AskBio Announces IND for LION-101, a Novel Investigational AAV Gene Therapy for the Treatment of Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9), Cleared to Proceed by U.S. FDA

-- LGMD2I/R9 is a Rare Form of Muscular Dystrophy with No Approved Therapies –

-- Phase 1/2 Clinical Study Expected to Begin Dosing in 1H 2022 –

Research Triangle Park, N.C. – May 25, 2021 – Asklepios BioPharmaceutical, Inc. (AskBio), a wholly owned and independently operated subsidiary of Bayer AG, announced that the U.S. Food & Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for LION-101 to proceed in a Phase 1/2 clinical study. LION-101 is a novel recombinant adeno-associated virus (rAAV) based vector being developed as a one-time intravenous infusion for the treatment of patients with Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9).

LION-101 will be evaluated in a Phase 1/2 multicenter study to evaluate a single intravenous (IV) infusion in adult and adolescent subjects with genotypically confirmed LGMD2I/R9. AskBio plans to initiate dosing for the LION-101 Phase 1/2 clinical study in the first half of 2022.

“In preclinical mouse models, LION-101 therapy demonstrated both dose-dependent efficacy and tolerability, providing a clear approach to study this novel AAV vector in clinical trials,” said Katherine High, MD, President, Therapeutics, AskBio. “Currently there are no approved therapies for LGMD2I/R9, and with limited treatment options that only address symptoms of the disease, the patient burden is profound. We are excited to begin clinical trials with this novel therapy, and to hopefully bring a new therapeutic option to patients and families in the LGMD2I/R9 community living with this devastating disease.”

“Clearing of the LION-101 IND to proceed underscores our commitment to address unmet needs in musculoskeletal disorders, where very few or no treatments exist today, while we continue to advance our broad therapeutic pipeline of novel AAV gene therapies,” said Sheila Mikhail, CEO and Co-Founder, AskBio. “With this clearance, we have now advanced five of our pipeline programs into Phase 1/2 clinical development. We look forward to advancing these clinical programs to bring much needed gene therapy innovation and potential treatments to patients and their families who live with devastating conditions like LGMD2I/R9.”

About Limb-Girdle Muscular Dystrophy (LGMD) and Limb-Girdle Muscular Dystrophy Type 2I (LGMD2I/R9)

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.[1] The muscles most affected are those closest to the body (proximal muscles), specifically the muscles of the shoulders, upper arms, pelvic area and thighs.[1] 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.[1] Signs and symptoms may first appear at any age and generally worsen with time, although in some cases they remain mild.[1]

Limb-Girdle Muscular Dystrophy Type 2I (LGMD2I/R9) is a form of LGMD and is caused by mutations in the FKRP gene.[2] In LGMD2I/R9, signs and symptoms often develop in late childhood and may include difficulty running and walking.[2] The symptoms gradually worsen over time towards significant disability, and affected people generally rely on a wheelchair for mobility approximately 23-26 years after onset.[2] Currently, there is no treatment that modifies the disease progression, and treatment is based on the signs and symptoms present in each individual.[2] Visit the National Institutes of Health Medline Plus website to learn more about Limb-Girdle Muscular Dystrophy and Limb-Girdle Muscular Dystrophy Type 2I.

About AskBio
Asklepios BioPharmaceutical, Inc. (AskBio), a wholly owned and independently operated subsidiary of Bayer AG acquired in 2020, is a fully integrated AAV gene therapy company dedicated to developing life-saving medicines that have the potential to cure genetic diseases. 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 therapeutics for Pompe disease, Parkinson's disease and congestive heart failure, as well as out-licensed clinical indications for hemophilia and Duchenne muscular dystrophy. AskBio's gene therapy platform includes Pro10™, an industry-leading proprietary cell line manufacturing process, and an extensive AAV capsid and promoter library. With global headquarters in Research Triangle Park, North Carolina, and European headquarters in Edinburgh, UK, the company has generated hundreds of proprietary third-generation AAV capsids and promoters, several of which have entered clinical testing. Founded in 2001 and an early innovator in the gene therapy field, the company holds more than 800 patents in areas such as AAV production and chimeric and self-complementary 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. Its 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 2020, the Group employed around 100,000 people and had sales of 41.4 billion euros. R&D expenses, before special items, amounted to 4.9 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, including statements regarding the AskBio's LION-101 program. 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 pipeline of development candidates. These forward-looking statements involve risks and uncertainties, many of which are beyond AskBio's control. Known risks include, among others: AskBio may not successfully bring a new therapeutic option to patients and families in the LGMD2I/R9 community, advance LION-101 as or when expected, be able to execute on its business plans and goals, including meeting its expected or planned 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 the ongoing COVID-19 pandemic, 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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[1] National Institutes of Health – Medline Plus: Limb-Girdle Muscular Dystrophy - Description. Accessed May 7, 2021. Limb-girdle muscular dystrophy: MedlinePlus Genetics

[2] National Institutes of Health -- National Center of Advancing Translational Sciences: Genetic and Rare Diseases Information Center. Limb-Girdle Muscular Dystrophy Type 2I – Summary. Accessed May 7, 2021. Limb-girdle muscular dystrophy type 2I | Genetic and Rare Diseases Information Center (GARD) – an NCATS Program (nih.gov)