First clinical results of ATA-100, a Gene Therapy for the Treatment of Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9), presented at ESGCT

- ATA-100 is being evaluated in a multi-center phase 1b/2b "ATA-001-FKRP" study
- Completion of enrollment of the first dose cohort of 3 patients
- First results of cohort one presented at ESGCT in oral presentation
- Good clinical and biological safety/tolerability in the first cohort
- Preliminary efficacy documented with biomarkers and functional assessments
- DSMB clearance to proceed with enrolment of the 2nd cohort with a 3-time higher dose

Evry, France (October 27, 2023) - Atamyo Therapeutics, a biotechnology company focused on the development of new-generation gene therapies targeting muscular dystrophies and cardiomyopathies, today announced the presentation of the first clinical results obtained with ATA-100 in the on-going ATA-001 Phase 1b/2b clinical trial. ATA-100 is a one-time gene therapy for the treatment of fukutin-related protein (FKRP) limb-girdle muscular dystrophy Type 2I/R9 (LGMD2I/R9). Atamyo also announced today that Data Safety Monitoring Board (DSMB) authorized the enrollment of the second dose cohort of the ATA-001 clinical trial.

ATA-100 is being evaluated in a multi-center phase 1b/2b in Denmark, France, and United-Kingdom, and has received IND clearance by FDA.

Preliminary results in the first cohort show:

- No unexpected safety signal identified
- Marked decline in levels of creatine kinase in all three patients
- Improved velocity, sustained at one year
- Disappearance of symptoms (cramps, myalgia) and improved quality of life
- Correction of centronucleation and evidence of transgene expression on the 3-month muscle biopsy

Pr. John Vissing, principal investigator of the study, noted that "the preliminary results from cohort 1 with the first dose tested already show encouraging results from a safety and efficacy perspective. ATA-100 treatment has a life-changing potential in an indication where there is no approved treatment'.

These first results will be presented during oral communications in two forthcoming conferences:

1) The 30th Annual Congress of the European Society of Gene & Cell Therapy (ESGCT) that will take place on October 24-27, 2023 in Brussel (Belgium):
   "Gene therapy for LGMDR9: preliminary results of a dose-escalation study"

- Oral presentation by Nicolai Preisler, MD, Rigshospitalet, Copenhagen, during the Cardiovascular and muscular diseases session, on Friday October 27, 11h30-13h00 – Le BEL, Tour & Taxis

2) The 2023 International Limb-Girdle Muscular Dystrophy Conference that will take place on 28-29 October 2023, Washington DC.
"Preliminary experiences with Atamyo's FKRP gene therapy"
   - Oral presentation by John Vissing, MD, Rigshospitalet, Copenhagen, during the Clinical Trials Updates (Part 1) Session, on October 29, 11h00am-12h10pm – Constitution Ballroom, Grand Hyatt

"These first results, in particular those related to transgene expression in muscle fibers are really exciting" said Dr. Sophie Olivier, Atamyo's Chief Medical Officer. "With the DSMB clearance to start the 2nd cohort, we are looking forward to enrolling new patients in Europe and in the US."

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. In the most common form, symptoms appear around late childhood or early adulthood. Patients suffer from progressive muscular weakness leading to loss of ambulation. They are also prone to respiratory impairment. There are currently no curative treatments for LGMD2I/R9.

ATA-100, a single-administration gene therapy candidate for LGMD2I/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.

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 muscular dystrophies and cardiomyopathies. 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 one clinical-stage program targeting LGMD-R9. Atamyo is committed 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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