



## **AskBio Receives FDA Fast Track Designation for LION-101, a Novel Investigational AAV Gene Therapy for the Treatment of Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9)**

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

**Research Triangle Park, N.C. –June 28, 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 granted Fast Track Designation for the LION-101 gene therapy program. 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).

The FDA recently cleared an Investigational New Drug (IND) application for LION-101, to authorize the start of a Phase 1/2 multicenter study which will evaluate the safety, tolerability, and efficacy of a single intravenous (IV) infusion of gene therapy, 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.

“The FDA Fast Track Designation for LION-101 is an important step for the development of this program and is a clear recognition of the profound burden faced by LGMD2I/R9 patients,” said Sheila Mikhail, Co-Founder & CEO, AskBio. “We look forward to initiating clinical trials with this novel therapy, and we hope to bring a new therapeutic option to patients and families in the LGMD2I/R9 community who live with this devastating disease.”

The FDA Fast Track Program is designed to facilitate the development and expedite the review of new therapeutics that are intended to treat serious conditions and fill an unmet medical need. The purpose of the Program is to get important new therapeutics to the patient earlier. Therapeutics that receive this designation receive a number of benefits that include more frequent meetings with the FDA to discuss development of the gene therapy and, if relevant criteria are met, eligibility for Accelerated Approval and Priority Review.

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

Limb-Girdle Muscular Dystrophy Type 2I (LGMD2I/R9) is a form of LGMD and is caused by mutations in the FKRP gene.<sup>2</sup> In LGMD2I/R9, signs and symptoms often develop in late childhood and may include difficulty running and walking.<sup>2</sup> 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.<sup>2</sup> Currently, there is no treatment that modifies the disease progression, and treatment is based on the signs and symptoms present in each individual.<sup>2</sup> 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](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. 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](http://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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<sup>1</sup> National Institutes of Health – Medline Plus: Limb-Girdle Muscular Dystrophy - Description. Accessed May 7, 2021. [Limb-girdle muscular dystrophy: MedlinePlus Genetics](#)

<sup>2</sup> 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 \(nih.gov\)](#)