Atamyo completes the dose-finding study in Limb-Girdle Muscular Dystrophy Type R9 (LGMD-R9) and obtains US Rare Pediatric Disease Designation for ATA-100

- *Multicenter Phase 1b study assessing two doses of ATA-100 gene therapy in two cohorts of 3 ambulant LGMD-2I/R9 patients each*
- *Good safety of ATA-100 in both cohorts, as stated by independent DSMB*
- *Marked efficacy in first 9E12 vg/kg dose cohort, with improvements of all functional and histological endpoints in all treated patients*
- *Significant improvement in αDG glycosylation observed in muscle biopsies*
- *Rare Pediatric Disease Designation awarded to ATA-100 from US FDA*

Evry, France (April 3, 2025) –Atamyo Therapeutics a clinical-stage biotechnology company focused on the development of new generation gene therapies targeting muscular dystrophies, announced the completion of the dose-escalation phase of its Phase 1b/2b clinical trial of ATA-100, a gene therapy for patients with limb-girdle muscular dystrophy Type 2I/R9 (LGMD-2I/R9), protocol code ATA-001-FKRP. The dose-finding part of this multi-center trial conducted in Europe (and cleared in US), tested 2 doses of ATA-100 (9E12 vg/kg and 2.7E13 vg/kg) in 2 cohorts of 3 ambulant patients each.

The independent Data Safety Monitoring Board held in February 2025 stated that the safety in the dose-finding phase showed no concern and that both ATA-100 doses could be selected for the phase 2b part of this trial, without any changes to be made to the protocol.

Satisfactory biodistribution and improvements in histology biomarkers, including αDG glycosylation increases, were observed in patients' muscle biopsies with both doses.

A marked efficacy was observed with single-injections of ATA-100 in the first dose cohort, with improvements of all functional and histological endpoints observed in all treated patients, leading to a reversion of the anticipated decline observed in the natural history of the disease:

- Sitting Forced Vital Capacity (FVC) increased on average by 5% (3.7 absolute FVC percent-points) at 12 months
- NSAD score was stabilized at 12 months and improved at 18 months
- Velocity improved on average by 19% (or 0.3 meter/seconds) in the 10 Meters Walk Test at 12 months

These functional improvements were maintained beyond 12 months and were associated with significant increases in quality-of-life scores (Activlim and gNMD) by 16% and 23% respectively, at 12 months.

The same level of efficacy was not observed so far with the 2nd ATA-100 dose (with a shorter follow-up since administration).

The 9E12 vg/kg dose has been selected for further ATA-100 clinical development.

Atamyo also announced that the US FDA has awarded the Rare Pediatric Disease Designation to ATA-100. This designation was requested based on the potential for ATA-100 to address an unmet medical need for LGMD-2i/R9, due to appearance of debilitating symptoms during childhood or adolescence.

“ATA-100 is the first treatment in LGMD-2i/R9 to present improvements in all functional endpoints in all patients treated. These results show Atamyo’s ability to design and develop next-gen AAV-based gene therapies with improved efficacy/safety profiles” said Atamyo CEO and Co-Founder Stephane Degove. “Detailed results of the clinical trial will be presented in scientific congress during the 2nd half of 2025”

“We are thrilled to have completed the dose-finding study for ATA-100 and to have selected the dose to carry over in the pivotal phase of the study,” said Sophie Olivier, MD, Chief Medical Officer of Atamyo. “The Rare Pediatric Disease Designation granted by FDA also confirms the potential of ATA-100 to address high unmet needs in pediatric populations.”

LGMD-2I/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. In the most common form, symptoms appear around late childhood. Patients suffer from progressive muscular weakness leading to loss of ambulation. They are also prone to respiratory impairment. There is currently no curative treatment for LGMD2I/R9.

ATA-100, a single-administration gene therapy candidate for LGMD-2I/R9, delivers a normal copy of the gene for production of FKRP protein. The therapy is based on the research of Atamyo Chief Scientific Officer Isabelle Richard, Ph.D., who heads the Progressive Muscular Dystrophies Laboratory at Genethon. ATA-100 has been granted Orphan Drug Designations in the US and Europe, as well as Fast Track and Rare Pediatric Designations in the US.

In addition to its LGMD-2I/R9 gene therapy program, Atamyo conducts a first-in-human trial in the US for ATA-200, its gene therapy for LGMD-2C/R5.

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

Contact: contact@atamyo.com