



CYTOO and AskBio enter into research agreement to screen gene therapy candidates for rare muscle disorder

Grenoble, France and Research Triangle Park, NC, 28 February, 2020 – CYTOO, a leading drug discovery company on muscle disorders, today announced that it has entered into a research collaboration aimed at selecting a gene therapy candidate for a rare muscle disorder with [Asklepios BioPharmaceutical, Inc.](#) (AskBio), a clinical-stage, fully integrated adeno-associated virus (AAV) gene therapy company. Under the terms of the agreement, AskBio and CYTOO will work together to develop an AAV-based screening platform derived from patient cells. The goal of the collaboration is to screen and select a preclinical candidate capable of restoring a healthy phenotype from patient cells cultured *in vitro*.

CYTOO has developed a muscle-on-a-plate platform using patients' primary cells, called MyoScreen. MyoScreen is an *in vitro* system in which skeletal muscle cells mimic the physiology, contractile and metabolic functions of human muscle *in vivo* and allow infection by AAV-based gene therapy vectors targeting muscle.

Dr. Philippe Moullier, Chief Scientific Officer, AskBio Europe, said, *"As a leader in the gene therapy space, the ability to quickly and efficiently screen potential therapeutic candidates will be invaluable. The expertise provided by Cytoo and the MyoScreen platform will potentially give us a better understanding of how those candidates perform in patient cells and improve efficiency throughout the R&D process."*

Luc Selig, CYTOO's CEO, added, *"Gene therapy for muscle disorders is becoming a reality for patients and their families, and we are proud that AskBio has chosen our expertise to investigate a potential new treatment. We have developed MyoScreen as a laboratory model of patient-derived muscle that can be used to screen gene therapy candidates and QC clinical and commercial batches."*

The financial terms of the agreement were not disclosed.

About CYTOO

CYTOO is a preclinical stage drug discovery company addressing muscular disorders (NMDs, muscle waste, muscle disuse, metabolic ageing). The company has developed MyoScreen™, a versatile and high-throughput muscle-on-a-plate R&D platform, from patient-derived myotubes, that can be used to model any muscle disorder and screen any type of therapeutic candidate. The platform is open to partnering with biotech and pharmaceutical companies and has been the starting point of CYTOO's internal drug discovery program on Duchenne Muscular Dystrophy. Among partners of CYTOO : Daiichi Sankyo, Pfizer, Axcella. CYTOO has offices in Grenoble, France and Bethesda, MD, USA.

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 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 type 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 www.askbio.com or follow us on [LinkedIn](#).

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Media Contacts

FP2COM

Florence Portejoie
fportejoie@fp2com.fr
Mob : + 33 (0)6 07 76 82 83

AskBio

Robin Fastenau
Vice President, Communications
rfastenau@askbio.com
+1 984 275 2705