AskBio Announces Publication of Complete Results of Phase 1b Trial of AB-1005 Gene Therapy in Participants with Parkinson's Disease in *Movement Disorders*

- AB-1005 was well tolerated with no attributed serious adverse events in participants with mild or moderate Parkinson's disease (PD) through 18 months post-gene transfer
- Non-motor assessments remained numerically stable throughout 18-month follow-up in mild and moderate cohorts
- Recruitment is ongoing in Europe and the United States for Phase 2 REGENERATE-PD trial to further explore the potential of AB-1005 in participants with moderate PD

Research Triangle Park, N.C.– MAY 27, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, announced today the recent publication of the complete results of its Phase 1b trial of AB-1005, a glial cell line-derived neurotrophic factor (GDNF) investigational gene therapy for the treatment of Parkinson's disease (PD), in *Movement Disorders,* an official peer-reviewed journal of the International Parkinson and Movement Disorder Society.[1]

"The exploration of the neurorestorative and neuroprotective potential of GDNF gene transfer is critical to advancing our understanding of AB-1005, which may one day be a groundbreaking treatment to slow the progression of Parkinson's disease," said Chad Christine, MD, Professor of Neurology at the University of California, San Francisco, and publication author. "These encouraging results support further evaluation of this therapy in a randomized trial."

The publication, which is available online, states that treatment with AB-1005 was well tolerated with no serious adverse events related to GDNF gene therapy in all 11 participants and is associated with numerical stability in clinical assessment readouts (mild cohort) and improvement (moderate cohort) in clinical assessments of motor function at 18 months post-gene transfer. In addition to these data, which were first presented in April 2024 at the American Academy of Neurology Annual Meeting, the publication notes that non-motor assessments remained numerically stable throughout the 18-month follow-up in the mild and moderate cohorts. Stability findings may be an indication of the potential for AB-1005 to positively affect disease progression.[1,2]

"These results support the continued study of AB-1005 as a potential gene therapy to slow the progression of Parkinson's disease," said Lila Collins, PhD, Associate Director of Portfolio Development & Review at the California Institute for Regenerative Medicine. "We are pleased to have supported this important trial and look forward to AskBio's Phase 2 REGENERATE-PD results, which will provide additional insights into the durability of the clinical response and potential disease-modifying effect of AB-1005."

In February 2025, AB-1005 was granted Regenerative Medicine Advanced Therapy (RMAT) designation from the United States Food and Drug Administration.[3]

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

About Parkinson's disease

Parkinson's disease (PD) is a progressive neurodegenerative disease.[4] It has a significant impact on a person's daily life.[4] In PD, the death of dopamine-producing nerve cells in the brain leads to the continuous loss of motor function.[5] Symptoms include tremors, muscle rigidity, and slowness of

movement.[6] Additionally, people with PD experience non-motor symptoms, including fatigue and lack of energy, cognitive issues, and depression.[6] Symptoms typically intensify over time and make everyday tasks increasingly demanding.[6] The prevalence of PD has doubled over the past 25 years.**Fehler! Textmarke nicht definiert.** Today, more than 10 million people worldwide are estimated to be living with PD.[7] This makes it the world's second most prevalent neurodegenerative disease.[8] It is also the most frequent movement disorder.[4,9] At present there is no cure, and current treatment options lack the holistic management of symptoms, so new therapies are needed.[10]

About the AB-1005 Phase 1b trial

In this Phase 1b, multicenter, multisite, open-label, uncontrolled trial, 11 participants were administered AB-1005 to the putamen via one-time bilateral convection-enhanced delivery. 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).[11] The objective of this investigation was to evaluate the safety and potential clinical effect of AB-1005 delivered to the putamen in participants with early/mild or moderate PD. 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.[11] 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, sham-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. The trial will include an estimated 87 participants with trial sites located in Germany, Poland, United Kingdom, and United States.[12] 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 convection-enhanced delivery.[13,14] In nonclinical studies, GDNF has been shown to promote the survival and morphological differentiation of dopaminergic neurons.[14,15] 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.[16] 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.[16]

About AskBio

AskBio Inc., a wholly owned and independently operated subsidiary of Bayer AG, is a fully integrated gene therapy company dedicated to developing life-saving medicines and changing lives. The company maintains a portfolio of clinical programs across a range of neuromuscular, central nervous system, cardiovascular, and metabolic disease indications 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 gene therapy platform includes Pro10™, an industry-leading proprietary cell line manufacturing process, and an extensive array of capsids and promoters. With global headquarters in Research Triangle Park, North Carolina, United States, 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 adeno-associated virus (AAV) production and chimeric capsids. Learn more at 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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