Recombinant Adeno-Associated Virus (rAAV) Technology Pioneered by AskBio's Dr. Jude Samulski is Key Component of All FDA Approved AAV Gene Therapeutics

Research Triangle Park, N.C. (December 20, 2022) - Asklepios BioPharmaceutical, Inc. (AskBio), a wholly-owned and independently-operated subsidiary of Bayer AG, today announced that the FDA's approval of a new adeno-associated virus (AAV) gene therapy to treat adults with hemophilia B marks another milestone for the advancement of AAV therapeutics and highlights the important research contributions made by AskBio Co-Founder, President and Chief Scientific Officer, R. Jude Samulski, PhD. The significance of Dr. Samulski's groundbreaking AAV work is validated by the role his pioneering research and discoveries have played in the development of all currently approved AAV gene therapies and most that are in the clinic. Dr. Samulski's early discovery of how to clone AAV led to its widespread use as a mechanism to deliver healthy genes to cells, and we believe this is the foundation for AAV genetic medicine as it stands today.

"The progress made in gene therapy to date is remarkable," said Jude Samulski, AskBio Co-Founder, President and Chief Scientific Officer. "It is humbling to see the impact of the research I completed over 40 years ago and how this is accelerating AAV therapeutic development. My ultimate vision, shared by all of us at AskBio, is to make gene therapy more accessible for everyone in need around the world. I believe we will be there soon."

Dr. Samulski led the UNC Gene Therapy Center from 1993 to 2016 and, in 2001, co-founded AskBio, where many advances in recombinant AAV (rAAV) and underlying AAV vector technology were, and are being, made. Since 2017, three AAV gene therapies have been approved for use by the FDA. Research performed at the UNC Gene Therapy Center, and advanced at AskBio, helped pave the way for the development of these therapeutics. Hemgenix®[1] (etranacogene dezaparvovec-drlb), for hemophilia B, uses the AAV vector with Padua variant $R_{33}8L$; Zolgensma®[2] (onasemnogene abeparvovec-xioi), for spinal muscular atrophy, uses self-complementary AAV vectors; and Luxturna®[3] (voretigene neparvovec-rzyl), for inherited retinal disease, uses the original AAV2 capsid. All are based on Dr. Samulski's pioneering research or on technology that he developed in collaboration with others in the field, as are Duchenne muscular dystrophy AAV therapeutics in clinical development.

"We are incredibly fortunate for Jude's scientific leadership at AskBio and for his continued contributions to genetic medicine," added AskBio's CEO, Sheila Mikhail. "Every day, we see the faces of the people who are running out of time and can't wait for answers. It is our responsibility to find solutions. I believe that Jude, our AskBio colleagues and others throughout the industry are providing hope for people with devastating diseases."

AskBio, co-founded by Dr. Jude Samulski and Sheila Mikhail, is a global leader in molecular medicine and serves as a key driver of Bayer's gene therapy research, manufacturing and clinical advancements.

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[1]Hemgenix® is a registered trademark of CSL Behring LLC

[2]Zolgensma® is a registered trademark of Novartis AG.

[3]Luxturna® is a registered trademark of Spark Therapeutics, Inc.

About AskBio

Asklepios BioPharmaceutical, Inc. (AskBio), a wholly owned and independently operated subsidiary of Bayer AG acquired in 2020, 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 therapeutics for Pompe disease, Parkinson's disease, and congestive heart failure. AskBio's gene therapy platform includes Pro10™, an industry-leading proprietary cell line manufacturing process, and an extensive 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 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 750 patents in areas such as AAV production and chimeric and self-complementary capsids. Learn more at www.askbio.com or follow us on LinkedIn.

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About Bayer

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