IND for ATA-200, a Gene Therapy for the Treatment of Limb-Girdle Muscular Dystrophy Type 2C/R5 (LGMD2C/R5), cleared to proceed by FDA

- *LGMD 2C/R5 is a rare muscle dystrophy affecting children, with no approved treatment*
- *Orphan Drug Designation awarded from US FDA to ATA-200*

Evry, France (November 12, 2024) - Atamyo Therapeutics a clinical-stage biotechnology company focused on the development of new generation gene therapies targeting muscular dystrophies and cardiomyopathies, today announced that the U.S. Food & Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for ATA-200 to proceed in a Phase 1b/2b clinical trial. ATA-200 is a one-time gene therapy for the treatment of γ-sarcoglycan related limb-girdle muscular dystrophy Type 2C/R5 (LGMD2C/R5), a serious and debilitating condition that affects children and leads to loss of ambulation before adulthood.

The multicenter Phase1b open-label dose escalation study (NCT05973630) will evaluate safety, pharmacodynamics, efficacy, and immunogenicity in children receiving intravenous ATA-200, a single-dose Adeno-Associated Virus (AAV) vector carrying the human γ-sarcoglycan transgene. The deployment in the US of Atamyo's clinical trial of ATA-200 is funded by The Dion Foundation for Children with Rare Diseases. This study has already received regulatory clearance in France and Italy.

"This IND clearance in an important step to bring ATA-200 to US children suffering from this highly debilitating LGMD-2C/R5 disease" said Sophie Olivier, MD, Chief Medical Officer of Atamyo. "We are proud to be the first treatment for LGMD-2C/R5 to enter into clinical development in the US and we are committed to opening the first US center before year-end" added Stéphane Degove, Atamyo's Chief Executive Officer.

In addition, Atamyo announced the US FDA has awarded Orphan Drug Designation for ATA-200. Orphan Drug Designations by the FDA grants seven years of market exclusivity in the US. This Orphan Drug Designation for ATA-200 was granted a few weeks after ATA-200 received Rare Pediatric Disease Designation by the FDA.

LGMD-2C/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 and in the US. 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 and will eventually impact life expectancy. There is no curative treatment. The management of LGMD-2C/R5 is solely supportive.

ATA-200, the gene therapy for LGMD-2C/R5, delivers a normal copy of the gene for production of γ-sarcoglycan. In preclinical mice models, ATA-200 demonstrated its tolerability and capability to correct symptoms and biomarkers of the pathology. Atamyo plans to initiate enrolling patients for this clinical trial in the 4th quarter of 2024.

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.

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 LGMD-R9 and LGMD-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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