back to all

AskBio Enters Research Collaboration and Licensing Agreement with University of North Carolina (UNC) for Angelman Syndrome

Collaboration builds upon preclinical research that demonstrates potential for gene therapy to correct deficiency in UBE3A gene

Research Triangle Park, N.C. and Chapel Hill, N.C. – March 18, 2020 – Asklepios BioPharmaceutical, Inc. (AskBio), a leading clinical-stage adeno-associated virus (AAV) gene therapy company, today announced that it has entered into a research collaboration and licensing agreement with the University of North Carolina at Chapel Hill (UNC) for the development and commercialization of gene therapy for Angelman syndrome.

"This collaboration allows us to leverage groundbreaking research from UNC and apply our AAV development capabilities to find a gene therapy treatment for Angelman syndrome," said Sheila Mikhail, JD, MBA, AskBio Chief Executive Officer and co-founder. "We look forward to advancing this program together."

Angelman syndrome is a rare neurogenetic disorder caused by the loss of function of the UBE3A gene. The disorder occurs in approximately one in 15,000 people, or about 500,000 individuals worldwide, and there is currently no cure. In addition to life-altering symptoms such as speech and motor deficits, more than 80 percent of Angelman syndrome patients experience epilepsy, which typically does not respond well to standard anti-seizure medications.

A UNC School of Medicine team, led by Mark Zylka, PhD, and Ben Philpot, PhD, has generated preclinical evidence that gene therapy may help individuals with Angelman syndrome by improving seizure and motor outcomes.

"Individuals with Angelman syndrome face lifelong challenges, and our gene therapy approaches hold the potential to correct this disorder at its genetic roots. We are incredibly excited to partner with AskBio, as they have been vanguards of clinical gene therapies for rare diseases," said Mark Zylka, PhD, Director of the UNC Neuroscience Center. Ben Philpot, PhD, Associate Director of the UNC Neuroscience Center added, "We look forward to advancing this transformative treatment to the clinic and potentially improving the lives of individuals with Angelman syndrome."

"The partnership between AskBio and UNC could transform the lives of people living with Angelman syndrome by providing them with a potential therapy for this rare disease," said Amanda Moore, Angelman Syndrome Foundation CEO. "The Angelman Syndrome Foundation has long been proud to support the work of UNC researchers, Drs. Ben Philpot and Mark Zylka, and invest in science that positively affects the Angelman syndrome community. The collaboration between UNC and AskBio brings us a step closer to delivering a viable gene therapy to the people and families we serve."

The financial terms of the agreement were not disclosed.

More about Angelman Syndrome

Deletion of the maternally inherited copy of the UBE3A gene causes Angelman syndrome. Symptoms include microcephaly (small head circumference), severe intellectual disability, seizures, balance and movement problems (ataxia), lack of speech, and sleep problems. Behavioral symptoms include frequent laughing, smiling and excitability. Angelman syndrome was first described in 1965, yet no treatment options have been approved in the 55 years since. While individuals with the disorder have a normal lifespan, they require life-long care and are not able to live independently.

About Angelman Syndrome Foundation

The mission of the Angelman Syndrome Foundation is to advance the awareness and treatment of Angelman syndrome through education and information, research and support for individuals with Angelman syndrome, their families and other concerned parties. We exist to give them a reason to smile, with the ultimate goal of finding a cure. To learn more, visit https://www.angelman.org.

About AskBio

Founded in 2001, Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, clinical-stage gene therapy company dedicated to improving the lives of children and adults with genetic disorders. AskBio's gene therapy platform includes an industry-leading proprietary cell line manufacturing process called Pro10™ and an extensive adeno-associated virus (AAV) capsid and promoter library. Based in Research Triangle Park, North Carolina, the company has generated hundreds of proprietary third-generation AAV capsids and promoters, several of which have entered clinical testing. An early innovator in the space, the company holds more than 500 patents in areas such as AAV production and chimeric and self-complementary capsids. AskBio maintains a portfolio of clinical programs across a range of neurodegenerative and neuromuscular indications with a current clinical pipeline that includes therapeutics for Pompe disease, limb-girdle muscular dystrophy 2i/R9 and congestive heart failure, as well as out-licensed clinical indications for hemophilia (Chatham Therapeutics acquired by Takeda) and Duchenne muscular dystrophy (Bamboo Therapeutics acquired by Pfizer). Learn more at https://www.askbio.com or follow us on LinkedIn.

###

Media Contacts:
AskBio
Robin Fastenau
Vice President, Communications
+1 984.275.2705
rfastenau@askbio.com

Angelman Syndrome Foundation
Amanda Moore
Chief Executive Officer
+1 317.514.6918
amoore@angelman.org

UNC Health | UNC School of Medicine
Mark Derewicz
Director, Research & News
+1 984.974.1915
Mark.Derewicz@unchealth.unc.edu

Privacy and Terms