



AskBio Announces Completion of Enrollment in Phase 1 Clinical Trial of AB-1005 Gene Therapy for Multiple System Atrophy-Parkinsonian Type (MSA-P)

- *Final participant randomized to complete enrollment in Phase 1 clinical trial assessing safety of investigational gene therapy AB-1005 delivered to the putamen in patients with multiple system atrophy-parkinsonian type (MSA-P)*
- *Completion of enrollment marks a significant milestone in progression of MSA-P clinical program and follows significant AB-1005 Parkinson's disease program updates*

Research Triangle Park, N.C. – SEPTEMBER 17, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced the completion of enrollment for REGENERATE MSA-101, its Phase 1 clinical trial of AB-1005, an investigational gene therapy being developed as a treatment for multiple system atrophy-parkinsonian type (MSA-P).¹

The completion of enrollment marks a significant milestone in the development of AB-1005, an investigational gene therapy based on adeno-associated viral vector serotype 2 (AAV2) containing the human glial cell line-derived neurotrophic factor (GDNF) transgene, and it brings this potential treatment option one step closer to reaching the many patients affected by MSA-P.

MSA-P can initially be difficult to distinguish from Parkinson's disease and is marked by slow movement, lack of coordination, imbalance, dizziness, and fainting, among other symptoms.² Individuals experience increasing difficulty with movement and autonomic dysfunction.² This is a result of progressive loss of nerve cells in the brain and spinal cord.² Affecting approximately 400,000 people worldwide, MSA is a rare disease that in most cases seems to occur for unknown reasons.^{2,3,4} Symptoms tend to develop in people over 50 years of age, followed by rapid progression within 5–10 years.²

“There is currently no disease-modifying treatment to stop or slow the progression of MSA, which makes the completion of enrollment in REGENERATE MSA-101 an important step in AskBio's efforts to advance this clinical program and potentially bring a safe treatment to patients” said Krystof Bankiewicz, MD, PhD, Scientific Chair, Parkinson's and MSA at AskBio. “This latest milestone follows recent additional AB-1005 announcements highlighting meaningful progress in our Parkinson's disease program, including Phase 1b favorable safety outcomes.”

AskBio is also exploring AB-1005 beyond MSA-P in participants living with Parkinson's disease in REGENERATE-PD, the company's randomized, double-blind, surgically controlled Phase 2 clinical trial evaluating the safety and efficacy of AB-1005 delivered to the putamen in the brain of adult participants aged 45–75 years with moderate-stage Parkinson's.⁵

In January, AskBio announced that the first participants had been randomized in REGENERATE-PD.⁶ In February, AskBio received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for AB-1005 for Parkinson's disease, following the presentation of Phase 1b 36-month data that demonstrated a favorable safety profile and continued positive trends in assessed clinical outcome measures with no product-related serious adverse events.^{7,8}

AB-1005 is an investigational gene therapy that has not been approved by any regulatory authority, and its efficacy and safety have not been established or fully evaluated.



About the Phase 1 REGENERATE MSA-101 Trial

REGENERATE MSA-101 is a randomized, double-blind, placebo-controlled Phase 1 trial designed to determine the safety of AB-1005, an investigational gene therapy delivered to the putamen in patients with multiple system atrophy-parkinsonian type (MSA-P).¹ The trial will include adults aged 35 to 75 years, with a clinical diagnosis of MSA-P (as defined by current consensus criteria), who are suitable surgical candidates.¹ The trial enrolled 11 participants who were randomized on the day of surgery to receive either AB-1005 or a non-invasive control surgery without any infusion to the putamen.⁹ The trial has a three-year follow-up period. Initial results will be available after all participants have completed one year of blind clinical assessments and the trial has been unblinded. The first participant in the REGENERATE MSA-101 trial was randomized at the Ohio State University Wexner Medical Center, Ohio, with participants enrolled by investigators based at University of California Irvine, California; Quest Research Institute, Michigan; Vanderbilt University, Tennessee; and New York University Langone, New York. Brain Neurotherapy Bio, Inc., an AskBio subsidiary, is the sponsor for REGENERATE MSA-101. For more information, visit clinicaltrials.gov (NCT#04680065), visit askbio.com, or send an email to AskFirst@askbio.com.

About GDNF and AB-1005

Glial cell line-derived neurotrophic factor (GDNF) is a homodimer that is a distantly related member of the transforming growth factor- β superfamily. In midbrain cell culture experimental models, recombinant human GDNF promoted the survival and morphological differentiation of dopaminergic neurons and increased their high-affinity dopamine uptake. GDNF has long been suspected to be a potential treatment for neurodegenerative diseases, such as Parkinson's, marked by progressive degeneration of midbrain dopaminergic neurons.¹⁰ AB-1005 is an adeno-associated viral vector serotype 2 (AAV2) containing the human GDNF transgene, which allows for stable and continuous expression of GDNF in localized regions of the brain after direct neurosurgical injection with MRI-monitored convection-enhanced delivery.^{11,12}

About AskBio

AskBio Inc., a wholly owned and independently operated subsidiary of Bayer AG, is a fully integrated gene therapy company dedicated to steering gene therapy into a new era where it can transform the lives of a wider range of people living with rare and more common diseases. The company maintains a portfolio of clinical programs across a range of disease indications related to a single gene or multiple factors across cardiovascular, central nervous system, and neuromuscular conditions, with a clinical-stage pipeline that includes investigational therapeutics for congestive heart failure, limb-girdle muscular dystrophy, multiple system atrophy, Parkinson's disease, and Pompe disease. AskBio's end-to-end gene therapy platform includes our Pro10™ technology, which makes gene therapies more accessible by making research and commercial grade manufacturing more affordable. With global headquarters in Research Triangle Park, North Carolina, the company has generated hundreds of proprietary capsids and promoters, several of which have entered pre-clinical and clinical testing. An early innovator in the gene therapy field, with over 900 employees in five countries, the company holds more than 600 patents and patent applications in areas such as AAV production and chimeric capsids. Learn more at <http://www.askbio.com/> or follow us on [LinkedIn](#).

About Bayer

Bayer is a global enterprise with core competencies in the life science fields of health care and nutrition. In line with its mission, "Health for all, Hunger for none," the company's products and services are designed to help people and the planet thrive by supporting efforts to master the major



challenges presented by a growing and aging global population. Bayer is committed to driving sustainable development and generating a positive impact with its businesses. At the same time, the Group aims to increase its earning power and create value through innovation and growth. The Bayer brand stands for trust, reliability and quality throughout the world. In fiscal 2024, the Group employed around 93,000 people and had sales of 46.6 billion euros. R&D expenses amounted to 6.2 billion euros. For more information, go to www.bayer.com.

AskBio Forward-Looking Statements

This press release contains "forward-looking statements." Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible," and similar expressions are intended to identify forward-looking statements. These forward-looking statements include, without limitation, statements regarding AskBio's clinical trials. These forward-looking statements involve risks and uncertainties, many of which are beyond AskBio's control. Known risks include, among others: AskBio may not be able to execute on its business plans and goals, including meeting its expected or planned clinical and regulatory milestones and timelines, its reliance on third-parties, clinical development plans, manufacturing processes and plans, and bringing its product candidates to market, due to a variety of reasons, including possible limitations of company financial and other resources, manufacturing limitations that may not be anticipated or resolved in a timely manner, potential disagreements or other issues with our third-party collaborators and partners, and regulatory, court or agency feedback or decisions, such as feedback and decisions from the United States Food and Drug Administration or the United States Patent and Trademark Office. Any of the foregoing risks could materially and adversely affect AskBio's business and results of operations. You should not place undue reliance on the forward-looking statements contained in this press release. AskBio does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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References

1. GDNF Gene Therapy for Multiple System Atrophy. Available at: <https://clinicaltrials.gov/study/NCT04680065>. Accessed September 2025.
2. NIH – Multiple System Atrophy. Available at: <https://www.ninds.nih.gov/health-information/disorders/multiple-system-atrophy#toc-how-is-multiple-system-atrophy-diagnosed-and-treated>. Accessed September 2025.
3. Vanacore N, Bonifati V, Fabbrini G, et al. Epidemiology of multiple system atrophy. ESGAP Consortium. European Study Group on Atypical Parkinsonisms. *Neurol Sci.* 2001;22(1):97-99.
4. Bower JH, Maraganore DM, McDonnell SK, Rocca WA. Incidence of progressive supranuclear palsy and multiple system atrophy in Olmsted County, Minnesota, 1976 to 1990. *Neurology.* 1997;49(5):1284-1288.
5. Clinicaltrials.gov. A Study of AAV2-GDNF in Adults With Moderate Parkinson's Disease (REGENERATE-PD). Available at: <https://clinicaltrials.gov/study/NCT06285643>. Accessed September 2025.
6. AskBio.com. First participants randomized in AskBio Phase II gene therapy trial for Parkinson's disease. Available at: <https://www.askbio.com/first-participants-randomized-in-askbio-phase-ii-gene-therapy-trial-for-parkinsons-disease/>. Accessed September 2025.

7. AskBio.com. AskBio Receives FDA Regenerative Medicine Advanced Therapy designation for Parkinson's disease investigational gene therapy. Available at: <https://www.askbio.com/askbio-receives-fda-regenerative-medicine-advanced-therapy-designation-for-parkinsons-disease-investigational-gene-therapy/>. Accessed September 2025.
8. Nicolas M, et al. Preliminary Efficacy of GDNF Gene Therapy (AAV2-GDNF; AB-1005) in Parkinson's Disease: 36-Month Follow-Up From a Phase 1b Study. Presented at the International Congress of Parkinson's Disease and Movement Disorders 2024.
9. AskBio. Data on file.
10. Lin LF, Doherty DH, Lile JD, Bektesh S, Collins F. GDNF: a glial cell line-derived neurotrophic factor for midbrain dopaminergic neurons. *Science*. 1993;260(5111):1130-1132.
11. Heiss JD, Lungu C, Hammoud DA, Herscovitch P, Ehrlich DJ, Argersinger DP, Sinharay S, Scott G, Wu T, Federoff HJ, Zaghloul KA, Hallett M, Lonser RR, Bankiewicz KS. Trial of magnetic resonance-guided putaminal gene therapy for advanced Parkinson's disease. *Mov Disord*. 2019 Jul;34(7):1073-1078. doi: 10.1002/mds.27724. Epub 2019 May 30. PMID: 31145831; PMCID: PMC6642028.
12. Kells AP, Eberling J, Su X, Pivrotto P, Bringas J, Hadaczek P, Narrow WC, Bowers WJ, Federoff HJ, Forsayeth J, Bankiewicz KS. Regeneration of the MPTP-lesioned dopaminergic system after convection-enhanced delivery of AAV2-GDNF. *J Neurosci*. 2010 Jul 14;30(28):9567-77. doi: 10.1523/JNEUROSCI.0942-10.2010. PMID: 20631185; PMCID: PMC2914692.