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News Release

Not intended for UK Media

First participants randomized in AskBio Phase II gene therapy trial for Parkinson's disease

- Enrollment ongoing in the United States for REGENERATE-PD clinical trial of AB-1005 (also known as AAV2-GDNF) glial cell line-derived neurotrophic factor (GDNF) investigational gene therapy for the treatment of moderate-stage Parkinson's disease
- Phase Ib 36-month data demonstrated a favorable safety profile and continued positive trends in assessed clinical measures of AB-1005 with no associated serious adverse events

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Berlin, Germany, and Research Triangle Park, N.C., USA, January 14, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the first participants have been randomized in its Phase II clinical trial in patients with Parkinson's disease (PD).

"The randomization of the first participants in REGENERATE-PD is positive news for people living with Parkinson's disease and the physicians treating them," said Dr Rajesh Pahwa, MD, Director, Parkinson's Disease and Movement Disorder Center, University of Kansas Medical Center, USA, and REGENERATE-PD 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 II clinical trial gives hope to patients and the medical community."

In 2024, AskBio initiated recruitment in the United States arm of REGENERATE-PD. The objective of this randomized, double-blind, Phase II clinical trial is to evaluate the safety and efficacy of AB-1005 delivered to the putamen in adult participants aged 45–75 years with moderate-stage PD. REGENERATE-PD is estimated to enroll approximately 87 participants across clinical centers that are being opened in the United States, Germany, Poland, and the United Kingdom.

AskBio also presented 36-month Phase Ib data at the International Congress of Parkinson’s Disease and Movement Disorders, which was held in Philadelphia, Pennsylvania, from September 27 to October 1, 2024. The data demonstrated that administration of AB-1005 was well tolerated with no attributed serious adverse events. The Moderate PD cohort showed trends for improvement or stability on several motor scales at 36 months, including Movement Disorder Society-Unified Parkinson's Disease Rating Scale (MDS-UPDRS) and motor diaries, and trends in reductions in Parkinson’s medications (levodopa-equivalent daily dose [LEDD]). Most participants in the Mild PD cohort showed an overall stable clinical status with little change in MDS-UPDRS, the self-reported PD motor diary, or LEDD.

“AskBio continues to mark significant milestones in the clinical development of the investigational gene therapy AB-1005 as we strive to bring a safe and effective gene therapy to patients with moderate stage Parkinson’s disease,” said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. “With REGENERATE-PD participants now being randomized, we are excited by our progress with AB-1005 and look forward to sharing further updates at an appropriate scientific forum as the program advances through next year and beyond.”

“The advancement of AskBio’s Parkinson’s program is a significant milestone that highlights our commitment to exploring the potential of gene therapy in an area of significant unmet medical need,” said Christian Rommel, Member of the Executive Committee of Bayer’s Pharmaceuticals Division, and Global Head of Research and Development. “We believe that therapeutic modalities like gene therapy can change the treatment landscape for people living with the debilitating effects of Parkinson’s disease and look forward to seeing what we will learn in this next phase of research and development for AB-1005.”

AskBio is also exploring AB-1005 beyond Parkinson’s disease and is currently enrolling participants in the United States with the parkinsonian subtype of multiple system atrophy (MSA-P) in a Phase I clinical trial to assess the preliminary safety, tolerability, and efficacy for this rapidly progressing condition.

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

About REGENERATE-PD

REGENERATE-PD is a Phase II, 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 subjects with trial sites located in the United States, Germany, Poland, and the United Kingdom. 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. In nonclinical studies, GDNF has been shown to promote the survival and morphological differentiation of dopaminergic neurons. 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. 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.

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, and European headquarters in Edinburgh, Scotland, 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 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 2023, the Group employed around 100,000 people and had sales of 47.6 billion euros. R&D expenses before special items amounted to 5.8 billion euros. For more information, go to www.bayer.com.

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Forward-Looking Statements
This release may contain forward-looking statements based on current assumptions and forecasts made by Bayer management. Various known and unknown risks, uncertainties and other factors could lead to material differences between the actual future results, financial situation, development or performance of the company and the estimates given here. These factors include those discussed in Bayer's public reports which are available on the Bayer website at www.bayer.com. The company assumes no liability whatsoever to update these forward-looking statements or to conform them to future events or developments.

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.