AskBio™

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ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

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ASKBIO RECEIVES FDA FAST TRACK DESIGNATION FOR AB-1002 INVESTIGATIONAL GENE THERAPY PROGRAM IN CONGESTIVE HEART FAILURE

- AB-1002 is being studied for the treatment of adults with non-ischemic cardiomyopathy and New York Heart Association (NYHA) Class III heart failure symptoms
- AskBio is currently enrolling patients in the Phase II GenePHIT trial of AB-1002 for the treatment of congestive heart failure (CHF)

Bayer AG and Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for the AB-1002 program. AB-1002 is an investigational one-time gene therapy that is administered to the heart with the intention of helping to promote the production of a constitutively active form of protein inhibitor 1 (I-1c) designed to block the action of protein phosphatase 1. Inhibiting the function of this protein, which is linked to congestive heart failure (CHF), could potentially lead to a therapeutic effect on the heart.

CLICK HERE TO READ THE FULL ANNOUNCEMENT

> “The FDA Fast Track Designation for AB-1002 is an important accomplishment for the clinical development of this program and highlights our goal of potentially bringing effective treatments to patients with advanced congestive heart failure. We look forward to completing our Phase II GenePHIT clinical trial, which is currently enrolling patients with severe heart failure, and are committed to exploring the full potential of AB-1002 for the treatment of this devastating disease.”

CANWEN JIANG, MD, PHD | CHIEF DEVELOPMENT OFFICER AND CHIEF MEDICAL OFFICER | ASKBIO

A CONGESTIVE HEART FAILURE (CHF) COMMUNITY INTERVIEW WITH DARLENE ANITA SCOTT, RICHMOND, VA


Darlene Anita Scott

The day before Darlene Anita Scott was diagnosed with congestive heart failure, she ran ten miles. A whole foods vegan training for her seventh marathon, she was considered healthy by most metrics. Since her surprise diagnosis, Darlene has committed to maintaining a purposeful life that includes a little less running and a lot more rest.

When did you first notice something was off?

Symptoms of heart failure may be subtle, and many believe that being overly tired with an inability to manage a lot of exertion may be a result of a lack of sleep or too much work. I was consistently tired and found myself with breathing difficulties, congestion, and leg swelling. As an athlete, I brushed it off for as long as I could, but eventually I just knew something was not right, so I was able to seek treatment while still in the early stages of the disease. I remember when my doctor delivered the news of my diagnosis and hearing the words “heart failure.” I was forty-one; that had devastating implications. I was shocked because I thought I was healthy, so my other reaction was fear. I always associated heart failure with death, but I can still run, I just can’t run as far nor as fast as I once did.

How has CHF personally affected you and your family?

Despite the challenges that CHF presents, I still exercise, determined to keep up a healthy pace in my long-distance run against CHF. I make it a priority in life and express myself through my passion for the visual arts and prose; my attempt to convey optimism and courage. I have an ICD implant (an internal defibrillator), a pacemaker, and take a myriad of medications to manage my CHF symptoms. As my symptoms progress, I learn how to adjust. I still teach daily as an English professor, but my routine has changed, and I require more rest, but it does not stop me from being the best version of myself.

What do you wish people knew about living with CHF?

The emotional impact, like the low hanging cloud over my head that comes and goes at will. I’ve had to make various lifestyle changes and, in addition to the implanted devices, a surgical procedure to optimize device performance and a daily regimen of medications, I’ve enlisted the support of a therapist. The advice I receive from that therapist and outreach to others who have CHF or similar conditions have been lifesavers.

How important is it to find new treatments for CHF?

Without a cure for CHF, the progression of the disease is different for each patient with varying forms of treatment to manage symptoms. Life expectancy can vary depending on disease severity and, for some patients, the only recourse is a heart transplant. Regardless of what phase of CHF a patient is experiencing, the continued weakening of the heart and the ensuing life altering symptoms are unavoidable. CHF is a disease that literally takes your breath away. My advice for others with CHF is the same for researchers working on better treatments and ultimately a cure for heart failure… Just keep going.

What are your hopes for the future?

I’ve kept busy as a writer, visual artist and university professor for the past 20 years. My hope is that with the rapid advancements in science, I (and the rest of the CHF community) will have at least another 20 more!

For more information about CHF, visit **WomenHeart**, the **Heart Failure Society of America (HFSA)** or the **American Heart Association**.

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.

Gene**PHIT**

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

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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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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.

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

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

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

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.

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

ASKBIO SPONSORED THE FIRST EUROPEAN LGMD2I COMMUNITY CONFERENCE

May 25, 2024 | Amsterdam, Netherlands

ASKBIO IS A PROUD SPONSOR OF THE UPCOMING CONFERENCES.

Advanced Therapeutics in Movement & Related Disorders® Congress
June 22–25, 2024 | Washington, DC

New Directions in Muscle Biology and Disease Conference
June 23–26, 2024 | Ft. Lauderdale, FL

The Dystroglycanopathies: Iowa Wellstone Center Patient and Family Conference
July 12–13, 2024 | Iowa City, IA

CLINICAL TRIALS

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

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