AskBio Presents Interim Safety Results from Phase 1/Phase 2 LION-CS101 Clinical Trial of AB-1003 in Participants with Limb-Girdle Muscular Dystrophy 2I/R9

- *Data from first cohort of five participants suggest an acceptable safety profile for AB-1003 in participants with limb-girdle muscular dystrophy (LGMD) 2I/R9, with no dose-limiting toxicities or serious adverse events reported up to 52 weeks post-treatment*
- *Based on these data, the Data Safety Monitoring Board recommended advancing to the second cohort, announced earlier in 2025*

Research Triangle Park, N.C. – October 10, 2025 – AskBio Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced it will present initial safety data from the first cohort of participants from its Phase 1/Phase 2 LION-CS101 clinical trial of investigational gene therapy AB-1003 in participants with limb-girdle muscular dystrophy (LGMD) 2I/R9 at the 30th Annual International Congress of the World Muscle Society, taking place October 7–11, 2025, in Vienna, Austria.

The presentation represents interim, blinded Cohort 1 safety data. Participants enrolled in Cohort 1 received a single intravenous infusion of AB-1003 or placebo and were followed for 52 weeks post-treatment during the main trial before entering a planned four-year long-term follow-up period. Safety assessments included adverse event monitoring, laboratory testing, physical exams, vital signs, electrocardiograms, and echocardiograms.[1]

There were no dose-limiting toxicities or serious adverse events reported up to 52 weeks post-treatment. Commonly reported (>2 participants) treatment-emergent adverse events were mild-to-moderate in severity and included headaches, falls, and nausea. Three participants reported asymptomatic transient transaminase elevations without changes in bilirubin levels, which returned to baseline levels after adjusting corticosteroid treatment.[1]

The data will be presented by Chris Passalacqua, MD, Vice President of Neuromuscular Medical Affairs, AskBio, at 3:45 p.m. CEST Friday, October 10, 2025.

“These initial safety data are encouraging and suggest an acceptable safety profile for AB-1003,” said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. “We believe AAV-mediated gene therapy has the potential to restore FKRP function and stabilize disease progression, and we are excited to continue our clinical research efforts with the goal of developing an effective treatment for limb-girdle muscular dystrophy.”

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 receive 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. Five participants were enrolled in the first cohort. All are actively participating and should remain in the trial until completion. Enrollment in Cohort 2 is ongoing. For more information about the LION-CS101 clinical trial, visit clinicaltrials.gov (NCT05230459) or askbio.com.

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 fukutin-related protein (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.[5] Symptoms may start to appear from childhood to adulthood, and affected individuals 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.[5] Currently, there is no approved treatment, and management is based on the signs and symptoms present in each individual.[6]

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, which makes gene therapies more accessible by making research and commercial grade manufacturing more affordable. 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 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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