AskBio Presents Preliminary Data from Phase 1 Trial of Gene Therapy for Congestive Heart Failure (CHF) at the 2023 American Heart Association Scientific Sessions

- *First-in-human trial of AB-1002 designed to establish safety and preliminary efficacy in patients with NYHA Class III Heart Failure*

- *Heart failure is a leading cause of morbidity and mortality worldwide despite available therapies, and quality of life is poor.*

- *It is estimated 26 million worldwide are living with heart failure.*

Research Triangle Park, N.C. – November 12, 2023 – Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today presented first-in-human data from a Phase 1 trial investigating AB-1002 (also known as NAN-101) for the treatment of patients with congestive heart failure (CHF) at this year’s American Heart Association Scientific Sessions, which are being held in Philadelphia, U.S., from November 11 to 13, 2023.3,4

The trial was designed to establish the safety and preliminary efficacy of gene therapy AB-1002 in patients with NYHA Class III Heart Failure, also known as advanced heart failure.4,5 AB-1002 is a rationally designed cardiotropic AAV vector targeting protein phosphatase inhibitor-1, which has been linked to heart failure.6,7

“We believe these encouraging early results in patients with advanced heart failure are important for the congestive heart failure community, as they bring hope to a sub-population where treatment options are needed,” said Litsa G. Kranias, PhD, Hanna Chair of Cardiology at the University of Cincinnati and US Coordinator, Cure-PLaN. “Seeing the potential of gene therapy being explored in heart failure is a key step forward in one day potentially changing the direction of this devastating disease, which is a leading cause of morbidity and mortality in westernized countries.”

Single-dose administration of AB-1002 resulted in clinically meaningful improvements in key efficacy parameters:3

- Among the six patients in Cohort 1, three who completed 12-month follow-up showed clinically meaningful improvements in left ventricular ejection fraction (LVEF), NYHA Functional Class (NYHA FC), Minnesota Living with Heart Failure Questionnaire (MLHFQ), cardiopulmonary exercise test (VO₂ max), and 6-minute walk test (6MWT) at 12 months post-dose. The three remaining patients in Cohort 1 are still within the first 3 months post-injection.

- Among the five patients* in Cohort 2, two showed clinically meaningful improvements in MLHFQ and NYHA FC, and four (100%) showed clinically meaningful improvements in LVEF at 12 months, compared with baseline. Meaningful changes from baseline were considered to be NYHA FC, ≥1 point; LVEF, ≥5%; MLHFQ, ≥10 points; VO₂ max, ≥1.5 mL/kg/min; 6MWT, 30 meters.
* only 4 of the 5 patients in Cohort 2 could be evaluated due to a fatal serious adverse event that was not deemed related to study treatment.

No treatment emergent adverse events (TEAEs) or serious adverse events (SAEs) were deemed related to study treatment.

Timothy D. Henry, MD, MSCAI, Principal Investigator and Steering Committee Member, commented, “Although current management has improved survival in CHF patients, most therapies do not treat the underlying causes, consequently the current standard of care does not reverse the trajectory of the disease to ultimate end-stage heart failure and death. These results show delivery of AB-1002 was well tolerated and resulted in positive efficacy outcomes in some patients with non-ischemic congestive heart failure.”

Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio, added, “At AskBio we are committed to our mission of transforming gene therapies from idea to impact. These results further validate that the AAV2i8 vector capsid is highly cardiotropic when injected through intracoronary means at relatively low doses. This is encouraging as it shows the exciting potential for gene therapy to reduce the considerable burden of congestive heart failure in a sub-population of heart failure patients where the current prognosis is particularly poor.”

About AB-1002

AB-1002 is a one-time gene therapy administered to the heart to help promote increased production of a therapeutic protein inhibitor 1 (I-1c) designed to block the action of protein phosphatase 1, which is linked to CHF. 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 happens when the heart cannot pump enough blood and oxygen to support other organs in your body. 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. Approximately 26 million people worldwide are living with congestive heart failure.

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.

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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