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News Release

Not intended for UK Media

AskBio receives FDA Fast Track and MHRA Innovation Passport designations for AB-1005 investigational GDNF gene therapy for Parkinson's disease

- AB-1005 (formerly known as AAV2-GDNF) is being studied for the treatment of patients with moderate Parkinson's disease
- AskBio is currently enrolling patients in its Phase II REGENERATE-PD trial in the United States
- European Union and United Kingdom clinical trial sites planned to open later in 2024

Berlin, Germany, and Research Triangle Park, N.C., USA, July 11, 2024 – Bayer AG and Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the United States (U.S.) Food and Drug Administration (FDA) has granted Fast Track Designation for AB-1005, which is being developed for moderate Parkinson's disease. AB-1005 has also been awarded the Innovation Passport, the United Kingdom Medicines and Healthcare products Regulatory Agency (UK MHRA) innovative medicine designation, for the treatment of Parkinson's disease.

AB-1005 is an investigational adeno-associated virus 2 glial cell line-derived neurotrophic factor (AAV2-GDNF) neurorestorative gene therapy being studied for the treatment of moderate Parkinson's disease. Earlier this year, AskBio presented the 18-month Phase Ib clinical trial results for AB-1005, which met its primary objective of evaluating the safety of a one-time bilateral delivery of AB-1005 directly to the putamen.

"These designations clearly underscore the importance of developing innovative therapies for those living with Parkinson's disease, where a significant unmet need still exists," said Krystof Bankiewicz, MD, PhD, Scientific Chair, Parkinson's and MSA, AskBio. "They

further highlight the willingness of key regulatory bodies to support the accelerated development of AB-1005 with a focus on the potential benefit to patients."

The FDA Fast Track Program is designed to facilitate the development and expedite the review of new therapeutics that are intended to treat serious conditions and fill unmet medical needs.[1] The purpose of the Program is to get important new therapeutics to patients earlier.[1] Therapeutics that receive this designation benefit from eligibility for more frequent meetings with the FDA to discuss the clinical development plan and, if relevant criteria are met, eligibility for Accelerated Approval and Priority Review.

The UK MHRA Innovation Passport is the entry point to the Innovative Licensing and Access Pathway (ILAP), which aims to accelerate time to market, facilitating patient access. This designation provides Innovation Passport holders with the opportunity to work with the UK MHRA and partners to create product-specific Target Development Profiles (TDP) for new therapies. The TDP will define key regulatory and development features, identify potential pitfalls, offer access to specialist toolkits, and create a roadmap for delivering early patient access.[2,3]

"The FDA Fast Track and the UK MHRA Innovation Passport designations represent important accomplishments for the clinical development of AB-1005 and receiving these highlights our goal of bringing a safe neurorestorative treatment to patients with moderate Parkinson's disease," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. "We look forward to advancing our Phase II REGENERATE-PD clinical trial, which is currently enrolling patients in the U.S. with sites in the European Union and UK planned to open later this year."

"We are excited about the opportunity to potentially accelerate the development of AB-1005, leveraging the frequent interaction with relevant regulatory bodies," said Christian Rommel, PhD, Global Head of Research & Development at Bayer's Pharmaceuticals Division. "The granted designations for AB-1005 highlight the demand to advance novel therapeutic modalities, like gene therapy, for people living with the debilitating effects of Parkinson's disease."

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.

AskBio is also exploring GDNF therapy beyond Parkinson's disease and is currently enrolling patients in the U.S. with the parkinsonian subtype of multiple system atrophy (MSA-P) in a Phase I trial to assess the preliminary safety, tolerability, and efficacy of GDNF therapy for this rapidly progressing condition.[4]

About Parkinson's disease

Parkinson's disease is a progressive neurodegenerative disorder caused by the death of nerve cells in the brain, leading to decreased dopamine levels.[5] At diagnosis, it is estimated that patients have already lost 50-80% of their dopaminergic neurons.[6] The loss of these neurons leads to a progressive loss of motor function and symptoms such as tremors, muscle rigidity, and slowness of movement.[7] Even with medication, the symptoms of Parkinson's disease can fluctuate during the course of the day. According to the Parkinson's Foundation, more than 10 million people worldwide suffer from Parkinson's disease, with approximately one million living in the U.S.[8] There is no cure, and the effectiveness of current treatments frequently decreases over time.[9]

About REGENERATE-PD

REGENERATE-PD is a Phase II, 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. Subjects will receive either bilateral image-guided infusion of AB-1005 into the putamen, single dose (active treatment arm), or bilateral partial burr/twist holes (control arm). The trial will include an estimated 87 subjects with trial sites located in the U.S., European Union, and the UK. 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 containing the human glial cell line-derived neurotrophic factor (AAV2-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.[10,11] GDNF is a homodimer that is a distantly related member of the transforming growth factor-β superfamily. In midbrain neuronal cell cultures, recombinant human GDNF promoted the survival and morphological differentiation of dopaminergic neurons and increased their high-affinity dopamine uptake. GDNF has long been

evaluated as a potential disease modifying neurorestorative treatment for diseases, such as Parkinson's, marked by progressive degeneration of midbrain dopaminergic neurons.[12]

About AskBio

Asklepios BioPharmaceutical, Inc. (AskBio), 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 therapeutics for congestive heart failure, Huntington's disease, 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 capsid and promoter library. 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 800 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.

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