# First European Participants Randomized in AskBio Phase 2 Gene Therapy Trial of AB-1005 for Parkinson's Disease

- *AB-1005, a glial cell line-derived neurotrophic factor (GDNF) investigational gene therapy, is being evaluated for the treatment of moderate-stage Parkinson's disease in the REGENERATE-PD trial*
- *Sites in Poland and the United Kingdom have started randomizing participants, with sites in Germany actively screening and following shortly*
- *AskBio initiated recruitment in the United States in 2024, with enrollment ongoing*

**Research Triangle Park, N.C. – September 22, 2025 –** AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the first European participants have been randomized in REGENERATE-PD, a Phase 2 clinical trial in participants with moderate-stage Parkinson's disease (PD).

"I believe the randomization of the first European participants in REGENERATE-PD, which makes this the first neurosurgical gene therapy program for Parkinson's to successfully randomize patients from both the United States and Europe into a single Phase 2 trial, is positive news for people living with Parkinson's disease and the physicians treating them," said Alan Whone, MD, PhD, REGENERATE-PD Europe Lead and Principal Investigator. "There is a significant need for neurorestorative therapies in Parkinson's and seeing the advancement of an important investigational gene therapy in a Phase 2 clinical trial will give hope to patients and the medical community alike."

PD is a progressive, neurodegenerative disorder affecting 1.2 million people in Europe, and this number is expected to double by 2030.[1] With the worldwide unmet medical need in mind, the objective of REGENERATE-PD, a randomized, double-blind, surgically controlled clinical trial, is to evaluate the safety and efficacy of investigational gene therapy AB-1005 delivered to the putamen in the brain of adult participants aged 45–75 years with moderate-stage PD.[2] REGENERATE-PD is estimated to enroll approximately 87 participants across clinical centers in Germany, Poland, the United Kingdom, and the United States.[2]

"Today's announcement marks an important milestone in the clinical development of our investigational gene therapy AB-1005, as we continue to work hard to deliver a safe and effective treatment option that may be neurorestorative for certain populations and may benefit people living with moderate-stage Parkinson's disease," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer at AskBio. "We are encouraged by the initiation of the REGENERATE-PD program in Europe and look forward to sharing further clinical updates as the program advances over the coming year."

Earlier this year, AB-1005 was granted Regenerative Medicine Advanced Therapy (RMAT) designation from the United States Food and Drug Administration (FDA).[3] RMAT is a designation granted by the FDA to regenerative therapies, including gene therapies, being developed to treat, modify, reverse, or cure serious or life-threatening diseases or conditions.[4] AB-1005 met the RMAT requirements as a gene therapy that is intended to slow disease progression and improve motor outcomes in patients with PD. RMAT provides recipients with enhanced access to the FDA, which could include intensive guidance on efficient drug development, rolling Biologics License Application (BLA) review, and other actions to expedite review.[4]

AskBio is also exploring AB-1005 beyond PD, in participants in the United States with the parkinsonian subtype of multiple system atrophy (MSA-P) in a Phase 1 clinical trial to assess the preliminary safety, tolerability, and efficacy for this rapidly progressing condition.[5]

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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 Parkinson's disease**

Parkinson's disease (PD) is a progressive neurodegenerative disease.[6] It has a significant impact on a person's daily life.[6] In PD, the death of dopamine producing nerve cells in the brain leads to the continuous loss of motor function.[7] Symptoms include tremors, muscle rigidity, and slowness of movement.[8] Additionally, people with PD experience non-motor symptoms, including fatigue and lack of energy, cognitive issues, and depression.[8] Symptoms typically intensify over time and make everyday tasks demanding.[8] The prevalence of PD has doubled over the past 25 years.[6] Today, more than 10 million people worldwide are estimated to be living with PD.[9] This makes it the world's second most prevalent neurodegenerative disease.[10] It is also the most frequent movement disorder.[6,11] At present there is no cure, and current treatment options are inadequate and lack the holistic management of symptoms so there is an urgent need for new therapies.[12]

**About the AB-1005 Phase 1b trial**

In the AB-1005 Phase 1b, multicenter, open-label, uncontrolled trial, participants were administered AB-1005 to the putamen via one-time bilateral convection-enhanced delivery.[13] Participants were enrolled into two cohorts, mild (6 participants) and moderate (5 participants), based upon the duration and stage of their Parkinson's disease (PD).[13] The objective of this investigation was to evaluate the safety and potential clinical effect of AB-1005 delivered to the putamen in participants with either mild- or moderate-stage PD.[13] The outcomes assessed at 36 months were incidence of treatment-emergent adverse events (TEAEs) as reported by the participants or assessed clinically by physical and neurological examinations, motor symptoms as reported via the Movement Disorder Society's Unified Parkinson's Disease Rating Scale (MDS-UPDRS), and PD Motor Diary self-assessments, non-motor symptoms of PD, and brain dopaminergic network integrity as measured by DaTSCAN.[13] These assessments will continue for up to five years. For more information, visit clinicaltrials.gov (NCT04167540 or askbio.com).

**About REGENERATE-PD**

REGENERATE-PD is a Phase 2, randomized, double-blind, surgery controlled trial of the efficacy and safety of intraputaminal AB-1005 in the treatment of adults (45–75 years) with moderate-stage Parkinson's disease.[2] The trial will include an estimated 87 participants with trial sites located in Germany, Poland, the United Kingdom, and the United States.[2] For more information about the REGENERATE-PD clinical trial, visit clinicaltrials.gov (NCT06285643), or visit askbio.com.

**About AB-1005**

AB-1005 is an investigational gene therapy based on adeno-associated viral vector serotype 2 (AAV2) containing the human glial cell line-derived neurotrophic factor (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.[14,15] In nonclinical studies, GDNF has been shown to promote the survival and morphological differentiation of dopaminergic neurons.[15,16] Recombinant GDNF has long been evaluated as a potential treatment for diseases, such as Parkinson's disease (PD), marked by progressive degeneration of midbrain dopaminergic neurons.[17] Through a combination of an investigational gene therapy and innovative neurosurgical delivery approach, we can now test the GDNF hypothesis in PD by getting this neurotrophic factor to these degenerating nigrostriatal neurons in a potentially more clinically relevant fashion.[17]

## 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 healthcare 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 http://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. European Parkinson's Disease Association. Let's talk about Parkinson's. Available at: https://www.age-platform.eu/sites/default/files/EPDA-Political_Manifesto_Parkinson.pdf. Accessed: August 2025.
2. ClinicalTrials.gov. A Study of AAV2-GDNF in Adults With Moderate Parkinson's Disease (REGENERATE-PD). Available at: https://clinicaltrials.gov/study/NCT06285643. Accessed: August 2025.
3. AskBio. 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: August 2025.
4. Food and Drug Administration. Expedited Programs for Regenerative Medicine Therapies for Serious Conditions. Available at: https://www.fda.gov/media/120267/download. Accessed: August 2025.
5. ClinicalTrials.gov. GDNF Gene Therapy for Multiple System Atrophy. Available at: https://clinicaltrials.gov/study/NCT04680065. Accessed: August 2025.
6. World Health Organization. Parkinson Disease. Available at: https://www.who.int/news-room/fact-sheets/detail/parkinson-disease. Accessed August 2025.
7. Ramesh S & Arachchige A. Depletion of dopamine in Parkinson's disease and relevant therapeutic options: A review of the literature. AIMS Neurosci. 2023 Aug 14;10(3):200-231.
8. National Institutes of Health. Parkinson's Disease. Available at: https://www.ninds.nih.gov/health-information/disorders/parkinsons-disease. Accessed: August 2025.
9. Maserejian N, et al. Estimation of the 2020 Global Population of Parkinson's Disease (PD). MDS Virtual Congress 2020. Abstract number 198. Available at: https://www.mdsabstracts.org/abstract/estimation-of-the-2020-global-population-of-parkinsons-disease-pd/. Accessed August 2025.
10. National Institute of Environmental Health Sciences. Neurodegenerative Diseases. Available at: https://www.niehs.nih.gov/research/supported/health/neurodegenerative. Accessed August 2025.
11. Stoker T & Barker R. Recent developments in the treatment of Parkinson's Disease. F1000Res. 2020 Jul 31;9:F1000 Faculty Rev-862.
12. Mayo Clinic. Parkinson's disease. Available at: https://www.mayoclinic.org/diseases-conditions/parkinsons-disease/diagnosis-treatment/drc-20376062. Accessed: August 2025.
13. Clinicaltrials.gov. GDNF Gene Therapy for Parkinson's Disease. Available at: https://clinicaltrials.gov/study/NCT04167540. Accessed August 2025.
14. Heiss J, et al. Trial of magnetic resonance-guided putaminal gene therapy for advanced Parkinson's disease. Mov Disord. 2019 Jul;34(7):1073-1078.
15. Kells A, et al. Regeneration of the MPTP-lesioned dopaminergic system after convection-enhanced delivery of AAV2-GDNF. J Neurosci. 2010 Jul 14;30(28):9567-77.
16. Lin L, et al. GDNF: a glial cell line-derived neurotrophic factor for midbrain dopaminergic neurons. Science. 1993;260(5111):1130-1132.
17. Barker RA, et al. GDNF and Parkinson's Disease: Where Next? A Summary from a Recent Workshop. J Parkinsons Dis. 2020;10(3):875-891.