AskBio Announces FDA Acceptance of Investigational New Drug (IND) Application for AB-1009 Gene Therapy for Treatment of Late-Onset Pompe Disease (LOPD)

- *AB-1009 program advances to Phase 1/Phase 2; clinical trial initiated in the United States*
- *Therapy granted United States Food and Drug Administration (FDA) Fast Track and Orphan Drug designations*
- *Pompe disease is a rare genetic metabolic disorder that without early detection and treatment can lead to premature death*

Durham, N.C. – JANUARY 8, 2026 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that the United States Food and Drug Administration (FDA) has accepted its Investigational New Drug (IND) application for AB-1009, an adeno-associated virus (AAV) gene therapy being developed for the treatment of late-onset Pompe disease (LOPD). With this announcement, the AB-1009 program advances to Phase 1/Phase 2, and AskBio has initiated a clinical trial in the United States to explore the safety of AB-1009. The company anticipates recruiting its first patient in early 2026.

"This investigational gene therapy is being studied for its potential to address the underlying genetic defect and to explore whether it can increase production of the deficient enzyme in patients with Pompe disease," said Tahseen Mozaffar, MD, Director of the UCI Health ALS & Neuromuscular Center, and Principal Investigator, AB-1009 Clinical Trial Program. "Patients receiving gene therapy may reduce reliance on exogenous enzyme replacement. AskBio's approach leverages its experience in gene therapy development as it seeks to advance treatment options for Pompe disease."

In addition to the initiation of the AB-1009 PROGRESS-GT LOPD (NCT07282847) clinical trial program, the therapy was recently granted FDA Fast Track and Orphan Drug designations. 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.[1] The purpose of the process is to get important new therapeutics to patients earlier.[1] 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.[2]

"These advancements in the AB-1009 program, particularly the recently granted regulatory designations, highlight the recognized need for late-onset Pompe treatments," said Mansuo Shannon, PhD, Chief Scientific Officer, AskBio. "Today's news demonstrates AskBio's commitment to progressing early-stage assets into the clinic and adding those to our clinical portfolio."

Pompe disease is a rare, progressive, debilitating genetic disorder that is estimated to affect at least 5,000 to 10,000 people worldwide.[3] Pompe disease ranges in severity from infantile-onset Pompe disease (IOPD) to LOPD.[4] LOPD is associated with significant morbidity and is characterized by progressive skeletal muscle weakness and respiratory insufficiency.[5] Patients typically present with progressive proximal myopathy; however, respiratory involvement can be the primary presenting clinical feature.[5] This causes severe muscle weakness and wasting, leading to the loss of mobility.[5] The disease can lead to premature death from respiratory failure.[5] Today, there are multiple approved

enzyme replacement therapies (ERTs) with recombinant human acid alpha-glucosidase (rhGAA), and these are chronically administered.[4,5] These can also be used in combination with a small-molecule pharmacological chaperone treatment.[6] There remains an 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.[7]

The advancement of AB-1009 was achieved in collaboration with Belief BioMed Inc., Genethon, and Duke University; and AskBio thanks these collaborators.

AskBio's clinical trial of ACTUS-101 (NCT03533673) in participants with LOPD will remain active but no longer recruit. The trial will be completed with the patients currently enrolled.

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

About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA).[4] Reduced or absent levels of GAA lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease.[4] The disease 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.[4,5] 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.[4] It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.[3]

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 congestive 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 2024, the Group employed around 93,000 people and had sales of 46.6 billion euros. R&D expenses amounted to 6.2 billion euros. For more information, go to **http://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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