Atamyo Therapeutics Obtains US FDA Fast Track Designation for ATA-100, a Gene Therapy in Phase 1/2 Clinical Trials for Limb-Girdle Muscular Dystrophy Type R9 (LGMD-R9)

- *In the clinical trial of ATA-100 for LGMD-R9, the company said enrollment progress is on track and the first 2 patients in the second high-dose cohort have been treated.*
- *Updated results of the clinical trials will be presented at the 29th International Annual Congress of the World Muscle Society (WMS) in Prague (8-12 October 2024).*

Evry, France (June 24, 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 US Food and Drug Administration has awarded Fast Track Designation for ATA-100, an AAV-based gene therapy for the treatment of LGMD-R9 in Phase 1/2 clinical trials in the US and Europe.

The designation was requested based on the potential for ATA-100 to address an unmet medical need for LGMD-R9, a serious and debilitating condition that affects young adults and leads to loss of ambulation within 10 to 15 years. Preliminary data from the ongoing European Phase 1b/2b study were submitted in support of the application.

The clinical trial (EudraCT 2021-004276-33, NCT105224505) is a multicenter Phase 1/2 studies evaluating safety, pharmacodynamics, efficacy, and immunogenicity of intravenous ATA-100, a single-dose Adeno-Associated Virus (AAV) vector carrying the human FKRP transgene. This study will consist of two phases: an open-label dose escalation phase (Stage 1) and a double-blind placebo controlled, randomized phase (Stage 2).

Enrollment of the first low-dose cohort of the study in Europe (protocol code ATA-001-FKRP) is completed with promising initial functional results and two patients have already been treated in the high-dose cohort. ATA-100 has been overall well tolerated to date in all treated patients with no unexpected safety signal.

Updated results of the clinical trials will be presented through an oral presentation at the 29th International Annual Congress of the World Muscle Society (WMS) in Prague, October 8 to 12, 2024.

“This new Fast Track Designation in the US and the good clinical progress of our LGMD-R9 program confirms its life-changing potential for patients affected by LGMD-R9,” said Stephane Degove, Chief Executive Officer and Co-Founder of Atamyo Therapeutics. “We look forward to sharing ATA-100 updated clinical trial results with the community in October,” he added.

About the LGMD-R9 program ATA-100
ATA-100 is a one-time gene replacement therapy for LGMD-R9/2I based on the research of Dr. Isabelle Richard, Atamyo’s Chief Scientific Officer. ATA-100 has been awarded Orphan Drug Designation status by the U.S. Food and Drug Administration and the European Medicines Agency.

LGMD-R9/2I 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 LGMD-R9.

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

U.S. Contact:
Charles Craig, Opus Biotech Communications
Charles.s.craig@gmail.com, 404-245-0591

European contact: contact@atmayo.com