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News Release

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AskBio Receives FDA Regenerative Medicine Advanced Therapy designation for Parkinson's disease investigational gene therapy

- Regenerative Medicine Advanced Therapy (RMAT) designation follows Phase Ib 36-month data
- Study demonstrated favorable safety profile and continued positive trends in assessed clinical outcome measures of the investigational gene therapy AB-1005 (formerly known as AAV2-GDNF) with no product-related serious adverse events
- Enrollment ongoing for Phase II REGENERATE-PD clinical trial of AB-1005, a glial cell line-derived neurotrophic factor (GDNF) investigational gene therapy for the treatment of moderate-stage Parkinson's disease

Berlin, Germany, and Research Triangle Park, N.C., USA, February 19, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that investigational gene therapy AB-1005 for the treatment of Parkinson's disease (PD) has been granted Regenerative Medicine Advanced Therapy (RMAT) designation from the United States Food and Drug Administration (FDA).

"The FDA's decision to grant RMAT designation to AB-1005 is exciting news for people living with Parkinson's disease and their loved ones," said Gustavo Pesquin, CEO, AskBio. "This milestone could potentially expedite the development of our important investigational gene therapy program, and it highlights our promising data and the potential of AB-1005 for patients and the medical community. We look forward to working closely with the FDA to accelerate our program."

The FDA determined that AB-1005, an investigational gene therapy intended to slow disease progression and improve motor outcomes in patients with PD, met the criteria for RMAT designation. This decision follows a review of information and data provided by

AskBio, including clinical evidence from the open label, uncontrolled study Phase Ib trial of AB-1005.

AskBio's 36-month Phase Ib data showed that the administration of AB-1005 was well tolerated with no product-related serious adverse events.[1] Further, the moderate PD cohort showed trends for improvement or stability on several PD-relevant clinical scales at 36 months compared to baseline, including Movement Disorder Society-Unified Parkinson's Disease Rating Scale (MDS-UPDRS) and self-reported PD motor diaries, together with trends in reductions in Parkinson's medications (levodopa-equivalent daily dose [LEDD]).[1] 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.[1]

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.[2] Investigational products receiving this designation must have produced preliminary clinical evidence indicating that they may have the potential to address unmet medical needs for such diseases or conditions.[2] 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.[2]

"The RMAT designation for AB-1005 underscores the high unmet medical need and the potential of this investigational gene therapy to make a difference for patients with Parkinson's disease," said Christian Rommel, Executive Vice President, Global Head of Research and Development and Member of the Pharmaceuticals Leadership Team at Bayer. "This is the latest example of what can be achieved through the joint commitment of AskBio and Bayer to deliver breakthrough innovation for patients."

The first participants in the AB-1005 Phase II REGENERATE-PD clinical trial have been randomized in the United States, and the trial is currently recruiting.[3] Additional study sites in the United States, Germany, Poland, and the United Kingdom are expected to be opened for enrollment in first half of 2025.[3]

AB-1005 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. 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.[4] Symptoms include tremors, muscle rigidity, and slowness of movement.[4] Additionally, people with PD experience non-motor symptoms, including fatigue and lack of energy, congestive issues, and depression.[4] Symptoms typically intensify over time and make everyday tasks increasingly demanding.[4] The prevalence of PD has doubled over the past 25 years.[5] Today, more than 10 million people worldwide are estimated to be living with PD.[6] This makes it the world’s second most prevalent neurodegenerative disease.[7] It is also the most frequent movement disorder.[8,9] At present there is no cure, and current treatment options lack the holistic management of symptoms, so new therapies are needed.[10,11]

About the AB-1005 Phase Ib trial

In this Phase Ib, multi-center, multi-site, open-label, uncontrolled trial, 11 patients were administered AB-1005 to the putamen via one-time bilateral convection-enhanced delivery. Patients were enrolled into two cohorts, mild (6 patients) and moderate (5 patients), based upon the duration and stage of their PD. The objective of this investigation was to evaluate the safety and potential clinical effect of AB-1005 delivered to the putamen in patients with early/mild or moderate PD. The outcomes assessed at 36 months were incidence of Treatment-Emergent Adverse Events (TEAEs) as reported by the patients 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.1 These assessments will continue for up to five years. For more information, visit clinicaltrials.gov (NCT#04167540 or askbio.com).

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.[12] In nonclinical studies, GDNF has been shown to promote the survival and morphological differentiation of dopaminergic neurons.[13,14] 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.[15] 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.[15]

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.

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