Atamyo Therapeutics Obtains Regulatory Authorization in Europe to Initiate a Clinical Trial for ATA-200, its Gene Therapy to Treat Limb-Girdle Muscular Dystrophy Type 2C/R5

- *Atamyo has received approval to initiate a clinical trial of ATA-200 gene therapy in France and Italy*
- *ATA-200 is a single-injection gene therapy aimed to treat LGMD2C/R5 caused by mutations in the γ-sarcoglycan gene*
- *The phase 1b, dose-escalation study will evaluate the safety and efficacy of ATA-200 in children*

Evry, France (March 26, 2024) - Atamyo Therapeutics, a clinical-stage biotechnology company focused on the development of new-generation gene therapies targeting muscular dystrophies and cardiomyopathies, today announced the first authorization of a Clinical Trial Application (CTA) in Europe for ATA-200, its gene therapy for the treatment of the γ-sarcoglycan related limb-girdle muscular dystrophy Type 2C/R5 (LGMD2C/R5). This authorization was first granted by the Italian Medicines Agency (AIFA), then by the French Medicines Agency (ANSM).

"We are thrilled to obtain our CTA approval in France and Italy for the devastating LGMD2C/R5 disease affecting primarily a pediatric population and for which there is no approved treatment," said Dr Sophie Olivier, Chief Medical Officer of Atamyo. "Atamyo plans to initiate dosing in patients for ATA-200 in third quarter 2024".

"LGMD 2C/R5 is a severe muscular dystrophy with an onset in early childhood and loss of ambulation generally occurring before adolescence" said Pr. Giacomo Comi, Full Professor of Neurology at the University of Milan (Italy), 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."

"This is a significant milestone for LGMD-2C/R5 patients and for Atamyo as ATA-200 is the first treatment targeting LGMD-2C/R5 to enter clinical trials," said Stéphane Degove, CEO of Atamyo Therapeutics. "With the ongoing clinical trial of ATA-100 in LGMD2I/R9, the initiation of the clinical program for ATA-200 confirms our unique capabilities in bringing to patients suffering from limb-girdle muscular dystrophies a new generation of safe and effective gene therapies."

This clinical trial (NCT05973630) is a multicenter, Phase 1b, open-label, dose escalation study evaluating safety, pharmacodynamic, efficacy, and immunogenicity of intravenous ATA-200, a single-dose Adeno-Associated Virus (AAV) vector carrying the human γ-sarcoglycan transgene.

LGMD2C/R5 is a rare genetic disease caused by mutations in the gene that produces γ-sarcoglycan, a transmembrane protein that is involved in the connection between muscle fibers and their environment. It affects an estimated 2,000 people in Europe. In the classical form, symptoms appear in early childhood and patients suffer from progressive muscular weakness leading to loss of ambulation before adulthood. Cardiac involvement, typically presenting as dilated cardiomyopathy is reported in approximately half of patients. There is no curative treatment. The management of LGMD2C/R5 is solely supportive.

ATA-200, a gene therapy candidate for LGMD2C/R5, delivers a normal copy of the gene for production of γ-sarcoglycan protein. In preclinical models, a single systemic injection of ATA-200 demonstrated its tolerability and capability to correct symptoms and biomarkers of the pathology. ATA-200 has been granted Orphan Medicinal Product Designation by the European Medicines Agency (EMA).

The therapy is based on the research of Atamyo Chief Scientific Officer Isabelle Richard, Ph.D., Research Director at National Center of Scientific Research (CNRS) in France and head of the Progressive Muscular Dystrophies Laboratory at Genethon.

In addition to its LGMD2C/R5 gene therapy, Atamyo is developing a clinical trial with ATA-100 gene therapy for LGMD2I/R9, related to deficiencies in FKRP; and is in IND-enabling studies for LGMD2A/R1, related to deficiencies in calpain protein.

About Atamyo Therapeutics

Atamyo Therapeutics is a clinical-stage biopharma 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), with two clinical-stage programs targeting respectively LGMD2I/R9 and LGMD2C/R5. 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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