Atamyo Therapeutics Obtains First Regulatory Authorization in Europe to Initiate a Clinical Trial for ATA-100, its Gene Therapy to Treat Limb-Girdle Muscular Dystrophy Type 2I/R9

First approval of Clinical Trial Application was received from United Kingdom Medicines & Healthcare products Regulatory Agency (MHRA)

Evry, France (December 6, 2021) - Atamyo Therapeutics, a biotechnology company focused on the development of new-generation gene therapies targeting neuromuscular diseases, today announced the first authorization of a Clinical Trial Application (CTA) in Europe for ATA-100, its gene therapy for the treatment of the fukutin-related protein (FKRP) limb-girdle muscular dystrophy Type 2I/R9 (LGMD2I/R9). This authorization was granted by the United Kingdom Medicines & Healthcare products Regulatory Agency (MHRA). Additional CTAs were filed in France and Denmark.

"We are thrilled to obtain our first CTA approval in the U.K. for the devastating LGMD2I/R9 disease," said Dr Sophie Olivier, Chief Medical Officer of Atamyo. "Atamyo plans to initiate dosing in patients for ATA-100 in the first half of 2022".

"LGMDR9 is a severe muscular dystrophy with progressive symptoms for which there is currently no approved treatment," said Pr John Vissing, Director of the Copenhagen Neuromuscular Center at the National Hospital, Rigshospitalet (Denmark), and principal investigator of this trial. "It is a great motivation to know that the work we are doing has the potential to make a life-changing difference for the patients affected by this disease."

"We are eager to start treating the first European patient and mark this as a milestone for the field in advancing a potential one-time treatment for patients with LGMD-R9," said Pr Volker Straub, Professor of Medicine and Director of the John Walton Muscular Dystrophy Research Centre, Newcastle University (UK).

"This is an important step in our mission to bring to patients suffering from limb-girdle muscular dystrophies (LGMD) a new generation of safe and effective gene therapies, after only one year of activity", said Stéphane Degove, CEO of Atamyo Therapeutics.

LGMD2I/R9 is a rare genetic disease caused by mutations in the gene that produces fukutin-related protein (FKRP). It affects an estimated 5,000 people in the US and Europe. Symptoms appear around late childhood or early adulthood. Patients suffer from progressive muscular weakness leading to loss of ambulation. They also are prone to respiratory impairment and myocardial dysfunction. There are currently no curative treatments for LGMDR9.

ATA-100, a gene therapy candidate for LGMD21/R9, delivers a normal copy of the gene for production of FKRP proteins. The therapy is based on the research of Atamyo Chief Scientific Officer Isabelle Richard, Ph.D., Research Director at CNRS who heads the Progressive Muscular Dystrophies Laboratory at Genethon.

In preclinical mice models, ATA-100 demonstrated its tolerability and capability to correct symptoms and biomarkers of the pathology at unprecedented low doses for systemic AAV-mediated gene transfer addressing muscle diseases.

About Atamyo Therapeutics

Atamyo Therapeutics is focused on the development of a new generation of effective and safe gene therapies for neuromuscular diseases. A spin-off of gene therapy pioneer Genethon, Atamyo leverages unique expertise in AAV-based gene therapy and muscular dystrophies from the Progressive Muscular Dystrophies Laboratory at Genethon. Atamyo's most advanced programs address different forms of limb-girdle muscular dystrophies (LGMD). The name of the company is derived from two words: Celtic Atao which means "Always" or "Forever" and Myo which is the Greek root for muscle. Atamyo conveys the spirit of its commitment to improve the life of patients affected by neuromuscular diseases with life-long efficient treatments. For more information visit www.atamyo.com

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