AskBio Presents Complete Results of Phase 1 Trial of AB-1002 Gene Therapy in Participants with Congestive Heart Failure at European Society of Cardiology Heart Failure Meeting

- *First-in-human trial of AB-1002 designed to evaluate safety and preliminary efficacy in participants with New York Heart Association (NYHA) Class III heart failure*
- *Trial enrolled 11 participants in total, and now includes 12-month data for all participants, including three who entered the trial late due to COVID-19 restrictions*
- *GenePHIT, a Phase 2, adaptive, randomized, double-blind, placebo-controlled trial exploring the safety and efficacy of AB-1002 in non-ischemic heart failure is currently enrolling in Canada, Europe, and the United States*

Research Triangle Park, N.C. – MAY 19, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, will present—during a dedicated late-breaking science session at this year's European Society of Cardiology Heart Failure meeting—the complete dataset from a Phase 1 trial investigating AB-1002 for the treatment of congestive heart failure (CHF). This year's meeting is being held in Belgrade, Serbia, from May 17 to 20, 2025.

This non-randomized, sequential dose escalation trial includes escalating dose cohorts to evaluate the safety and preliminary efficacy of investigational gene therapy AB-1002 in participants with NYHA Class III non-ischemic heart failure with reduced ejection fraction (HFrEF).[1] It is estimated that 64 million people worldwide are living with heart failure, and despite standard of care, mortality and morbidity in heart failure remain very high.[2,3]

The late-breaking oral presentation highlights that single-dose administration of AB-1002 at 12 months post-dose resulted in the following outcomes:[4]

- No treatment-emergent or serious adverse events were deemed related to trial treatment.
- Among the six participants in Cohort 1 receiving a lower dose, five showed clinically meaningful improvements in left ventricular ejection fraction (LVEF). Five participants showed clinically meaningful improvements in NYHA Functional Class (NYHA FC), with one showing a stabilization in symptoms. Three showed clinically meaningful improvements in Minnesota Living with Heart Failure Questionnaire (MLHFQ) results, with one showing stabilization and one showing a worsening of symptoms. Two showed improvements in cardiopulmonary exercise test (VO_2 max) results, with two showing stabilization. Three showed an improvement in 6-minute walk test (6MWT) results, with two showing stabilization.
- Among the five participants in Cohort 2 receiving a higher dose, two showed clinically meaningful improvements in MLHFQ results and NYHA FC, and three showed clinically meaningful improvements in LVEF compared with baseline.

Meaningful changes from baseline were considered to be: NYHA FC, ≥1 point; LVEF, ≥5%; MLHFQ, ≥10 points; VO_2 max, ≥1.5 mL/kg/min; 6MWT, 30 meters.

"No treatment-emergent or serious adverse events were deemed related to AB-1002 in this trial, and we saw clinically meaningful improvement of several efficacy assessments in some participants with non-ischemic CHF," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. "These results support our belief that the AB-1002 chimeric capsid may be highly cardiotropic when administered as a single intracoronary injection at relatively low doses, and we're

excited to further evaluate the safety and efficacy of AB-1002 in GenePHIT, our currently recruiting Phase 2 trial."

Innovative therapies are urgently needed to address the underlying mechanisms associated with CHF, including improving pump function in participants with HFrEF.[5] AB-1002 is a rationally designed cardiotropic adeno-associated virus (AAV) vector delivering a transgene for continuous onsite expression of the inhibitor 1 (I-1c), inhibitor of protein phosphatase 1, a protein that has been linked to heart failure.

This Phase 1 trial is ongoing, and participants will be followed for 36 months post-intervention. Preliminary results of this trial, which did not include the additional three participants, were presented at the American Heart Association Scientific Sessions in November of 2023.[6]

AB-1002 is an investigational gene therapy that has not been approved by any regulatory authority, and its efficacy and safety have not been fully established or evaluated.

About AB-1002

AB-1002 is a one-time gene therapy administered to the heart to help promote increased 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.[6,7] 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 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.[8] 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.[9] This causes congestion in the body's tissues.[10] Symptoms may include shortness of breath, swelling in the legs and ankles caused by fluid retention, and fatigue.[10] More than 64 million people worldwide are estimated to be living with heart failure.[2]

About GenePHIT

GenePHIT is a Phase 2 adaptive, double-blinded, placebo-controlled, randomized, multi-center trial conducted to evaluate the safety and efficacy of the one-time administration of AB-1002, via antegrade intracoronary artery infusion, in males and females age >18 years with non-ischemic cardiomyopathy and NYHA Class III heart failure symptoms.[1] For more information, please visit euclinicaltrials.eu (EUCT#2024-510581-17-00), clinicaltrials.gov (**NCT#05598333**), or askbio.com.

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, 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 **http://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.

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