Bayer AG
Communications
51368 Leverkusen
Germany
Phone +49 214 30-1
www.bayer.com/en/media

News Release

Not intended for UK Media

AskBio receives FDA Fast Track Designation for AB-1002 investigational gene therapy program in congestive heart failure

- AB-1002 is being studied for the treatment of adults with non-ischemic cardiomyopathy and New York Heart Association (NYHA) Class III heart failure symptoms
- AskBio is currently enrolling patients in the Phase II GenePHIT trial of AB-1002 for the treatment of congestive heart failure

Berlin, Germany, and Research Triangle Park, NC, USA, April 18, 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 U.S. Food and Drug Administration (FDA) has granted Fast Track Designation for the AB-1002 program. AB-1002 is an investigational one-time gene therapy that is administered to the heart with the intention of helping to promote the production of a constitutively active form of protein inhibitor 1 (I-1c) designed to block the action of protein phosphatase 1. Inhibiting the function of this protein, which is linked to congestive heart failure (CHF), could potentially lead to a therapeutic effect on the heart.[1,2]

“The FDA Fast Track Designation for AB-1002 is an important accomplishment for the clinical development of this program and highlights our goal of bringing potentially effective treatments to patients with advanced congestive heart failure,” said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. “We look forward to completing our Phase II GenePHIT clinical trial, which is currently enrolling patients with severe heart failure, and are committed to exploring the full potential of AB-1002 for the treatment of this devastating disease.”

The FDA Fast Track Program is designed to facilitate the development and expedite the review of new therapeutics that are intended to treat serious conditions and fill unmet medical needs.[3] The purpose of the Program is to get important new therapeutics to patients earlier.[3]

Therapeutics that receive this designation benefit from eligibility for more frequent meetings with the FDA to discuss the development plan and, if relevant criteria are met, eligibility for Accelerated Approval and Priority Review.

“The Fast Track Designation for AB-1002 emphasizes the need to rapidly advance new therapeutic modalities such as gene therapy for people living with congestive heart failure,” said Christian Rommel, PhD, Head of Research and Development at Bayer’s Pharmaceuticals Division. “This designation underpins the potential of AB-1002 to address currently high unmet medical need, and we are excited about the opportunity to accelerate its development.”

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. AskBio is currently enrolling patients in the Phase II GenePHIT (Gene Phosphatase Inhibition Therapy) trial of AB-1002 (also known as NAN-101) for the treatment of CHF.[4]

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 New York Heart Association (NYHA) Class III heart failure symptoms.[4] 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).[4] For more information, please visit clinicaltrials.gov (NCT#05598333) or visit 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.[5] 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.[6] 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. Approximately 26 million people worldwide are living with congestive heart failure.[7]

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. 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 Media Contact:
Phil McNamara, phone +1 (984) 5207211
Email: pmcnamara@askbio.com

Bayer Global Media Contact:
Dr. Nuria Aiguabella Font, phone +49 (173) 2329691
Email: nuria.aiguabellafont@bayer.com

Bayer U.S. Media Contact:
Carolyn Nagle, phone +1 (201) 4190337
Email: carolyn.nagle@bayer.com

Find more information about AskBio at www.askbio.com
Find more information about Bayer at https://pharma.bayer.com/
Follow Bayer on Facebook: http://www.facebook.com/bayer
Follow Bayer on Twitter: @BayerPharma

naf (2024-0069e)

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.

---

[1] Fish KM, Ladage D, Kawase Y, et al. AAV9.I-1c delivered via direct coronary infusion in a porcine model of heart failure improves contractility and mitigates adverse remodeling. Circ Heart Fail. 2013;6(2):310-317.
[2] Pathak A, del Monte F, Zhao W, et al. Enhancement of cardiac function and suppression of heart failure progression by inhibition of protein phosphatase 1. Circ Res. 2005;96(7):756-766.
[3] US FDA – Fast Track. Available at: https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/fast-track. Accessed April 2024.
[4] 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 April 2024.
[5] CDC. Heart failure. Centers for Disease Control and Prevention. Published 2022. Available at: https://www.cdc.gov/heartdisease/heart_failure.htm. Accessed April 2024.
[6] American Heart Association: Types of Heart Failure. Available at: https://www.heart.org/en/health-topics/heart-failure/what-is-heart-failure/types-of-heart-failure. April 2024
[7] 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.