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ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

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Philip Fortier, MA
Founder and Chair
Defeat MSA Alliance

ASKBIO ANNOUNCES FIRST PATIENT RANDOMIZED IN PHASE 1 TRIAL OF AB-1005 (AAV2-GDNF) GENE THERAPY FOR MULTIPLE SYSTEM ATROPHY-PARKINSONISN TYPE (MSA-P)

- Gene therapy AB-1005 being developed to locally increase GDNF levels in the brain for the treatment of MSA-P
- Phase 1 randomized, controlled trial to assess safety of AB-1005 delivered to the putamen in patients with MSA-P

AskBio announced that the first patient has been randomized in the Phase 1 REGENERATE MSA-101 clinical trial of AB-1005, a gene therapy being developed as a treatment for multiple system atrophy-parkinsonian type (MSA-P). This marks a significant milestone in the development of AB-1005 gene therapy, an adeno-associated viral vector encoding for glial cell line-derived neurotrophic factor (AAV2-GDNF) that is delivered to the putamen, and brings this therapeutic one step closer to potentially reaching patients.

Brain Neurotherapy Bio, Inc., an AskBio subsidiary, is the sponsor for REGENERATE MSA-101.

CLICK HERE TO READ THE FULL ANNOUNCEMENT

> "It means a lot to the MSA community to know that the first patient has been enrolled in the Phase I REGENERATE MSA-101 trial. There is no cure for MSA, and there are currently no treatments to stop or slow the progression of the disease. This makes it especially hard for patients given the rapid decline many will experience. Today's milestone hopefully brings us one step closer to potentially changing the outcome for MSA patients around the world."
>
> PHILIP FORTIER, MA | FOUNDER AND CHAIR | DEFEAT MSA ALLIANCE

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 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

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 PRESENTS PRELIMINARY FIRST IN-HUMAN PHASE 1 DATA IN CONGESTIVE HEART FAILURE (CHF) AT 2023 AHA SCIENTIFIC SESSIONS

- CHF is a leading cause of morbidity and mortality in westernized countries
- Current standard of care does not modify the disease and patients ultimately progress to end stage heart failure and death

AskBio presented first in-human Phase 1 data at this year's American Heart Association Scientific Sessions, which concluded in Philadelphia, U.S., on November 13, 2023. The study sought to establish safety and preliminary efficacy of gene therapy AB-1002 (also known as NAN-101) in patients with advanced heart failure. AB-1002 is a rationally designed cardiotropic AAV vector targeting protein phosphatase inhibitor-1.

> "These encouraging early results in patients with advanced heart failure are important for the CHF community, as they bring hope to a sub-population where treatment options are needed. Seeing the potential of gene therapy being explored in heart failure is an important step forward in one day potentially changing the direction of this devastating disease, which is a leading cause of morbidity and mortality in westernized countries."

LITSA KRANIAS, PHD
HANNA CHAIR OF CARDIOLOGY, UNIVERSITY OF CINCINNATI
US COORDINATOR, CURE-PLAN

mytomorrows

ANNOUNCING ASKBIO'S COLLABORATION WITH MYTOMORROWS

AskBio is pleased to announce our new collaboration with myTomorrows, a platform connecting patients and physicians with clinical trials taking place around the world. This collaboration is set to transform the clinical trial recruitment experience, streamlining the triaging process for patients and other stakeholders involved. Our initial engagement is focused on AskBio's gene therapy trials related to MSA (Multiple System Atrophy) and LGMD (Limb-Girdle Muscular Dystrophy), aiming to significantly enhance the experience of patient recruitment and participation in these rare disease areas of study. *mytomorrows.com*

Our collaboration

myTomorrows works with pharmaceutical companies and patient advocacy groups to fulfill their mission of enabling earlier and better access to all possible treatment options. The team includes patient navigators offering a white-glove service to guide patients and their families through the clinical trials process. Our collaboration reflects a shared dedication to patient empowerment and innovative clinical care. The launch of AskBio's AskFirst™ program, a patient- and family-centric initiative in gene therapy, dovetails with myTomorrows commitment to putting patients first. We are each focused on educating and supporting (potential) clinical trial participants. myTomorrows eases the process of discovering and accessing relevant clinical trials, supporting the recruitment and pre-screening of MSA and LGMD patients who may be interested in joining a study.

How does myTomorrows help patients access AskBio's MSA and LGMD clinical trials?

myTomorrows market-leading patient navigator service is pivotal in enhancing AskBio's clinical trial pre-screening for both programs. It plays a crucial role in identifying potential patients by assessing main eligibility criteria. This is key not only to the patient's journey but also to streamline clinical trial access. By proactively identifying and tackling potential challenges that patients and other key stakeholders may encounter in discovering and accessing AskBio's clinical trials, patient navigators aim to understand the medical profile of each individual, performing a first eligibility assessment. This personalized attention enhances the overall experience for patients as they are supported and guided throughout the complete process with a human touch. Moreover, it also simplifies the workflow for health care professionals, significantly reducing the typical obstacles in clinical trial recruitment and participation.

myTomorrows Patient Navigators Terri Ellsworth and Madeleine Carrier, AskBio LGMD Medical Affairs, Patient Advocacy & Clinical Development Lead.

By ensuring that patients and their families are thoroughly engaged and well-informed at every step, myTomorrows creates a more effective and satisfying clinical trial referral experience, broadening accessibility and potentially yielding improved outcomes for everyone involved.

Madeleine Carrier, one of the dedicated patient navigators, shares, "**My role is to be a steadfast ally for our patients. It is about ensuring they feel heard, understood, and supported at every phase of their journey. We counter common misconceptions about clinical trials and strive to ensure that patients thoroughly understand what to expect at each stage.**"

Reflecting on this collaboration, the Head of Patient Advocacy at AskBio, Kara Witcoff, adds, "**Our partnership with myTomorrows demonstrates the evolution of AskBio's commitment to patients and our patient-centric culture, offering comprehensive support to those considering our trials.**"

Join a Clinical Trial

Are you or a loved one considering participation in an AskBio clinical trial for MSA or LGMD? Please visit *www.askbio.com/gene-therapy-clinical-trials* to learn more and start your journey. Patient navigators are ready to assist.

International LGMD Summit in Washington, D.C.

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 or go to clinicaltrials.gov (NCT05230459)

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Neuro Challenge Foundation for Parkinson's Expo
March 2, 2024 | Brandenton, FL

MDA Clinical & Scientific Conference
March 3–6, 2024 | Orlando, FL

Huntington Disease Society of America's Team Hope Walks in Cary, NC (NC Chapter) left and Philadelphia, PA (Eastern PA Chapter) right.

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

NEWSLETTER
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