AskBio Receives FDA Rare Pediatric Disease and Orphan-Drug Designations for AB-1003 for the Treatment of Limb-Girdle Muscular Dystrophy Type 2I/R9

- *First patient dosed in Phase 1/Phase 2 LION-CS101 trial of AB-1003 in August 2023, with enrollment continuing*

Research Triangle Park, N.C. – November 7, 2024 – Asklepios BioPharmaceutical, Inc. (AskBio), a gene therapy company wholly owned and independently operated as a subsidiary of Bayer AG, today announced that AB-1003 (also known as LION-101) has received rare pediatric disease designation and orphan-drug designation from the US Food and Drug Administration (FDA) for the treatment of limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9).

FDA grants rare pediatric disease designation to incentivize the development of new treatments for serious and life-threatening diseases that primarily affect children aged 18 years or younger, with fewer than 200,000 people affected in the US. If AB-1003 is approved, AskBio may qualify for a priority review voucher based on receipt of this designation. A priority review voucher can be applied to another therapy in the company's pipeline, enabling a shorter review timeline during marketing application review or can be sold and transferred to another company.[1]

Orphan 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 designations for AB-1003 are clear recognition of the significant unmet medical need in LGMD, including type 2I/R9, which is the focus of AskBio's clinical program and for which there is no approved therapy," said Canwen Jiang, MD, PhD, Chief Development Officer and Chief Medical Officer, AskBio. "The burden of this rare form of muscular dystrophy on patients and their families is profound, and these decisions support our efforts to potentially bring a new therapeutic option to people living with the 2I/R9 type of this devastating disease."

LGMD2I/R9 is a form of LGMD caused by changes in the FKRP gene and is associated with weakness and wasting of arm and leg muscles.[3] People living with LGMD2I/R9 may notice symptoms including loss of mobility, impaired heart or lung function. These symptoms can occur in school age and younger children.[3] As symptoms worsen, individuals generally require wheelchairs.[3] LGMD2I/R9 is a rare disease, estimated to affect fewer than 5,000 people in the US.[3] Currently, there is no treatment that modifies disease progression, and management is based on the signs and symptoms present in each individual.[3]

With a broad portfolio of investigational gene therapies at various stages of research and development, AskBio continues to develop adeno-associated virus (AAV)-based therapies to treat some of the world's most debilitating diseases. The company maintains a portfolio of clinical programs across a range of neuromuscular, central nervous system, cardiovascular, and metabolic disease indications and aims to deliver breakthrough treatments that could potentially benefit tens of millions of patients worldwide.[4-10]

About Limb-Girdle Muscular Dystrophy (LGMD)

Limb-girdle muscular dystrophy (LGMD) is a term for a group of diseases that cause progressive weakness and wasting of the muscles in the arms and legs.[9] The muscles most affected are those closest to the body (proximal muscles), specifically the muscles of the shoulders, upper arms, pelvic area and thighs.[9] The severity, age of onset, and features of LGMD vary among the many subtypes of the condition and are often

inconsistent, even within the same family.[9] Signs and symptoms may first appear at any age and generally worsen with time, although in some cases they remain mild.[9] Visit the National Institutes of Health Medline Plus website to learn more about LGMD.

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 investigational 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 array of capsids and promoters. 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 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 2023, the Group employed around 100,000 people and had sales of 47.6 billion euros. R&D expenses before special items amounted to 5.8 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.

Media Contact:
Phil McNamara
Vice President, Corporate Communications, AskBio
E: pmcnamara@askbio.com T +1 (984) 520 7211

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