



## PRESS RELEASE

### **Asklepios BioPharmaceutical, Inc. Announces Former Portfolio Company's Preclinical Asset for Duchenne Muscular Dystrophy Has Advanced into the Clinic**

*Pfizer, Inc. Dosed First Patient in its Mini-Dystrophin Gene Therapy Phase 1b Trial*

*Another AskBio Platform Product to be Acquired by Big Pharma Novartis*

CHAPEL HILL, N.C., April 25, 2018 – Asklepios BioPharmaceutical, Inc. (AskBio), an advanced gene therapy (GT) platform company, today announced that a pre-clinical GT asset for Duchenne Muscular Dystrophy (DMD), initially developed by AskBio spin-out Bamboo Therapeutics, Inc. and a wholly-owned subsidiary of Pfizer Inc. since 2016, has advanced into the clinic.

Pfizer announced April 12, 2018 the initiation of a Phase 1b clinical trial for its gene therapy candidate mini-dystrophin that was procured in its acquisition of Bamboo Therapeutics. Richard Jude Samulski, PhD, AskBio founder and Chief Scientific Officer, and Executive Chairman of Bamboo, remained with Pfizer through February 2018 to further accelerate the asset's development. According to Pfizer, the trial will evaluate safety, tolerability, dystrophin expression and distribution, as well as other key biomarkers, in patients with DMD, a serious childhood genetic disease that primarily affects boys. They anticipate early data from the trial in the first half of 2019.

AskBio's GT platform is also the basis for AveXis, Inc.'s lead therapeutic for spinal muscular atrophy (SMA), an often life-threatening neurological genetic disorder. In 2015, AveXis licensed non-exclusive rights to AskBio's proprietary self-complementary (SC) technology, also called Duplex vectors, to further support AveXis' SMA therapeutic development efforts. SC technology has been shown to activate therapeutic product quickly, increase vector potency, and thereby decrease dosing requirements and thus reduce manufacturing demands across a broad range of therapeutic areas. Specific to SMA, the technology has also allowed for the rapid production of the survival motor neuron (SMN) protein, critical to these patients. Novartis announced their intention to acquire AveXis April 9, 2018.

"The DMD and SMA programs are the beginning of a line of therapeutics demonstrating success in the clinic which use AskBio's AAV platform technology," said Dr. Jude Samulski, AskBio's Scientific Founder.

## **About AskBio**

Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, leading gene therapy (GT) platform company established to develop GT technologies that target curative therapies for underserved patient populations with rare genetic and adult-onset disorders.

AskBio's proprietary gene therapy platform includes an industry-leading cell line manufacturing process, an extensive capsid library, and has generated hundreds of proprietary third-generation gene vectors. AskBio's technology has set the standard for gene therapy clinical development, and has supported commercialization of a highly diverse therapeutic pipeline for over a decade. In addition, it has been the basis of AskBio portfolio companies, including NanoCor Therapeutics, currently developing molecular cardiovascular therapies for the treatment of congestive heart failure, Actus Therapeutics, Inc., focused on developing a gene therapy for Pompe Disease, Chatham Therapeutics, which was sold to Baxter in 2014 to continue the development of gene therapies for hemophilia, and Bamboo Therapeutics, Inc., which was sold to Pfizer, Inc. in 2016 to continue the development of gene therapies for rare neuromuscular diseases including muscular dystrophy. More information is available at [www.askbio.com](http://www.askbio.com).

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