AskBio™

# The AAVenger™

ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

FALL 2023 | VOL.7

## ASKBIO ANNOUNCES FIRST PATIENT DOSED IN PHASE 1 / PHASE 2 TRIAL OF AB-1003 GENE THERAPY FOR LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2I/R9 (LGMD2I/R9)

### Gene Therapy Developed to Restore FKRP Enzyme Activity, Primarily Inside Muscle Cells, for the Treatment of LGMD2I/R9.

"Hearing that the first patient has been dosed in this study evaluating AB-1003 is an exciting moment for the limb-girdle muscular dystrophy community and individuals living with this debilitating disease," said Kelly Brazzo, Co-Founder & CEO, CureLGMD2i, and mother (pictured middle-back) to Sammy, living with 2I (far left in photo).

CLICK HERE TO READ THE FULL ARTICLE

## 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 (ages 18 and 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 or go to clinicaltrials.gov (NCT05230459)**

Jamie Davis, Rock Artist

# AN INTERVIEW WITH JAMIE, POMPE STRONG ROCK ARTIST

**AskBio Medical Affairs – Patient Advocacy Met Jamie at the Duke Health & United Pompe Foundation's Annual Adult Pompe Disease Patient Meeting.**

### How did you become involved with the Duke Health conference?

When I was in my early thirties, I wore heels every day, but it became difficult to walk and, even without my shoes, the stairs presented a challenge. My balance was off, and I took multiple falls, plus initially attributed my symptoms to weight gain. In 2012, my symptoms progressed so I started my diagnostic journey at a walk-in clinic! Through strength testing they referred me to a neurologist who diagnosed me with a type of muscular dystrophy. However, lacking confidence in his diagnosis, I was sent to Virgina Commonwealth University Medical Center (VCU) Neurology where they did genetic testing, and in 2013 I received my Pompe diagnosis. Through Facebook, I found several online communities; that's where I discovered Duke and have attended the health conference ever since.

### How has Pompe personally affected you and your family?

I was a single mom of four and the disease didn't affect me as much when they were younger. As they grew older, I bore the brunt of the disease. It was debilitating at times which led me to miss out on numerous events and I couldn't be there for them. That's my biggest sense of loss, just enjoying moments with my children. I simply didn't have the energy to participate.

### What do you wish people knew about living with Pompe?

With or without a muscular disease, not all struggles are physical. There's a huge mental, emotional aspect to Pompe. My greatest symptom isn't visible on the outside, it's my emotional struggle with the disease. It makes me feel very sad. I've always been empathetic but what I've learned from Pompe is that I feel for people a lot more and am happy to be a support for others.

### What led you to become a Rock Artist?

In 2017, because of Pompe, I had to stop working and came across a global movement called Kindness Rocks: **thekindnessrocksproject.com**. We paint rocks and leave them in public places as a token of kindness or inspiration for others to find, in the hopes that it will brighten their day! I joined my local chapter in the Richmond area called RVA Rocks. I have more patience than talent because I find the entire process very calming and therapeutic; I can sit and work on one small rock for hours. I never in my wildest dreams thought I would have a craft room, but I created one, it's my sanctuary. When we were at the peak of Covid, I painted quarter-sized rocks with cartoony flowers. When I'd go in for my infusions, I lined the windows of the parking deck with those little flowers. I wanted to plant happiness!


### How important is it to find new treatments for Pompe?

It's extremely important. That's one reason I go to the Duke conference every year. I've always had an interest in new innovations; I want to know what treatments and clinical trials are on the horizon and would be open to one in the future.

### What are your hopes for the future?

Once I was diagnosed and became involved in the Facebook community support groups, they referenced elevated liver enzymes and it reminded me that my younger brother (I'm one of six children) during routine blood work, had elevated liver enzymes. Sure enough, he tested positive for Pompe and has been on treatment ever since. Last year, my younger sister gave birth, and since Pompe was recently added to newborn screening in Virginia, my nephew was flagged for Pompe. My hope for the future is that Pompe will be identified early and that treatments will become available that can halt progression of the disease. My body's done a lot for me. My body brought four children into the world. My body is dealing with a muscle disease and is still supporting me. Every day is a gift, so I need to treat my body better! Though I live with Pompe, Pompe isn't everything. We're people with dreams just like everyone else, with hope for a future without Pompe. **@jahmay1234 on Instagram**

# 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 (also known as AAV2-GDNF-MSA) 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 chance 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.*

## ASKBIO TECHNOLOGY AND MANUFACTURING

Many great ideas don't reach their full potential in the process of translating them into products – we're all still waiting on flying cars and the final books of the Game of Thrones series. Gene therapy is no different – and as the adeno-associated virus (AVV) technologies that underpin our therapeutics gain momentum, the world is starting to take notice. Life-changing therapeutics may be developed, but in some cases not scaled up to meet demand, leaving patients and families looking at medicine that is out of reach because of supply constraints, cost, or access. Making safe, effective gene therapy medicines is exceptionally challenging in its complexity, and for much of the industry the focus was on concept, not what it would take to move forward if the concept worked. For AskBio, though, thanks to long years of work that was in many ways ahead of its time, we can be confident not just in the concept but the full story of development and hope of manufacturing incredible future medicines.

AAV depends on other viruses to be present in order to replicate – a great advantage in terms of safety, but a real challenge when the job is to make as much of it as possible. Through years of research (significant portions of which were done by AskBio's scientific leadership, Jude Samulski, PhD, Chief Scientific Officer) a number of methods have been developed to create the right conditions for AAV to be produced – some use other viruses, and some methods, like AskBio's manufacturing platform, combine a receptive cell type with the genetic elements AAV needs encoded on linear or circular DNA that are then put inside those cells. AAV virus is then produced, purified, and made ready for the patient. Along the way, a quality panel that consists of more than 30 different tests are run to ensure safety and efficacy.

While these manufacturing methods have been well understood for many years, AskBio has been a leader in making these methods ready for patients that need large amounts of AAV. People like our Chief Technology Officer, Josh Grieger, worked to isolate productive cells and put them in growth medias that meet modern standards for manufacturing. And we're continuing to innovate, optimizing our current methods while developing new ones with the goal of making gene therapy more safe, more accessible and available at a lower cost. AskBio's manufacturing partner Viralgen, a fully integrated Contract Development and Manufacturing Organization (CDMO), allows our AAV production technology to be more accessible to the industry from development to commercial manufacture.

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

**Defeat MSA Alliance**
September 22–24, 2023 | Virtual Conference

**CureLGMD2i Foundation – Connecting for a Cure**
September 30, 2023 | Conshohocken, PA

**Heart Failure Society of America Annual Scientific Meeting**
October 6–9, 2023 | Cleveland, OH

**The MSA Coalition – Friends & Family**
October 7, 2023 | Virtual Conference

**Michael J. Fox Foundation – Parkinson's IQ & You**
October 14, 2023 | Columbus, OH

**Australian Pompe Association & New Zealand Pompe Network – Family Support Forum**
October 20–22, 2023 | Virtual Conference

**Parkinson's Foundation – Moving Day Miami**
October 21, 2023 | Miami, FL

**Huntington's Disease Society of America Eastern Pennsylvania Chapter – Team Hope Walk**
October 22, 2023 | Philadelphia, PA

**ESGCT – Annual Congress**
October 24–27, 2023 | Brussels, Belgium

**International Limb-Girdle Muscular Dystrophy Conference**
October 27–29, 2023 | Washington, D.C.

**Huntington's Disease Society of America North Carolina Chapter – Team Hope Walk**
October 28, 2023 | Cary, NC

**Cure Huntington's Disease Initiative HSG (CHDI) Annual Meeting**
November 2–5, 2023 | Chandler, AZ

**American Heart Association Annual Scientific Sessions**
November 11–13, 2023 | Philadelphia, PA


**Parkinson's Foundation Florida Chapter** *Living with Parkinson's: Mind, Mood, and Motion*, The Villages, FL


## CLINICAL TRIALS

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

## NEWSLETTER

Sign up to get our latest patient news at
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