First Participant Dosed in AskBio Phase 1/Phase 2 Gene Therapy Trial of AB-1009 for Late-Onset Pompe Disease (LOPD)

- *AB-1009, an adeno-associated virus (AAV) investigational gene therapy, is being evaluated for the treatment of late-onset Pompe disease (LOPD) in the PROGRESS-GT LOPD trial*
- *AskBio initiated recruitment in the United States earlier in 2026, with enrollment ongoing*

Durham, N.C. – May 11, 2026 – 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 dosed with investigational gene therapy AB-1009 as part of PROGRESS-GT LOPD, a Phase 1/Phase 2 clinical trial in participants with late-onset Pompe disease (LOPD).

LOPD is a rare, progressive, debilitating genetic disorder that is estimated to affect at least 5,000 to 10,000 people worldwide.[1] While there are multiple approved enzyme replacement therapies (ERTs) with recombinant human acid alpha-glucosidase (rhGAA), there remains a strong level of unmet medical need, as some individuals receiving ERT may experience a decrease in clinical response over time, which can contribute to overall increased treatment demands.[2-5]

PROGRESS-GT LOPD (NCT07282847) is evaluating the safety, tolerability, and efficacy of AB-1009 in adult participants with LOPD.[6] PROGRESS-GT LOPD is estimated to enroll 12 participants across the United States.[6]

“Current treatment approaches may not fully meet the long-term needs of patients, and ongoing research into potential new options remains critical,” said Tahseen Mozaffar, MD, Director of the UCI Health ALS & Neuromuscular Center, and Principal Investigator, AB-1009 Clinical Trial Program. “We look forward to advancing the PROGRESS-GT LOPD trial and generating data with the potential to help inform the future of care in the Pompe community.”

AB-1009 was granted United States Food and Drug Administration (FDA) Fast Track and Orphan Drug designations.[7] The FDA Fast Track process 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.[8] The purpose of the process is to get important new therapeutics to patients earlier.[8] Therapeutics that receive this designation benefit from eligibility for more frequent meetings with the FDA to discuss the clinical development plan and, if relevant criteria are met, eligibility for Accelerated Approval and Priority Review. Orphan Drug Designation provides orphan status to drugs and biologics for rare diseases that meet certain criteria and potentially gives a company exclusive marketing rights for a seven-year period, along with other benefits.[9] Additionally, in January, AskBio announced that the FDA accepted the investigational new drug application for AB-1009.[7]

“Reaching this milestone reflects the momentum behind our gene therapy platform and our commitment to working toward improving quality of life for people with rare diseases,” said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer at AskBio. “The initiation of PROGRESS-GT LOPD marks an important step in the development of AB-1009 and demonstrates our commitment to moving our clinical program forward with determination, to investigate a potential new treatment approach for people living with Pompe disease.”

AB-1009 is an investigational gene therapy that has not been approved by any regulatory authority, and its efficacy and safety have not been fully established or evaluated.

About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA).[2] Reduced or absent levels of GAA lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of the disease.[2] Pompe can be debilitating and is characterized by severe muscle weakness that worsens over time and is accompanied by diaphragmatic involvement, leading to respiratory insufficiency early in the course of the disease.[2,3] Pompe disease ranges from a rapidly fatal infantile form, with significant impacts to heart function, to a more slowly progressive, late-onset form primarily affecting skeletal muscle.[2] It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.[1]

About PROGRESS-GT LOPD

PROGRESS-GT LOPD is a non-randomized, single-arm, open-label, dose-escalation clinical trial, and evaluates the safety, tolerability, and efficacy of investigational gene therapy AB-1009 in adult participants with LOPD.[6] PROGRESS-GT LOPD is estimated to enroll approximately 12 participants across the United States.[6] For more information about the PROGRESS-GT LOPD clinical trial, visit clinicaltrials.gov (NCT07282847), or visit askbio.com.

About AB-1009

AB-1009 is an investigational adeno-associated virus (AAV)-based gene therapy being studied for its potential to address LOPD's underlying genetic defect and explore its ability to increase production of the deficient enzyme in people with this disease.[7] It is designed to target the disease's underlying cause by enabling sustained production of acid alpha-glucosidase (GAA), the enzyme that is deficient in affected patients.[7]

AskBio thanks Genethon, Belief BioMed, and Duke University for their early contributions to AskBio's Pompe disease program.

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.

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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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