

**FOR IMMEDIATE RELEASE**

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**First Patient Dosed with Gene Therapy in Phase 1/2 Study of ACTUS-101 in Patients with Pompe Disease**

*-Initial clinical study to evaluate ACTUS-101 over 52 weeks that could improve quality of life for those who are affected with rare genetic disorder - Pompe Disease. -*

Research Triangle Park, NC - Jan. 22, 2019 – Actus Therapeutics, a privately held portfolio company of Asklepios BioPharmaceutical, Inc. (AskBio), today announced the dosing of the first patient in a phase 1/2 clinical study of ACTUS-101 in patients with Pompe Disease. Pompe Disease is a serious disease associated with high morbidity and often leads to premature death. There are variable rates of disease progression and different ages of onset characterized as infantile-onset and late-onset disease.

The initial clinical trial (ACT-CS101) is a study designed to assess the safety, bioactivity, and immune responses following treatment with a single infusion of ACTUS-101.

“This is an exciting milestone for our company but most importantly, if ACTUS-101 is successful, it could have a meaningful impact on the quality of life for those who suffer from Pompe Disease. Further, ACTUS 101 could replace enzyme replacement therapy (ERT) every other week with the potential to be a groundbreaking metabolic treatment for an unforgiving disease now showing up in an increasing number of patients” said Sheila Mikhail, J.D., Chief Executive Officer and Co-Founder of AskBio.

ACTUS-101 is delivered via intravenous injection and transduces the liver, leading to the continuous production of acid  $\alpha$ -glucosidase (GAA). All study participants in this open label evaluation will receive ACTUS-101 while maintaining their current ERT with alglucosidase alfa. As the trial progresses, subjects who meet prespecified criteria for safety, transgene expression, and motor function will be eligible to suspend treatment with ERT.

“Our strategy at AskBio is to create transformative medicines for people with serious diseases through focusing on validated targets that address causal human biology,” added Mikhail. “ACTUS-101 is a next generation gene therapy, reflective of our commitment to discovering, developing, and manufacturing innovative AAV gene therapy candidates.”

In collaboration with leading experts in the field of genetic disease and specifically Pompe Disease at Duke University, Dwight Koeberl, M.D., Ph.D. and Priya Kishnani, M.D., have played an integral role in the development of gene therapies that may directly address the underlying cause of Pompe Disease, such as ACTUS-101. Their world-renowned expertise in genetics and the treatment of patients with Pompe Disease as well as other metabolic disorders has been instrumental in bringing the technology forward to the clinic as the first ever gene therapy dosing for Pompe Disease. Dr. Koeberl has been working toward the initiation of this clinical

trial for the past decade and Dr. Kishnani has been treating Pompe patients for over 20 years, including playing an instrumental role in Duke's ERT trials in 1999 that led to FDA approval of IV alglucosidase alfa in 2006.

Dr. Koeberl stated, "Preclinical data suggest that this gene therapy product may prove to continuously produce the GAA they (Pompe patients) are lacking. If enough GAA is produced, patients may be able to stop ERT entirely. This would improve their overall quality of life by eliminating the need for weekly or bi-weekly ERT treatments while also strengthening their outlook for living with Pompe."

According to Dr. Kishnani, "If we are successful with ACTUS-101, this carefully planned therapeutic approach will bring a renewed sense of hope to the Pompe Disease community."

As a physician who has been at the forefront of Pompe treatment since Duke committed its institutional resources to the disease in 1990, Dr. Kishnani added, "This has the potential to not only be life-changing for our patients with Pompe disease but also could impact how we approach other inherited metabolic disorders. It is an exciting time in the field, with gene therapy treatment as a potential therapeutic approach for many conditions for which there is no treatment or as a way to enhance treatment outcomes."

#### **About the Pompe Disease Program Actus-101**

Actus-101 is an AAV gene therapy treatment for Pompe Disease now in a Phase 1/2 clinical study. Late-onset Pompe Disease is a genetic disease caused by partial to complete deficiency of acid-alpha-glucosidase (GAA). Progressive accumulation of glycogen in organs and tissues, especially skeletal and cardiac muscle, impair their ability to function normally.

While GAA enzyme replacement with alglucosidase alfa has shown benefit, many patients have persistent muscle weakness. In addition, some patients develop anti-GAA antibodies that limit efficacy and no curative therapy is available. Preclinical models have shown that liver-directed GAA AAV gene therapy has the potential to overcome the limitations of ERT by delivering sustained and efficacious GAA plasma levels.

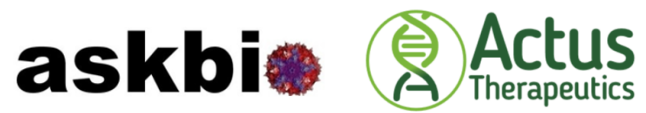
The U.S. Food and Drug Administration recently granted Fast Track Designation for Actus-101 for the treatment of patients with Pompe Disease. Patient recruitment for the Phase 1/2 clinical study began in November, and AskBio also has a follow-on program in late stage preclinical development.

#### **Disclaimer**

Dr. Koeberl and Dr. Kishnani have equity in Actus Therapeutics, which is developing gene therapies for Pompe Disease.

#### **About AskBio**

Asklepios BioPharmaceutical, Inc. (AskBio) is a privately held, 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, an extensive capsid library, and has generated hundreds of proprietary third generation gene



vectors that have entered clinical testing for such indications as Hemophilia (Chatham Therapeutic/Shire) and Duchenne Muscular Dystrophy (Bamboo Therapeutics/Pfizer). More information is available at [www.askbio.com](http://www.askbio.com).

**About Actus Therapeutics**

Actus Therapeutics is a privately held portfolio company of Asklepios BioPharmaceutical, Inc. Actus is dedicated to developing new therapies for rare genetic childhood and adult-onset diseases. More information is available at [www.actustherapeutics.com](http://www.actustherapeutics.com).

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