AskBio Presents Baseline Characteristics from Phase 2 Heart Failure Gene Therapy Trial of Umiposgene Parvec (AB-1002) at European Society of Cardiology Congress

- *Phase 2 GenePHIT trial successfully randomized and completed enrollment of one of the largest gene therapy trials conducted in heart failure to date, with more than 170 participants randomized across 64 sites in 12 countries*[1]
- *Baseline characteristics demonstrate successful enrollment of a well-characterized population of patients with non-ischemic heart failure with reduced ejection fraction (HFrEF)*
- *Initial efficacy and safety outcomes from GenePHIT are expected in the first half of 2027*

Durham, N.C., – August 29, 2026 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced the presentation of baseline participant characteristics from GenePHIT, one of the largest randomized gene therapy trials conducted in heart failure to date. This presentation highlights the trial's successful enrollment and further validates that multinational gene therapy trials of this size can be conducted. The Phase 2 clinical trial is evaluating the investigational one-time gene therapy umiposgene parvec (AB-1002) for its potential to improve cardiac function. Baseline characteristics from the trial are being presented at the European Society of Cardiology (ESC) Congress, which takes place in Munich, Germany, August 28–31, 2026.

The baseline characteristics provide an overview of the population enrolled in GenePHIT and demonstrate the successful recruitment of a representative non-ischemic heart failure with reduced ejection fraction (HFrEF) population receiving guideline-directed medical therapy.[1] GenePHIT is a randomized, double-blind, placebo-controlled, multicenter Phase 2 trial designed to evaluate the efficacy, safety, and tolerability of umiposgene parvec following a direct infusion to the heart, with no immune suppression, via a standard catheter.[2]

"Despite remarkable advances in heart failure therapy, many patients continue to experience progressive ventricular dysfunction, recurrent hospitalization, and premature death," said Timothy D. Henry, MD, MSCAI, GenePHIT Principal Investigator and Steering Committee Member. "GenePHIT represents an important milestone for the field because it demonstrates that successful enrollment of large, multinational randomized gene therapy trials in heart failure is feasible. The enrolled population closely reflects contemporary patients receiving current standard of care, providing an excellent foundation to evaluate whether a one-time investigational gene therapy can meaningfully improve outcomes."

GenePHIT randomized more than 170 participants with umiposgene parvec or placebo.[1] GenePHIT participants were treated with established heart failure therapies, including beta-blockers, angiotensin receptor-neprilysin inhibitors, sodium-glucose cotransporter 2 inhibitors, and mineralocorticoid receptor antagonists.[1]

"We are pleased to share these baseline characteristics from GenePHIT, which represents an important milestone in the advancement of our umiposgene parvec program for heart failure," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer at AskBio. "Having 64 sites in 12 countries demonstrates the feasibility of evaluating this investigative approach in a representative population. These baseline characteristics establish a strong foundation for interpreting the initial efficacy and safety outcomes, which are expected in the first half of 2027 and will further inform the continued development of umiposgene parvec as a potential treatment for patients with heart failure."

GenePHIT participants were treated across 64 sites in multiple countries, including Canada, the United States, and the United Kingdom, as well as several in the European Union. The clinical trial is among the largest to evaluate intracoronary administration of gene therapy in patients with heart failure.[2] The enrolled population included participants with significant disease burden, with nearly half having a history of atrial fibrillation and almost 45% having an implantable cardioverter defibrillator.[1]

Umiposgene parvec has not been approved by any regulatory authority, and its safety and efficacy have not been established.

About umiposgene parvec (AB-1002)
Umiposgene parvec is an investigational one-time gene therapy administered directly to the heart to promote production of a modified version (I-1c) of the naturally occurring protein inhibitor-1, designed to block the action of protein phosphatase 1, which is linked to heart failure, with the goal of improving cardiac contractility by restoring intracellular calcium signaling.[3,4]

About 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.[5] This causes congestion in the body's tissues.[6] Symptoms may include shortness of breath, swelling in the legs and ankles caused by fluid retention, and fatigue.[6] More than 64 million people worldwide are estimated to be living with heart failure.[7]

About GenePHIT
GenePHIT is a Phase 2 adaptive, double-blinded, placebo-controlled, randomized, multi-center trial conducted to evaluate the efficacy, safety, and tolerability of the one-time administration of investigational gene therapy umiposgene parvec, via antegrade intracoronary artery infusion, in males and females age >18 years with non-ischemic cardiomyopathy, New York Heart Association (NYHA) Class III heart failure symptoms, and reduced ejection fraction.[2] The trial was designed to evaluate cardiovascular-related deaths and change from baseline in NYHA classification, left ventricular ejection fraction, and six-minute walking distance.[2] GenePHIT participants were randomized at 64 locations across the United States, Canada, Austria, Germany, the Netherlands, Spain, Belgium, Hungary, Poland, Bulgaria, Romania, and the United Kingdom.[2] 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 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 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 Durham, 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 2025, the Group employed around 88,000 people and had sales of 45.6 billion euros. R&D expenses amounted to 5.8 billion euros. For more information, go to **www.bayer.com**.

Media Contact:
Phil McNamara
Vice President, Corporate Communications, AskBio
E: **pmcnamara@askbio.com**
T: +1 (984) 520-7211

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.

References

[1] Henry T, et al. GenePHIT Phase 2 baseline characteristics: a double blind, placebo-controlled trial to assess safety and efficacy of AB-1002 gene therapy in heart failure with reduced ejection fraction. Presented at European Society of Cardiology Congress, August 2026.

[2] Phosphatase Inhibition by Intracoronary Gene Therapy in Subjects With Non-Ischemic NYHA Class III Heart Failure (GenePHIT). Available at: https://clinicaltrials.gov/study/NCT05598333. Last accessed: August 2026.

[3] Henry T, Chung E, Alvisiet M, et al. Preliminary safety and efficacy of a Phase 1 clinical gene therapy trial in patients with advanced heart failure using a rationally designed cardiotropic AAV vector targeting Protein Phosphatase Inhibitor-1. Presented at American Heart Association Scientific Sessions, November 2023.

[4] Nicolaou P & Kranias E. Role of PP1 in the regulation of Ca cycling in cardiac physiology and pathophysiology. Front Biosci (Landmark Ed). 2009 Jan 1;14(9):3571-85.

[5] Centers for Disease Control and Prevention. Heart failure. Published 2024. Available at: https://www.cdc.gov/heart-disease/about/heart-failure.html. Last accessed: August 2026.

[6] American Heart Association. Heart Failure Signs and Symptoms. Available at: https://www.heart.org/en/health-topics/heart-failure/warning-signs-of-heart-failure. Last accessed: August 2026.

[7] Savarese G, Becher PM, Lund LH, et al. Global burden of heart failure: a comprehensive and updated review of epidemiology. Cardiovasc Res. 2023 Jan 18;118(17):3272-3287.