AskBio Advances Gene Therapy Clinical Trial for Limb-Girdle Muscular Dystrophy Type 2I/R9 with Dosing of First Participant in Second Cohort

- *Data Safety Monitoring Board (DSMB) recommendation to initiate second cohort follows review of Phase 1/Phase 2 LION-CS101 trial safety data for AB-1003*
- *Second cohort enrollment is ongoing*

Research Triangle Park, N.C. – March 7, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced the advancement of the Phase 1/Phase 2 LION-CS101 clinical trial of investigational gene therapy AB-1003 in patients with limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9) with dosing of the first participant in the second cohort. The recommendation to advance to the second cohort followed the completion of a Data Safety Monitoring Board (DSMB) review of trial recruitment activity, and safety reporting from the first cohort that determined it was safe to proceed to cohort two. DSMBs are independent groups of experts appointed to periodically review information from clinical trials. These reviews typically include risk-benefit assessments and monitoring for serious or unexpected adverse safety events alongside any significant benefit of therapies.[1]

The LION-CS101 clinical trial is a double-blind, randomized, placebo-controlled, dose-escalation clinical trial to evaluate the safety of AB-1003 gene therapy in adult participants (18–65 years) who have genetic confirmation of LGMD2I/R9. The trial includes two sequential, dose-level cohorts. Adult participants diagnosed with LGMD2I/R9 will be given a single intravenous infusion of AB-1003 or placebo.

The trial was initiated in 2023. It will include up to 14 participants at six sites throughout the United States.[2] Participants in the first cohort remain in the study until completion. Enrollment in cohort two is ongoing. For more information about the LION-CS101 clinical trial, visit clinicaltrials.gov (NCT05230459) or askbio.com.

“The burden of LGMD2I/R9 on patients and their families is profound,” said Nicholas Johnson, MD, Principal Investigator and Vice Chair of Research at the Department of Neurology, Virginia Commonwealth University School of Medicine. “Dosing the first participant in the second cohort of the trial is positive news for people living with LGMD2I/R9. This is a rare and debilitating type of muscular dystrophy, and this advancement brings the LION-CS101 trial another step closer to completion.”

AskBio has received rare pediatric disease designation (RPDD), orphan-drug designation (ODD) and fast track designation (FTD) for AB-1003 for the potential treatment of LGMD2I/R9 from the United States Food and Drug Administration (FDA).[3-4] These designations serve as clear recognition of the significant unmet medical need in LGMD2I/R9, for which there is no approved therapy.[5]

“The dosing of the first participant in cohort two marks an important milestone for the trial as enrollment continues for LION-CS101,” said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. “We are encouraged by the DSMB’s recommendation to advance our study, following their thorough assessment of AB-1003 in cohort one and are excited to proceed with the second cohort.”

AB-1003 is an investigational recombinant adeno-associative virus (AAV)-based gene therapy that has not been approved by any regulatory authority, and its efficacy and safety have not been fully established or evaluated. It is designed to restore FKRP enzyme activity, primarily inside muscle cells, for the treatment of LGMD2I/R9 as a one-time intravenous (IV) infusion.[2-4]

About Limb-Girdle Muscular Dystrophy Type 2I/R9

LGMD2I/R9 is a rare form of LGMD caused by mutations in the FKRP gene and is associated with weakness and wasting of arm and leg muscles.[6] Those with LGMD2I/R9 notice symptoms in late childhood, typically around 11 years of age, and may experience difficulty running and walking. The symptoms gradually worsen over time, and affected people generally rely on wheelchairs for mobility and may experience impaired heart and lung function.[5-7] LGMD2I/R9 is estimated to affect fewer than 5,000 people in the United States.[6] Currently, there is no approved treatment, and management is based on the signs and symptoms present in each individual.[5]

About AskBio

AskBio Inc., 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 investigational therapeutics for congestive heart failure, 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 array of capsids and promoters. With global headquarters in Research Triangle Park, 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 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 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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