AskBio Announces Publication in *Nature Medicine* of 12-month data from Phase 1 Trial of AB-1002 Gene Therapy in Participants with Congestive Heart Failure

- *First-in-human trial of investigational gene therapy AB-1002 designed to evaluate safety and preliminary efficacy in participants with New York Heart Association (NYHA) Class III heart failure*
- *No adverse events were deemed related to AB-1002, and clinically meaningful improvement of several efficacy assessments was seen in participants with non-ischemic congestive heart failure (CHF)*
- *Phase 2 GenePHIT trial is currently enrolling in Canada, Europe, the United Kingdom, and the United States*

Research Triangle Park, N.C.– October 21, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced the publication in peer-reviewed journal *Nature Medicine* of 12-month data from its Phase 1 trial of AB-1002 investigational gene therapy in participants with congestive heart failure (CHF).[1]

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 participants with New York Heart Association (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 remain very high.[2,3]

The publication, which is available online, confirms that no adverse events were deemed related to AB-1002 in this trial and that clinically meaningful improvements were recorded across several efficacy assessments in participants with non-ischemic CHF.[1] The data further support that the AB-1002 capsid may be highly cardiotrophic when administered as a single intracoronary injection.

AskBio thanks the participants who volunteered for this important clinical trial, the sites that made this effort possible, and the skilled investigators who conducted this invaluable research and contributed to the scientific body of knowledge related to AB-1002.

"We believe there is a critical need to progress innovative therapies that target the root causes of congestive heart failure, so we're pleased to see these data for AB-1002 published and shared with the scientific community via *Nature Medicine*, a high-impact peer-reviewed journal," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer at AskBio. "We're eager to further assess the safety and efficacy of AB-1002 in our ongoing Phase 2 trial, GenePHIT, which is currently enrolling in Canada, Europe, the United Kingdom, and the United States, and look forward to sharing those results once available."

GenePHIT is a Phase 2, adaptive, randomized, double-blind, placebo-controlled trial investigating the safety and efficacy of AB-1002 in non-ischemic heart failure.

About AB-1002

AB-1002 is an investigational one-time gene therapy 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.[4,5] 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.[6] 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.[7] This causes congestion in the body's tissues.[8] Symptoms may include shortness of breath, swelling in the legs and ankles caused by fluid retention, and fatigue.[8] 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 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.[9] 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, which makes 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 http://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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