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Statement: Pfizer Phase 1b Clinical Trial Results

Research Triangle Park, N.C. (July 1, 2019) – On June 28, 2019, Pfizer reported results from a Phase 1b clinical trial of PF-06939926, its investigational gene therapy for Duchenne Muscular Dystrophy (DMD). Affecting young boys almost exclusively, DMD is a rare genetic disease that causes progressive muscle weakness. Most patients typically lose the ability to walk by age 12 and die of cardiac or respiratory failure by their mid-20s.

Pfizer acquired the therapeutic platform to treat DMD when they purchased Bamboo Therapeutics from AskBio in 2016. It was one of many milestones that have been reached since AskBio was founded in 2001 on the groundbreaking gene therapy work of our co-founders, Dr. Jude Samulski and Dr. Xiao Xiao.

More importantly, Pfizer presented data that for the first time demonstrated striking improvement in North Star scores for patients of this age range that typically show steady functional decline. In addition, Pfizer presented safety data for each patient treated. This included side effects such as nausea, vomiting, reduced appetite, etc., observations commonly seen from all high-dose AAV treatments to date. Of particular note, one patient required hospitalization for IV anti-emetics for vomiting, while another at the two-week time point was asymptomatic but tested positive for "complementation activation" and acute kidney injury, an observation previously documented from Solid Biosciences' DMD trial. All symptoms were clinically addressed, and Pfizer announced continued efforts to advance to Phase 3 pivotal studies worldwide.

We commend Pfizer, Solid, Sarepta Therapeutics and all other companies for their work in finding curative therapies for life-threatening diseases such as DMD. Seeing this through the eyes of the families, the gain of muscle functions in the Pfizer study in six- to 12-year-old patients provided real benefit in quality of life improvements and halting disease progression.

We congratulate Pfizer on the progress with a potentially curative therapy for DMD and companies like AveXis that recently gained FDA approval for Zolgensma® to treat Spinal Muscular Atrophy. The research that companies like Pfizer and AveXis are doing based on the adeno-associated virus (AAV) technology originally developed by Dr. Samulski and Dr. Xiao is the foundation used by more than two-thirds of the gene therapy industry worldwide.

Patient safety is and must remain the most important concern in clinical studies, and while there may be some adverse effects in early stage trials for new gene therapies, we are all working to minimize those instances. We should all celebrate the accomplishments that bring us closer to finding cures for the millions of patients in need. To that end, it is important to recognize the truly innovative AAV discoveries made by Dr. Samulski and Dr. Xiao over the past three decades that have made potential treatments and other AAV therapies possible.

- Dr. Samulski's discovery of how the AAV could be safely used to deliver corrected genes to cells with genetic defects propelled one of the most exciting and inspiring fields in medical research today, now the foundation for an entire industry.
- Dr. Xiao Xiao was the first to create a mini-dystrophin gene that opened the door for the development of potential DMD therapies.
- Dr. Josh Grieger, AskBio's Chief Technology Officer, pioneered the Pro10 producer cell line that is now paving the way for increasing the efficiency of developing and producing potentially curative therapies to reach all patients.

AskBio is on the forefront of the gene therapy industry with an extensive gene therapy platform that includes a pipeline of clinical stage therapeutics, streamlined manufacturing processes, an extensive capsid library and a rich portfolio of AAV-related intellectual property. The company's commitment to bring effective therapies to market is only rivaled by its commitment to reduce the cost of production and increase access with new innovative technology such as Doggybone DNA, thin-film technology and other AAV development processes.

About AskBio

Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, clinical stage gene therapy platform company dedicated to improving the lives of children and adults with rare genetic disorders. AskBio's gene therapy platform includes an industry-leading proprietary cell line manufacturing process known as Pro10™ and an extensive AAV capsid library. The company has generated hundreds of proprietary third generation gene vectors, several of which that have entered clinical testing, and maintains a portfolio of clinical programs across a range of indications including Pompe, Limb Girdle Muscular Dystrophy, Cystic Fibrosis, Myotonic Muscular Dystrophy, Huntington's, Hemophilia (Chatham Therapeutic/Takeda) and Duchenne Muscular Dystrophy (Bamboo Therapeutics/Pfizer). For more information, visit www.askbio.com.

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