

AskBio Initiates Recruitment to its Phase 2 Parkinson's Disease Trial

- *REGENERATE-PD, which is now enrolling, will study AB-1005, an adeno-associated virus 2 (AAV2) glial cell line-derived neurotrophic factor (GDNF) gene therapy for the treatment of moderate-stage Parkinson's disease*
- *Follows January announcement that Phase 1b trial of AB-1005 met its primary endpoint, and presentation of 18-month results at AAN in April 2024*
- *Enrolment will initially begin in the United States, with clinical trials sites in the UK and Europe opening later*

Research Triangle Park, N.C. – JUNE 25, 2024 – Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced initiation of recruitment to REGENERATE-PD, a Phase 2 clinical study assessing efficacy and safety of AB-1005, an investigational adeno-associated virus 2 (AAV2) glial cell line-derived neurotrophic factor (GDNF) gene therapy for the treatment of moderate-stage Parkinson's disease.

"By enhancing levels of a naturally occurring growth factor, glial cell line-derived neurotrophic factor (GDNF) gene therapy is intended to promote the survival and functioning of vulnerable brain cells that degenerate in Parkinson's disease", said Alan Whone, MD, PhD, Consultant Senior Lecturer in Movement Disorder, Bristol Medical School, and Honorary Consultant Neurologist at North Bristol Trust, UK. "The advancement of AB-1005 is a significant milestone in the development of a gene therapy for Parkinson's disease and has the potential to bring an effective treatment one step closer to patients". Dr Whone will act as European Lead Principal Investigator (PI) on REGENERATE-PD once the program becomes active in the UK.

According to the Parkinson's Foundation, more than 10 million people worldwide suffer from Parkinson's disease.¹

"Following the encouraging results from the Phase 1b study and the presentation of 18-month data at the American Association of Neurology (AAN) meeting in April, we are excited to be progressing AB-1005 to this larger, Phase 2 study," said Krystof Bankiewicz, MD, PhD, Scientific Chair, Parkinson's and MSA, AskBio. "This latest advancement highlights our confidence in the potential of AB-1005 to provide a transformative impact for patients with Parkinson's disease."

In January, AskBio announced that the Phase 1b trial of AB-1005 met its primary endpoint, which was to evaluate the safety of a one-time bilateral delivery of AB-1005 directly to the putamen. The investigational gene therapy for the treatment of Parkinson's disease was well tolerated with no serious adverse events that were considered related to AB-1005 in all 11 patients at 18 months.²

AskBio is also exploring GDNF therapy beyond Parkinson's disease and is currently enrolling patients in the US with the parkinsonian subtype of multiple system atrophy (MSA-P) in a Phase 1 trial to assess the preliminary safety, tolerability, and efficacy of GDNF therapy for this rapidly progressing condition.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 established or fully evaluated.



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.⁴ At diagnosis, it is estimated that patients have already lost 50-80% of their dopaminergic neurons.⁵ The loss of these neurons leads to a progressive loss of motor function and symptoms such as tremors, muscle rigidity, and slowness of movement.⁶ 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 United States.¹ There is no cure, and the effectiveness of current treatments frequently decreases over time.⁷

About REGENERATE-PD

REGENERATE-PD is a Phase 2, randomized, double-blind, surgery-controlled study of the efficacy and safety of intraputamin AAV2-GDNF in the treatment of adults (45-75 years) with moderate stage Parkinson's disease. Subjects will receive either bilateral image-guided infusion of AAV2-GDNF 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 study sites located in the United States, UK and Europe. For more information about the REGENERATE-PD clinical trial, visit [clinicaltrials.gov](https://clinicaltrials.gov/ct2/show/study/NCT06285643) (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.^{8,9} 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 treatment for diseases, such as Parkinson's, marked by progressive degeneration of midbrain dopaminergic neurons.¹⁰

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.

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.

AskBio Media Contact:

Andrew Thomas, phone +44 (7803) 585254

Email: athomas-c@askbio.com

Find more information about AskBio at www.askbio.com

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