

THE AAV^{EN}GER

ADVANCING
GENE THERAPY
TOGETHER



Dear AAVenger Community,

Just as the season turns, progress in gene therapy continues to evolve. In this fall edition of The AAVenger, we provide updates on the latest developments in gene therapy and patient support, highlighting clinical breakthroughs that have the potential to make a real impact on people's lives.

This edition highlights promising research in investigational and potential new treatments for congestive heart failure, aimed at improving patient outcomes and quality of life. Additionally, it explores ongoing clinical trials and recent advances in Parkinson's disease to expand treatment options and promote long-term care.

By connecting patients with personalized resources and expert insights, we aim to make the journey to care more straightforward.

Pooja Merchant, MD, MPH

Vice President, Medical Affairs, Global Head of External Engagement & Public Affairs

FRESH FROM THE FRONTLINES OF SCIENCE: LATEST NEWS

AskBio Announces Publication in Nature Medicine of 12-month data from Phase 1 Trial of AB-1002 Gene Therapy in Participants with Congestive Heart Failure

AskBio Presents Interim Safety Results from Phase 1/Phase 2 LION-CS101 Clinical Trial of AB-1003 in Participants with Limb-Girdle Muscular Dystrophy 2I/R9

AskBio Announces 6 Presentations at the European Society of Gene and Cell Therapy 32nd Annual Meeting

AskBio Announces Completion of Enrollment in Phase 1 Clinical Trial of AB-1005 Gene Therapy for Multiple System Atrophy-Parkinsonian Type (MSA-P)

First European Participants Randomized in AskBio Phase 2 Gene Therapy Trial of AB-1005 for Parkinson's Disease

EXPLORING NEW HORIZONS IN HEART FAILURE TREATMENT: A DISCUSSION WITH DR. AHMED

In this insightful webinar, Dr. Mustafa Ahmed, a leading expert in advanced heart failure, delves into the potential of gene therapy to transform the treatment landscape for congestive heart failure (CHF). He discusses how AskBio's investigational gene therapy, AB-1002, aims to address the underlying causes of heart failure by targeting specific proteins involved in heart muscle function. Dr. Ahmed provides an overview of the GenePHIT Phase 2 clinical trial, designed to evaluate the safety and efficacy of this one-time investigational gene therapy in patients with advanced heart failure. To learn more, watch the full webinar [here](#).



Mustafa Mohammed Ahmed, MD



Congestive heart failure (CHF) is a major cause of morbidity and mortality affecting over 64 million people worldwide¹ and approximately 6.7 million Americans over the age of 20.²

IN THEIR WORDS: VOICES FROM THE COMMUNITY DAVIS PHINNEY FOUNDATION FOR PARKINSON'S

The REGENERATE-PD Trial: Unpacking Gene Therapy for Parkinson's

Insights from Dr. Mark Richardson

In a recent webinar, Dr. Mark Richardson discussed the REGENERATE-PD trial, an investigational gene therapy study for Parkinson's disease. Dr. Richardson explained how this approach differs from traditional treatments, which primarily focus on managing symptoms, by targeting the underlying dysfunction in the brain's dopamine pathways. Gene therapy uses viruses like adeno-associated virus (AAV) that leverage the virus' unique ability to enter affected cells. AAV delivers a therapeutic gene to replace missing or defective genes, or turn off bad genes, helping treat diseases at their source. Using a specialized viral vector, the therapy aims to deliver genes that help neurons produce essential support factors, potentially slowing disease progression and improving motor function over time. While this therapy is still investigational and not a cure, the trial represents a significant step forward in expanding treatment options for people with Parkinson's. Listen to the discussion in full [here](#).

For more expert insights and up-to-date information on Parkinson's disease, visit the Davis Phinney Foundation website to explore blog articles on topics such as: "[Will Gene Therapy Deliver a Parkinson's Breakthrough?](#)"



Mark Richardson, MD, PhD

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.**



AB-1002, the AAV investigational therapy in the GenePHIT trial, aims to transfer a gene that codes for protein inhibitor-1c (I-1c) that improves how the heart stores and uses calcium, which is essential for healthy heart function.

RECENT & UPCOMING CONFERENCES

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

ADVOCACY

Parkinson's Assoc. of the Rockies E3 Conference
September 20, 2025 • Denver, CO

Parkinson Foundation Western PA
Step Forward Walk
September 27, 2025 • Pittsburgh, PA

110 Fitness Patient Symposium
October 17, 2025 • Rockland, MA

Parkinson's UK Par-Con
October 24-25, 2025 • Birmingham, United
Kingdom

Michael J. Fox Foundation's Boston Run/Walk
October 26, 2025 • Boston, MA

Michael J. Fox Foundation's Houston Run/Walk
November 15, 2025 • Houston, TX

SCIENTIFIC

International Parkinson and
Movement Disorder Society (MDS)
October 5-9, 2025 • Honolulu, HI

Neurocme Movement Disorders Review 2025
October 18, 2025 • Chicago, IL

German Neurology Congress
November 12-15, 2025 • Berlin, Germany

PSG 2025 Annual Meeting
December 4-6, 2025 • San Diego, CA

Neurocme Movement Disorders Review 2025
December 6, 2025 • Orange County, CA

UK MND Clinical Studies Group



COMMUNITY CONNECTIONS

Big Science, Bigger Smiles: AskBio at MDS



Every Step Counts: AskBio at Step Forward Parkinson's



Connecting, Learning, and Leading at HFSA



Advancing Knowledge, Empowering Patients: 110 Fitness Parkinson's Symposium 2025



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

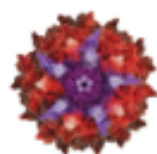
www.askbio.com/patient-advocacy



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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AskBio

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1. GBD 2017 Disease and Injury Incidence and Prevalence Collaborators. Lancet. 2018;392(10159):1789-1858.
2. Tsao CW, et al. Circulation. 2023;147(8):e93-e621.



For questions about our pipeline or participating in a trial: AskFirst@askbio.com.

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