 | Powell, OH

### Parkinson's Foundation Western Pennsylvania Living Well Conference

April 13, 2024 | Pittsburgh, PA

### American Academy of Neurology

April 13–18, 2024 | Denver, CO

### MJFF Unity Walk

April 27, 2024 | New York City, NY

### AMDA/IPA International Patient and Scientific Conference

May 3–5, 2024 | San Antonio, TX

### ASGCT

May 7–11, 2024 | Baltimore, MD

### European Society of Cardiology

May 11–14, 2024 | Lisbon, Portugal

### HDSA Annual Convention

May 30–June 1, 2024 | Spokane, WA

### New Directions in Muscle Biology and Disease Conference

June 23–26, 2024 | Ft. Lauderdale, FL



### CLINICAL TRIALS

For more information please visit [www.askbio.com/gene-therapy-clinical-trials](https://www.askbio.com/gene-therapy-clinical-trials)



### NEWSLETTER

Sign up to get our latest patient news at [www.askbio.com/patient-advocacy](https://www.askbio.com/patient-advocacy)



### LET'S TALK

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