Atamyo Therapeutics Announces First Patient Dosed with ATA-100 Gene Therapy in LGMD-R9 Clinical Trial

- *First patient dosed with ATA-100 gene replacement therapy for LGMD-R9*
- *On-going multicenter, Phase 1/2 study evaluating safety, pharmacodynamic and efficacy of ATA-100*

Evry, France (September 26, 2022) - Atamyo Therapeutics, a biotechnology company focused on the development of new-generation gene therapies targeting neuromuscular diseases, today announced the dosing with ATA-100 of a first patient in a phase 1/2 clinical study in FRKP-related limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9).

"This is an exciting milestone for our company but most importantly, if this clinical trial is successful, it could have a life-changing impact for patients affected by LGMD-R9" said Stephane Degove, Chief Executive Officer and Co-Founder of Atamyo Therapeutics.

This clinical trial (EudraCT 2021-004276-33, NCT05224505) is a multicenter, Phase 1/2 study evaluating safety, pharmacodynamic, 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 2 phases: an open-label dose escalation phase (Stage 1) and a double-blind placebo controlled, randomized phase (Stage 2).

"LGMD-R9 is a severe progressive and debilitating disease with no approved treatment." said Pr. John Vissing, Director of the Copenhagen Neuromuscular Center at the National Hospital, Rigshospitalet, in Copenhagen, where the first patient was dosed, and principal investigator of this trial. "This experimental treatment represents a new hope for the patients. It is a great motivation to know that the work we are doing here has the potential to make a life-changing difference."

"After the first patient dosed in Copenhagen, we are now expecting recruitments at the 2 other approved clinical sites (Paris, FR, and Newcastle, UK) to complete enrollment of the dose escalation phase (Stage 1) of the study. For Stage 2 (after dose selection), we plan to open additional clinical sites in Europe and in the United States." said Dr Sophie Olivier, Chief Medical Officer of Atamyo.

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, who heads the Progressive Muscular Dystrophies Laboratory at Genethon (UMR 951 INSERM/Genethon/UEVE). ATA-100 has been awarded Orphan Drug Designation status by the U.S. Food and Drug Administration and the European Medicines Agency.

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. 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 LGMDR9.

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). 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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