AskBio™

The AAVenger™

ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

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ASKBIO RECEIVES FDA RARE PEDIATRIC DISEASE AND ORPHAN-DRUG DESIGNATIONS FOR AB-1003 FOR THE TREATMENT OF LIMB-GIRDLE MUSCULAR DYSTROPHY TYPE 2I/R9

First patient dosed in Phase 1/Phase 2 LION-CS101 trial of AB-1003 in August 2023, with enrollment continuing

Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that AB-1003 (also known as LION-101) has received rare pediatric disease designation and orphan-drug designation from the US Food and Drug Administration (FDA) for the treatment of limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9).

FDA grants rare pediatric disease designation to incentivize the development of new treatments for serious and life-threatening diseases that primarily affect children aged 18 years or younger, with fewer than 200,000 people affected in the US. If AB-1003 is approved, AskBio may qualify for a priority review voucher based on receipt of this designation. A priority review voucher can be applied to another therapy in the company's pipeline, enabling a shorter review timeline during marketing application review or can be sold and transferred to another company.

Orphan designation provides orphan status to drugs and biologics for rare diseases that meet certain criteria and potentially gives a company exclusive marketing rights for a seven-year period, along with other benefits.

CLICK HERE TO READ THE FULL ANNOUNCEMENT

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

"AskBio's receiving FDA rare pediatric disease and orphan-drug designations for AB-1003 marks an important moment for people living with limb-girdle muscular dystrophy type 2I/R9 and their loved ones. These designations support the work the company is doing to explore the potential of gene therapy in an area where there is tremendous unmet medical need. Congratulations to everyone on the AskBio team. We are grateful for the partnership we have with AskBio as we collaborate in support of the LGMD2I/R9 patient community. We appreciate all you do, and we're counting on you."

KELLY BRAZZO | CO-FOUNDER & CEO | CURELGMD2I FOUNDATION

Kelly with her daughter, Sammy, living with LGMD2I/R9

Colleen and Mike McLain

A MULTIPLE SYSTEM ATROPHY (MSA) COMMUNITY INTERVIEW WITH MIKE AND COLLEEN FROM RESTON, VA

It is difficult, at best, to put into words the enormity of the everyday life challenges a patient living with Multiple System Atrophy (MSA) faces. Imagine life as successful engineer with two advanced degrees, one in which you graduated first in your class. For years, you awoke each day with the promise of all that life offers – nurturing a successful career, participating in family activities, attending sporting events with your kids, traveling with ease, and enjoying the outdoors… then, one day you receive a devastating diagnosis. This is the story of Mike McLain, a rocket scientist who lives with MSA, a rare genetic condition that impedes every aspect of daily life, which has also placed massive demands on his wife Colleen.

How has MSA personally affected your family?

MSA is a condition that causes gradual and permanent damage to critical nerve cells in the brain. For Mike, "living with MSA is a challenge because you never know the day or time when your involuntary systems are going to go out of whack. I was a high-level aerospace engineer and now I can barely balance a checkbook." Mostly confined to a wheelchair, Mike wears a helmet to protect himself from the unexpected times when he blacks out, about 50 – 80 times a month. A good day is when Mike is not bedridden, his blood pressure is somewhat under control, and he can use the bathroom without passing out. It's a day when he can sit in a wheelchair without difficulty breathing, when he can speak without a mumble and when his constant tremors are tolerable.

What do you wish people knew about living with MSA?

Colleen has been caregiving for Mike since his symptoms began to appear in 2010 and his official diagnosis in 2020. MSA is usually fatal within 10 years, however, Mike's symptoms were more gradual, and today are especially apparent. Unquestionably aware of the fate that lies ahead, Mike and Colleen face every day with an unwavering appreciation for daily life. Good days or bad, Colleen takes on the daily responsibilities of every household chore knowing her spouse is unable to assist. She must also monitor and document Mike's bodily functions every hour from sunup to sundown and even during sleep. Mike makes his regimen of pills abundantly clear proclaiming, "I'm on an ungodly amount of medication. For every single involuntary function my body has – I am on some type of medication."

What are your hopes for the future?

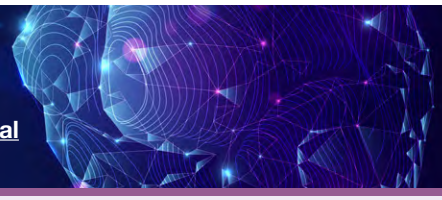
Despite the many mental and physical challenges that are a result of this devastating condition, Colleen remarks, "What impresses me is how Mike faces MSA. It impacts every aspect of our lives, yet he has never let it define him. Deep down, he is still the same person that I first met and fell in love with." Mike is well aware that his world is getting smaller as his MSA progresses and the care required continues to intensify. Speaking with admiration about his wife and caregiver, "a lot of people say this is too much, but Colleen has been my biggest advocate. I've never had to worry if she was going to be there for me. With no cure to directly treat MSA, our hope is that gene therapy can ultimately control the progression or one day provide a cure."

For more information about MSA, visit **Defeat MSA Alliance** and **Mission MSA**.

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

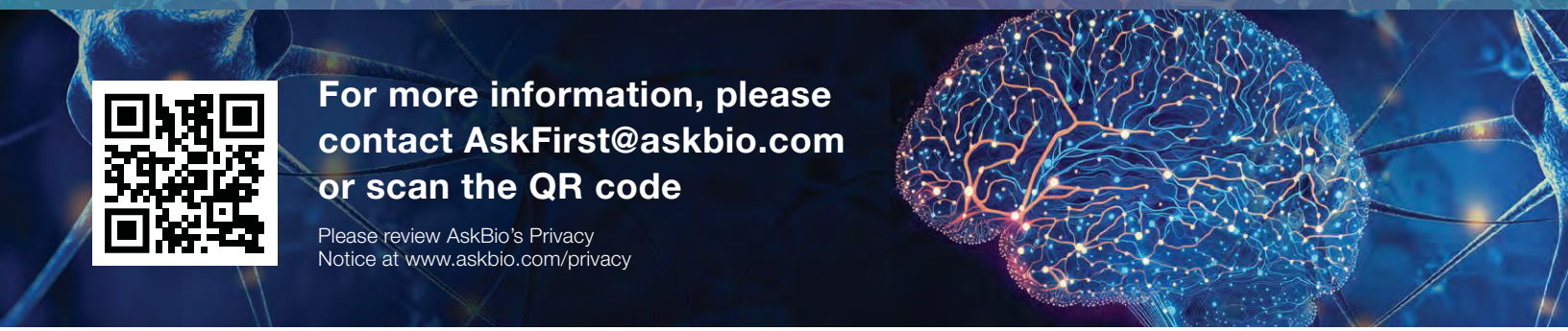
For more information, please contact AskFirst@askbio.com or scan the QR code

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

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>>**



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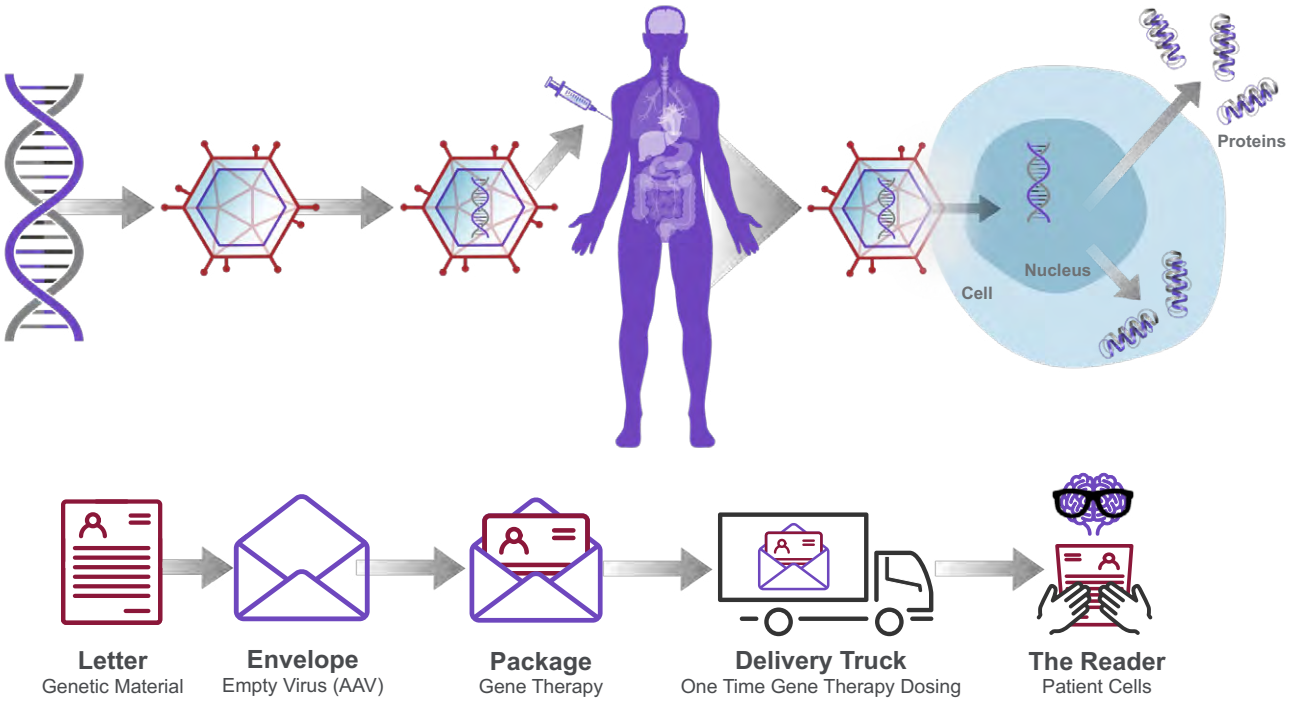
UNDERSTANDING AAV GENE THERAPY

Genes are the blueprints of the human body. They instruct the body's cells how to make proteins critical to their function. Many familiar and not-so-familiar diseases and disorders are caused by the faulty expression of proteins from corrupted or missing genes or by environmental modifications of genes or proteins.

AAV Gene therapy is a one-time therapy that uses the shell of a naturally occurring virus, not known to cause disease, where the viral DNA has been removed and replaced with a correct copy of the gene and it becomes a precisely coded vector and is no longer considered a virus, as most of the viral components have been replaced. The therapeutic gene is then delivered to cells or tissues to address genetic diseases at their source. One of the most exciting advances in modern medicine has been the discovery of how the adeno-associated virus (AAV) can be used as an effective delivery system for therapeutic genetic material into living tissue.

Today, AAV technology has advanced to target a wide range of tissues and cells for the treatment of many genetic diseases.

How an Adeno-Associated Virus Works



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.

GenePHIT

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Parkinson's Foundation Moving Day NC Triangle
November 2, 2024, Raleigh, NC

AHA Heart Walk
November 2, 2024, Philadelphia, PA

CLINICAL TRIALS

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

NEWSLETTER

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LET'S TALK

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