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News Release

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AskBio's AB-1005 and AB-1002 Receive Pioneering Regenerative Medical Product Designation in Japan

Product designation supports development of innovative investigational gene therapies for Parkinson's disease and congestive heart failure

Berlin, Germany, and Durham, N.C., USA, December 9, 2025 – AskBio Inc., a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that Japan's Ministry of Health, Labour and Welfare (MHLW) has granted the Pioneering Regenerative Medical Product designation (SAKIGAKE) for two of AskBio's investigational gene therapy programs: AB-1005, for the treatment of Parkinson's disease (PD), and AB-1002, for the treatment of non-ischemic heart failure with reduced left ventricular ejection fraction and New York Heart Association (NYHA) Class III heart failure despite appropriate medical therapy.

The designation reflects Japan's commitment to expediting the development and review of breakthrough therapies and is awarded to products demonstrating innovativeness (a new mode of action), prominent efficacy or safety data, and the potential to address severe diseases, especially when submitted first or simultaneously with other countries. This recognition offers significant advantages, including priority consultations and accelerated review timelines, thereby facilitating earlier participant access to transformative treatments.

"Having AB-1005 and AB-1002 receive the Pioneering Regenerative Medical Product designation in Japan highlights our dedication to advancing innovative gene therapies for participants facing serious diseases," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. "This recognition not only accelerates regulatory review but also reaffirms our commitment to delivering advanced treatments to those living with serious chronic diseases that lack therapies targeting root causes."

AB-1005, currently being evaluated in the Phase II REGENERATE-PD trial, is an investigational gene therapy with adeno-associated viral (AAV) vector-mediated delivery of the glial cell line-derived neurotrophic factor (GDNF) gene for participants with moderate-stage PD. The therapy aims to restore neuronal function and potentially slow disease progression for people with limited treatment options. AB-1005 previously received US Food and Drug Administration (FDA) Regenerative Medicine Advanced Therapy (RMAT), FDA Fast Track, and UK Medicines and Healthcare products Regulatory Agency (MHRA) Innovation Passport designations, underscoring its global significance and potential for participants.[1]

AB-1002 is an investigational AAV gene therapy being studied for the treatment of adults with NYHA Class III heart failure with non-ischemic etiology. It previously received FDA Fast Track designation, and is designed as a one-time gene therapy targeting protein phosphatase 1 inhibition, with the intention of improving cardiac function and addressing the substantial global burden of congestive heart failure.[2]

“Bayer and AskBio’s collaboration continues to drive progress in gene therapy with a robust pipeline targeting central nervous system, cardiovascular, and other disease indications,” said Christian Rommel, PhD and Global Head of Research and Development for Bayer's Pharmaceuticals Division. “Receiving the designation in Japan, which is a first for Bayer, marks an important milestone in expanding global access to pioneering therapies and reinforces our shared commitment to delivering breakthrough science to improve outcomes for patients worldwide.”

FDA 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.[3] 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.[3] 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.[3]

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 address unmet medical needs.[3] The purpose of the program is to get important new therapeutics

to participants earlier.[3] 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.[3]

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 participants access to innovative medicines. 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 participants access.[4,5]

AB-1005 and AB-1002 are investigational gene therapies that have not been approved by any regulatory health authority, and their efficacy and safety have not been established or fully evaluated.

AskBio is also exploring AB-1005 beyond PD and is currently conducting the MSA-101 trial in the U.S. for participants diagnosed with multiple system atrophy of the parkinsonian subtype (MSA-P) in a Phase I trial to assess the preliminary safety, tolerability, and efficacy of GDNF gene therapy for this rapidly progressing condition for which no treatment options are currently available.[6]

About Parkinson's disease

Parkinson's disease (PD) is a progressive neurodegenerative disease.[7] It has a significant impact on a person's daily life.[7] In PD, the death of dopamine producing nerve cells in the brain leads to the continuous loss of motor function.[8] Symptoms include tremors, muscle rigidity, and slowness of movement.[9] Additionally, people with PD experience non-motor symptoms, including fatigue and lack of energy, cognitive issues, and depression.[9] Symptoms typically intensify over time and make everyday tasks increasingly demanding.[9] The prevalence of PD has doubled over the past 25 years.[7] Today, more than 10 million people worldwide are estimated to be living with PD.[10] This makes it the world's second most prevalent neurodegenerative disease.[11] It is also the most frequent movement disorder.[7,12] At present there is no cure, and current treatment options lack the holistic management of symptoms, so there is an urgent need for new therapies.[13]

About AB-1005

AB-1005 is an investigational gene therapy based on an adeno-associated viral vector 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.[14,15] In nonclinical studies, GDNF has been shown to promote the survival and morphological differentiation of dopaminergic neurons, which could aid in the preservation and restoration of dopaminergic neuronal circuitries normally lost in the disease.[15,16] 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.[17] Through a combination of an investigational gene therapy and innovative neurosurgical delivery approach, the GDNF hypothesis in PD can now be tested by getting this neurotrophic factor to these degenerating nigrostriatal neurons in a potentially more clinically relevant fashion.[17]

About the AB-1005 Phase Ib trial

In the AB-1005 Phase Ib, multicenter, open-label, uncontrolled trial, participants were administered AB-1005 to the putamen via one-time bilateral convection-enhanced delivery.[18] 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).[18] The objective of this investigation was to evaluate the safety and potential clinical effect of AB-1005 delivered to the putamen in participants with either mild- or moderate-stage 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.[18] 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 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.[19] The trial will include an estimated 87 participants with trial sites located in Germany, Poland, the United Kingdom, and the

United States.[19] For more information about the REGENERATE-PD clinical trial, visit clinicaltrials.gov (NCT06285643), or visit askbio.com.

About Congestive Heart Failure

Heart failure occurs when the heart cannot pump blood efficiently enough to meet the body's needs, including providing sufficient oxygen to the organs.[20] Congestive heart failure results in the slowing of the blood flow out of the heart, which causes the blood returning to the heart through the veins to back up.[21] This causes congestion in the body's tissues.[22] Symptoms may include shortness of breath, swelling in the legs and ankles caused by fluid retention, and fatigue.[22] More than 64 million people worldwide are estimated to be living with heart failure.[23]

About AB-1002

AB-1002 is an investigational one-time gene therapy based on an adeno-associated viral vector administered to the heart to promote production of a modified version of the therapeutic inhibitor 1 (I-1c) protein designed to block the action of protein phosphatase 1, which is linked to CHF.[24,25] This investigational gene therapy has not been approved by any regulatory authority, and its efficacy and safety have not yet been established or fully evaluated.

About the AB-1002 Phase I trial

This non-randomized, sequential dose-escalation trial (NCT04179643) includes escalating dose cohorts to evaluate the safety and preliminary efficacy of investigational gene therapy AB-1002 in 11 participants with New York Heart Association (NYHA) Class III non-ischemic heart failure with reduced ejection fraction (HFrEF).[2] No adverse events were deemed related to AB-1002 in this trial, and clinically meaningful improvements were recorded across several efficacy assessments.[26] The data further support that AB-1002 may be highly cardiotropic when administered as a single intracoronary injection. Full results were published in October 2025 in the prestigious peer-reviewed journal *Nature Medicine* and are available via open access.[26]

About GenePHIT

GenePHIT is a Phase II adaptive, double-blinded, placebo-controlled, randomized, multi-center trial conducted to evaluate the safety and efficacy of the one-time administration of investigational gene therapy AB-1002, via antegrade intracoronary artery infusion, in males and females age >18 years with non-ischemic cardiomyopathy and New York

Heart Association (NYHA) Class III heart failure symptoms.[27] For more information, please visit euclinicaltrials.eu (EUCT#2024-510581-17-00), clinicaltrials.gov (NCT#05598333), or askbio.com.

GenePHIT is being conducted at 46 locations across the United States, Austria, Germany, the Netherlands, Spain, and the United Kingdom.

About AskBio

AskBio Inc., a wholly owned and independently operated subsidiary of Bayer AG, is a fully integrated gene therapy company dedicated to steering gene therapy into a new era where it can transform the lives of a wider range of people living with rare and more common diseases. The company maintains a portfolio of clinical programs across a range of disease indications related to a single gene or multiple factors across cardiovascular, central nervous system, and neuromuscular conditions, 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 end-to-end gene therapy platform includes our Pro10™ technology and Aava™ manufacturing platform, which make gene therapies more accessible by making research and commercial grade manufacturing more affordable. With global headquarters in Research Triangle Park, North Carolina, 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 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 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.

Forward-Looking Statements AskBio
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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