First Participant Randomized in Europe in AskBio Phase 2 Gene Therapy Trial for Congestive Heart Failure

- *AB-1002 is an investigational gene therapy for the treatment of adults with non-ischemic cardiomyopathy and New York Heart Association (NYHA) Class III heart failure symptoms*
- *GenePHIT is a clinical trial to evaluate the safety and efficacy of the one-time administration of AB-1002 and will include the largest number of participants to date to receive this investigational therapy*

Research Triangle Park, NC, USA, February 13, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the first participant has been randomized in Europe in GenePHIT (Gene PHosphatase Inhibition Therapy), a Phase 2 clinical trial of AB-1002 for the treatment of congestive heart failure (CHF). This European milestone follows the announcement of the first participant randomized in the US arm of GenePHIT in 2024.

GenePHIT is an adaptive, double-blind, placebo-controlled, randomized, multicenter trial to evaluate the safety and efficacy of a single intracoronary infusion of AB-1002 in adults with non-ischemic cardiomyopathy and New York Heart Association (NYHA) Class III heart failure symptoms who have been medically stable for at least four weeks.[1] This clinical milestone in the development of AB-1002 potentially brings this investigational one-time gene therapy one step closer to treating patients in Europe with high unmet medical need.[2]

GenePHIT will include between 90 and 150 adults with heart failure with reduced ejection fraction (HFrEF) between 15 and 35 percent who continue to suffer from symptoms despite guideline-recommended therapy.[1] The study is enrolling participants across clinical centers in the United States and Europe (Austria, Germany, Hungary, Netherlands, Spain, UK).

The successful randomization of the first participant in Europe, which happened in Spain, is an important landmark in the Phase 2 GenePHIT clinical trial, which will include the largest number of participants to receive AB-1002 to date.[1]

“Cardiovascular disease is the most common cause of death in Europe, claiming nearly four million lives each year, and it accounts for 45 percent of all deaths in females and 39 percent of all deaths in males,” said Timothy D. Henry, MD, MSCAI, GenePHIT Principal Investigator and Steering Committee Member. “As society ages, the burden heart failure places on individuals and healthcare systems will only increase unless a solution is found. Today’s announcement is important as it represents meaningful progress in the development of a potentially disease-modifying therapy.”

“The potential of gene therapy to address the intracellular abnormalities that characterize heart failure is immense, and we are excited to have achieved this critical milestone for the GenePHIT study in Europe,” said Canwen Jiang MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. “Today’s news demonstrates AskBio’s ability to advance AB-1002 gene therapy for the potential treatment of congestive heart failure, which is deadly and devastating for affected patients and has a significant impact on their loved ones.”

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

About GenePHIT

GenePHIT is a Phase 2 adaptive, double-blinded, placebo-controlled, randomized, multi-center trial 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 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.[3] 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.[4] This causes congestion in the body's tissues.[5] Symptoms may include shortness of breath, swelling in the legs and ankles caused by fluid retention, and fatigue.[5] More than 64 million people worldwide are living with congestive heart failure.[6]

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, 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 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 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.

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