

News Release

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First patient randomized in AskBio Phase II 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 symptoms¹
 - 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 patients to date to receive this therapy¹
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Berlin, Germany and Research Triangle Park, NC, USA, February 13, 2024 – Bayer AG and Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the first patient has been randomized in GenePHIT (Gene PHosphatase Inhibition Therapy), a Phase II trial of AB-1002 (also known as NAN-101) for the treatment of congestive heart failure (CHF).

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. This milestone in the development of AB-1002 for the treatment of CHF potentially brings this investigational therapy one step closer to treating patients with high unmet medical need.²

GenePHIT will include between 90 and 150 adults with left ventricular ejection fraction between 15 and 35 percent, who continue to suffer from heart failure symptoms despite guideline recommended therapy. The primary efficacy endpoint at 52 weeks is a modified win ratio of several clinically meaningful assessments.¹

“The randomization of the first patient as a part of the Phase II GenePHIT trial is an important moment for the heart failure community,” said Timothy D. Henry, MD, MSCAI, Principal Investigator and Steering Committee Member. “GenePHIT will evaluate the safety and efficacy of AB-1002 in the largest number of patients to date and improve our understanding of gene therapy overall for the treatment of congestive heart failure. The initiation of this trial brings us a step closer to potentially changing the course of this deadly and devastating disease.”

“Being able to announce this important GenePHIT trial update during Heart Failure Awareness Week adds special significance to this milestone,” said Roger J. Hajjar, MD, Scientific Chair CHF, AskBio. “The enrollment of this first patient in the Phase II trial represents the culmination of many years of dedicated research and development in all aspects of cardiac gene therapy for congestive heart failure. Although there is still much to learn about this early-stage investigational gene therapy, we hope today’s announcement, which highlights AskBio’s ability to advance AB-1002 gene therapy for the treatment of congestive heart failure, is encouraging news for everyone hoping to see new treatment options.”

“Heart failure is a devastating disease with increasing unmet medical need, especially in a progressively aging population,” said Christian Rommel, PhD, Member of the Executive Committee of Bayer’s Pharmaceuticals Division and Head of Research and Development. “The potential impact of gene therapy to address this disease at its root cause is immense, and we are thrilled about this step in our path to deliver truly innovative treatment options for patients.”

AB-1002 is an investigational gene therapy that has not received marketing authorization, and its efficacy and safety have not been established or fully evaluated. AB-1002 is manufactured by Viralgen Vector Core, S.L., a wholly owned and independently operated subsidiary of AskBio.

About GenePHIT

GenePHIT is a Phase II 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. Fehler! Textmarke nicht

definiert. Subjects are randomized into one of three treatment groups in a 1:1:1 fashion to either low dose, high dose, or placebo. Primary outcome measures include cardiovascular related death and change from baseline in NYHA classification, left ventricular ejection fraction (LVEF), peak oxygen uptake (pVO₂), and Six Minute Walk Test (6MWT).^{Fehler!}
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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. This causes congestion in the body's tissues. Symptoms include swelling in the legs and ankles. Sometimes, fluid collects in the lungs and interferes with breathing.⁴ Approximately 26 million people worldwide are living with congestive heart failure.⁵

About AskBio

Asklepios BioPharmaceutical, Inc. (AskBio), 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 therapeutics for congestive heart failure, Huntington's disease, 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 capsid and promoter library. 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 800 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. Its 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 2022, the Group employed around 101,000 people and had sales of 50.7 billion euros. R&D expenses before special items amounted to 6.2 billion euros. For more information, go to www.bayer.com.

About Viralgen Vector Core

Viralgen is a Contract Development and Manufacturing Organization (CDMO) founded in 2017 and exists as an independently operated subsidiary of AskBio, which is wholly owned and independently operated as a subsidiary of Bayer AG. As a manufacturer of Current Good Manufacturing Practice (cGMP) certified AAV, Viralgen offers the Pro10™ based suspension manufacturing platform, a technology licensed from AskBio and developed by Chief Technical Officer Josh Grieger, PhD, and Co-Founder R. Jude Samulski, PhD, at University of North Carolina. The Pro10™ platform has been found to increase scalability, performance, and yield of AAV therapies.⁶ Located in Spain, in the Gipuzkoa Science and Technology Park, Viralgen produces AAV gene therapy treatments for pharmaceutical and biotech companies with the aim of accelerating the delivery of new treatments that may improve patients' lives.

The company's clinical facilities have four cGMP manufacturing suites, with 250-liter and 500-liter bioreactors. In 2020, Viralgen expanded within the Scientific Park by constructing a new building with three modules for large-scale commercial manufacturing. Each module of the state-of-the-art space includes three cGMP suites with a manufacturing capacity of >2,000 liters. The first module, which includes a suite dedicated to fully automated fill and finish operations, has received cGMP certification by the Spanish Agency for Medicines and Medical Devices (AEMPS) as part of the EMA network. For more information, visit viralgenvc.com.

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Find more information about AskBio at www.askbio.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.

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.

¹ Phosphatase Inhibition by Intracoronary Gene Therapy in Subjects With Non-Ischemic NYHA Class III Heart Failure (GenePHIT). Available at: <https://classic.clinicaltrials.gov/ct2/show/NCT05598333>. Accessed February 2024.

² Malik A, Brito D, Vaqar S, Chhabra L. Congestive Heart Failure. In: StatPearls. Treasure Island (FL): StatPearls Publishing; November 5, 2023.

³ CDC. Heart failure. Centers for Disease Control and Prevention. Published 2022. Available at: https://www.cdc.gov/heartdisease/heart_failure.htm. Accessed February 2024.

⁴ AHA. Types of Heart Failure. American Heart Association. Published 2023. Available at: <https://www.heart.org/en/health-topics/heart-failure/what-is-heart-failure/types-of-heart-failure>. Accessed February 2024.

⁵ Savarese G, Lund LH. Global Public Health Burden of Heart Failure. Card Fail Rev. 2017;3(1):7-11. doi:10.15420/cfr.2016:25:2..

⁶ Grieger JC, Soltys SM, Samulski RJ. Production of Recombinant Adeno-associated Virus Vectors Using Suspension HEK293 Cells and Continuous Harvest of Vector From the Culture Media for GMP FIX and FLT1 Clinical Vector. Mol Ther. 2016;24(2):287-297.