

THE AAV[®]ENGER

ADVANCING
GENE THERAPY
TOGETHER



Dear AAVenger Community,

We're excited to bring you this refreshed edition of the AAVenger, where we continue our commitment to keeping you informed and connected in the world of AAV gene therapy. Each quarter, we bring you updates, insights, and personal stories that highlight the progress we're making together.

In this issue you'll hear from Jimmy Choi who is living with Parkinson's disease, and his wife and care partner, Cherryl. Dr. William Ondo will provide his take on the value of clinical trials, and you'll get the latest updates on AskBio's clinical trials and research advancements. You'll also be introduced to our educational series on AAV gene therapy, exploring its potential to transform lives. We hope you find this newsletter valuable and engaging as we work to advance research and make a meaningful impact on individuals and families affected by both genetic and non-inherited diseases.

Thank you for being part of our community—we're grateful to share this journey with you!

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

FRESH FROM THE FRONTLINES OF SCIENCE: LATEST NEWS

AskBio Receives FDA Regenerative Medicine Advanced Therapy designation for Parkinson's disease investigational gene therapy

First Participant Randomized in Europe in AskBio Phase 2 Gene Therapy Trial for Congestive Heart Failure

First participants randomized in AskBio Phase II gene therapy trial for Parkinson's disease

AskBio Advances Gene Therapy Clinical Trial for Limb-Girdle Muscular Dystrophy Type 2I/R9 with Dosing of First Participant in Second Cohort



AskBio was the first company to produce adeno-associated viral (AAV) gene therapy for the brain.¹

SCIENCE, SIMPLIFIED: AN EDUCATIONAL SERIES ON AAV GENE THERAPY

What is AAV gene therapy?

AAV gene therapy has the potential to address a wide range of conditions caused by faulty or missing genes, that can be influenced by genetic or environmental factors. You have thousands of working genes in every cell in your body. Each of these genes contains a set of instructions for the cell like a blueprint, which guides your body to make the proteins or building blocks needed to function normally. 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.

The virus has been stripped of its original viral genes, so it can't replicate or cause infection. Instead of integrating into your DNA—which could potentially disrupt genes—it usually stays outside the chromosomes in a stable form. This often allows the treatment effect to last for a long time, especially in cells that don't divide a lot, like muscle or nerve cells. That said, everyone's immune system is different. Some people may have immune reactions to the virus, which could impact how well the treatment works. We check for that before treatment and monitor carefully afterward.



IN THEIR WORDS: A PATIENT'S JOURNEY

Living with Parkinson's: Jimmy and Cherryl Choi's Story

Jimmy Choi, an endurance athlete and Parkinson's advocate, has refused to let his diagnosis define him—pushing through challenges to complete marathons, set world records, and inspire others. By his side every step of the way is his wife, Cherryl, who has embraced the role of a PACER—partner, advocate, cheerleader, and source of empowerment—helping him and the Parkinson's community fight for a brighter future.

Jimmy, what led to your diagnosis?

Honestly, I didn't think much of my symptoms. I chalked it up to everyday life—stress, fatigue, aging. It wasn't until I had a physical for life insurance that a nurse noticed something wasn't right. She worked in a neurology office and suggested I see a specialist. That was the moment everything changed. Without her, I might have gone years without answers.

How has Parkinson's affected your family?

For our kids, having a dad with Parkinson's is just normal. They've grown up knowing what the disease is and, more importantly, how to live with it. They understand when to let me rest, when to step in and help, and when to call for backup. It's shaped their awareness of others with disabilities too—they see things differently, with more empathy. I think that's an unexpected silver lining.

Cherryl, what has been your experience as a caregiver?

It's a journey that constantly changes. Some days, Parkinson's feels like just another part of life, and other days, it's overwhelming. The hardest part is watching someone you love go through something you can't fix. But, Jimmy is incredible—he's always pushing forward, finding ways to adapt, and showing our kids that resilience is everything.

Jimmy, what do you wish people knew about living with Parkinson's disease?

Most people think Parkinson's is just tremors, but it's so much more. It's classified as a movement disorder, but that barely scratches the surface. There are cognitive and emotional effects—depression, apathy—and even everyday struggles like losing your sense of smell or dealing with severe constipation. What surprises many people is that around 30% of Parkinson's patients don't even have tremors. It's different for everyone, which makes understanding and treating it so complex.

What advice would you give to others living with Parkinson's?

You have to take ownership. Your mindset makes a huge difference in how you manage the disease. The people who live well with Parkinson's are the ones who stay active, advocate for themselves, and build a strong support network. You can't just sit on the sidelines—you have to make time for Parkinson's and take control of your journey.



Cherryl and Jimmy Choi

You competed on American Ninja Warrior. How did that journey begin?

Honestly? It started with denial. When I was diagnosed, I focused on making sure my family was financially secure—I thought that was all I could do. But then, I found exercise. I started challenging myself, learning what I needed to do to live well instead of just waiting for symptoms to get worse. Ninja Warrior was part of that journey. It wasn't about competing—it was about proving to myself that Parkinson's wasn't going to define me.

What's one thing about Parkinson's that might surprise people?

People often say, "You don't die from Parkinson's, you die with it." But quality of life matters—and for some, it can mean life or death. Apathy and depression can make it hard to find reasons to keep pushing forward. Parkinson's is a debilitating disease, and it's frustrating when research efforts aren't always prioritized because "it's not fatal." But imagine living with this for 40, 50+ years. That's the reality for so many of us.

How important is it to find new treatments?

It's everything. Right now, the standard of care we have was discovered in the 1960s. Think about how much technology has advanced since then, and yet, we're still relying on a treatment that's over 60 years old. There's hope, though. Research is moving forward but it takes time. We need better options, faster.

INSIGHTS FROM THE EXPERTS

The Power of Clinical Trials

Clinical trials are the backbone of medical progress. Without them, new treatments would never reach the patients who need them most. Clinical trials are the only way to gain FDA approval for new medications.

By comparing potential treatments against placebos or some other control, these studies determine whether a therapy is safe and effective, ensuring that medical advances are backed by rigorous scientific evidence.

One particularly exciting frontier in Parkinson's disease research is gene therapy. Unlike traditional treatments that manage symptoms, gene therapies can target the root cause of disease. Dr. Ondo explains that this approach involves delivering a therapeutic gene into affected cells, enabling the body to produce essential proteins to fight the disease.

While new treatments are emerging, personalized medicine remains a critical part of Parkinson's care. No two patients experience the disease the same way—symptoms, age of onset, and progression all vary widely. Dr. Ondo emphasizes that tailored treatment plans, including combinations of existing medications and emerging therapies, will continue to improve patient outcomes.

For those considering participation in a clinical trial, Dr. Ondo offers a compelling reminder: without volunteers, new treatments cannot be investigated, approved, or made available. By joining a clinical trial, patients play a direct role in shaping the future of Parkinson's treatment—offering hope to themselves and generations to come.



William G. Ondo, MD

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.



AskBio was the first to clone AAV for
therapeutic purposes.²

UPCOMING CONFERENCES

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

ADVOCACY

NeuroChallenge Expo - March 8, 2025 -
Bradenton/Sarasota, FL

Michael J. Fox Foundation Parkinson's Disease IQ &
You - March 15, 2025 - Orlando, FL

Muscular Dystrophy Association (MDA) Clinical &
Sci Conference - March 16-19, 2025 - Dallas Fort
Worth, TX

Mission MSA Capitol Hill Day
March 25, 2025 - Washington, DC

Duke Health & United Pompe Foundation
April 5, 2025 - Durham, NC

Parkinson's Foundation Western PA Living Well
Conference - May 3, 2025 - Pittsburgh, PA

Mission MSA International Conference
May 9-11, 2025 - Cambridge, MA

Michael J. Fox Foundation Parkinson's Disease IQ &
You - May 17, 2025 - Minneapolis, MN

SCIENTIFIC

American College of Cardiology (ACC)
March 29-31, 2025 - Chicago, IL

American Academy of Neurology (AAN)
April 5-9, 2025 - San Diego, CA

Heart Failure Update 2025
May 2-3, 2025 - Toronto, Canada

Association of British Neurologists (ABN)
May 7-9, 2025 - Liverpool, UK

International Association of Parkinsonism and
Related Disorders (IAPRD) - May 7-10, 2025 - NYC,
NY

8th International Workshop for Glycosylation
Defects in Muscular Dystrophy
May 7-9, 2025 - Charlotte, NC

American Society of Gene & Cell Therapy (ASGCT)
May 13-16, 2025 - New Orleans, LA

European Society of Cardiology - Heart Failure
(ESC-HF) - May 17-20, 2025 - Belgrade, Serbia

Duke

THE MICHAEL J. FOX FOUNDATION
FOR PARKINSON'S RESEARCH

American Academy
of Neurology

MISSION MSA

Neuro Challenge
EXPERIENCE THE CHALLENGE

COMMUNITY CONNECTIONS

IN CASE YOU MISSED IT



The 8th Annual Neuro Challenge Foundation for Parkinson's Expo - March 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.
Please review AskBio's Privacy Notice at
www.askbio.com/privacy



AskBio

A Bayer Cell & Gene Therapy Platform Company

