AskBio™

The AAVenger™

ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

SUMMER 2023 | VOL.6

Gustavo Pesquin
Chief Executive Officer
AskBio

ASKBIO ANNOUNCES GUSTAVO PESQUIN AS NEW CEO

Gustavo Pesquin became the company's new Chief Executive Officer (CEO) as of March 11, 2023.

Gustavo joins AskBio from Amneal Pharmaceuticals, Inc., where he served as Chief Commercial Officer. In addition, he brings to AskBio his more than 10 years of experience at Sanofi, a leading global pharmaceutical company, where he served in leadership roles of increasing responsibility, including North America Head for General Medicines as well as Global Head of the Diabetes and Cardiovascular Franchise. Prior to Sanofi, he held Regional Head, General Manager, Sales Head and Strategy Head roles at Abbott and Pfizer; Brand Management roles at Procter & Gamble; and consultant roles with Boston Consulting Group.

Since 2017, AskBio has grown to over 800 employees operating in five countries and has advanced six therapeutics through IND, including potential treatments for congestive heart failure, Parkinson's disease, Huntington's disease, multiple system atrophy, Pompe disease and limb-girdle muscular dystrophy type 2I/R9. Today we are in the clinic with treatments for multiple diseases, and through our subsidiaries we are the leading manufacturer of synthetic DNA and AAV vectors at commercial scale.

Under Gustavo's leadership, AskBio will continue to advance its world-class therapeutics and manufacturing operations, and further its work as a cornerstone of the Bayer Cell & Gene Therapy Platform. "I couldn't be more excited to join a team that has been at the forefront of a scientific revolution," said Pesquin. "It's humbling to work with colleagues of such an incredibly high scientific caliber. I think we have the opportunity to help patients in a way that was unthinkable not long ago. It's very energizing to join an organization with such purpose."

Asklepios BioPharmaceutical, Inc. (AskBio) is a wholly owned and independently operated subsidiary of Bayer AG.

AskBio was proud to be an exhibitor at the ASGCT 2023 Annual Meeting.
annualmeeting.asgct.org

ASGCT 26TH ANNUAL MEETING

May 16-20, 2023 | Los Angeles, CA

AskBio
A Bayer Cell & Gene Therapy Platform Company

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.

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

AN INTERVIEW WITH PHILIP FORTIER – EXECUTIVE DIRECTOR OF THE DEFEAT MSA ALLIANCE

(L to R): Philip Fortier, Nadia di Lorio, Mattia Fontana, Tiliano Fontana

How has Multiple System Atrophy (MSA) personally affected you?

My entry into the MSA world came with my brother Joe who had MSA. He was diagnosed at 53 and passed away at 56, after a particularly cruel decline. His MSA was confirmed upon autopsy.

My brother Joe had a career in the medical field, managing sleep disorder clinics throughout Southeastern Michigan and helping to conduct sleep research.

The most significant symptom of his MSA was orthostatic hypotension. He had severe drops in BP, when changing positions and after eating. Even while on max doses of the very few BP drugs on the market at the time, he could barely lift his head. He was entirely bedridden, unable to move or to speak.

During his life, I was involved in trying to get droxidopa, a generic medication that showed promise in treating orthostatic hypotension. Eventually he was able to get the drug via the expanded use process and he benefitted from it for the last 6 months of his life. He was able to relate with his family more and it improved the quality of his life. True, it did not slow the underlying disease, but it helped make his life worth living – it improved the quality of his life. After my brother's death in 2013, I helped in the successful effort to get droxidopa approved by the FDA, testifying on behalf of MSA patients and their families.

Since that time, I have continued to be involved in the MSA cause. With the help of my spouse who is a neurologist, I started Defeat MSA Alliance (USA) in 2013, Defeat MSA/ Vaincre MSA Canada in 2019, Defeat MSA Australia in 2020 and Defeat MSA New Zealand in 2021. Also in 2021, with the addition of 2 other previously formed charities in Spain and Italy, the MSA United Consortium was founded. In 2022, MSA Denmark joined the global consortium.

What led you to start Defeat MSA Alliance?

Despite having good connections in the medical field, my brother encountered many medical professionals that knew little, if anything, about MSA. And because there was little knowledge, people did not seem to care about this devastating condition. MSA patients are confronted with a dim prognosis and left with few options for treatments. My brother also thought it was important to get the word out there about the lives of people affected by MSA.

How important is it to find new treatments for MSA?

The starting point toward the progress to new treatments begins with awareness, with more awareness comes more support and true support brings with it a concern for effective treatments. Awareness and support are key but there must be real movement toward new treatments. Otherwise, it is just lip service. We must be willing to put our words into action!

What do you wish people knew about living with MSA?

Like any chronic disease, there are challenges in everyday living. But people suffering from MSA have added complications, which are oftentimes not present in other more well-known diseases. There are very few treatments and little knowledge about the condition among the public. The social isolation of having this disease can be overwhelming. We need to increase medical knowledge about MSA while also raising greater awareness among the public.

What are your hopes for the future?

Simply put, my hope is that each person diagnosed with MSA will be afforded a fighting chance to live and flourish, despite the rarity of their condition. That their life will be valued. There is no disease so rare that it does not deserve treatment. I see medical treatment, even for rare and chronic neurodegenerative disorders such as MSA as a human right that needs to be honored and uplifted. We are getting better as a society, recognizing the human toll in the rare disease community but we can do so much more. I am often reminded of a civil rights motto: "if not us, then who, if not now, then when?" This is true of MSA, just as any other serious social ill. We have the moral duty to make the change we want to see, and the time is now to do it. **defeatmsa.org**

Now Recruiting

ASKBIO LGMD2I/R9 PHASE 1/2 CLINICAL PROGRAM

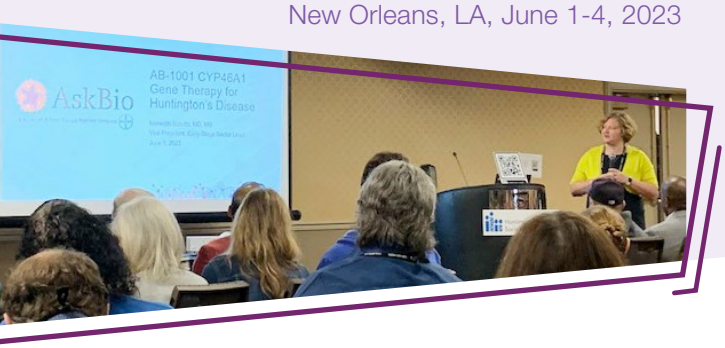
AskBio is conducting a clinical study of an investigational gene therapy for individuals with a confirmed genetic diagnosis of LGMD2I/R9.

- 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 AB-1003 (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 AB-1003 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)

ASKBIO WAS A PROUD SPONSOR AND EXHIBITOR AT THE HDSA 2023 ANNUAL CONVENTION.

Huntington's Disease Society of America
New Orleans, LA, June 1-4, 2023



CLICK HERE FOR MORE INFO

ASKBIO WAS A PROUD SPONSOR AND EXHIBITOR AT THE ANNUAL DYSTROGLYCANOPATHY PATIENT AND FAMILY CONFERENCE.

Dystroglycanopathy Patient and Family Conference
Iowa City, IA, June 23-24, 2023

CLICK HERE FOR MORE INFO

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*

FOLLOW US