ATA-200, Atamyo Therapeutics' Gene Therapy to Treat Limb-Girdle Muscular Dystrophy Type 2C/R5, reaches key milestones with the filing of a clinical trial application in Europe and a non-dilutive financing from France 2030 program

- *A multicenter phase 1b study will evaluate safety, pharmacodynamic and efficacy of ATA-200*
- *ATA-200 is the second Atamyo's next generation gene replacement therapy entering the clinic*
- *ATA-200 is eligible to receive up to €8m ($8.6m) of non-dilutive financing from France 2030 program*

Evry, France (September 19, 2023) - Atamyo Therapeutics, a biotechnology company focused on the development of new-generation gene therapies targeting neuromuscular diseases, today announced the filing of a Clinical Trial Application (CTA) in Europe for ATA-200, its gene therapy targeting γ-sarcoglycan (SGCG) related limb-girdle muscular dystrophy Type 2C/R5 (LGMD2C/R5).

The company also announced that ATA-200 has been granted a non-dilutive financing up to €8m ($8.6m) from France 2030 public program managed by Bpifrance, to support its clinical trials and manufacturing development programs.

This clinical trial (EUCT 2023-506440-16-00) is a multicenter, Phase 1b, open-label, dose escalation study evaluating safety, pharmacodynamic, efficacy, and immunogenicity of intravenous ATA-200, a single-dose Adeno-Associated Virus (AAV) vector carrying the human γ-sarcoglycan transgene.

"LGMD-R5 is the most rapidly progressive and debilitating form of LGMDs with onset of symptoms in early childhood, a loss of ambulation before adulthood, and frequent cardiac impairment. No curative treatment exists for this disease" said Dr Sophie Olivier, Chief Medical Officer of Atamyo.

"ATA-200 incorporates a new promoter that enhances the liver and cardiac safety of gene therapy" said Isabelle Richard, Ph.D., Co-founder and Chief Scientific Officer of Atamyo. "This first-in-class experimental treatment represents a new hope for the patients".

"We are thrilled to file this European Clinical trial and to receive such a strong public financing for the devastating LGMD2C/R5 disease" said Stephane Degove, Co-Founder and Chief Executive Officer of Atamyo. "With the ongoing clinical trial in LGMD-R9, the initiation of the clinical program for LGMD-R5/2C confirms our unique capabilities in bringing to patients

suffering from limb-girdle muscular dystrophies a new generation of safe and effective gene therapies."

LGMD2C/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 only. 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. There is no curative treatment. The management of LGMDR5 is solely supportive.

ATA-200, the gene therapy for LGMD2C/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 dosing in patients for ATA-200 in the first half of 2024.

In addition to its LGMD2C/R5 gene therapy, Atamyo is developing a clinical trial with ATA-100 gene therapy for LGMD2I/R9, related to deficiencies in FKRP; and is in IND-enabling studies for LGMD2A/R1, related to deficiencies in calpain protein.

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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About France 2030
Launched by the President of France Republic in October 2021, France 2030 is a €54 ($58) billion program which aims to accelerate the transformation of key sectors of the French economy through innovation. From fundamental research, emergence of an idea, and up to the production of a new product or service, France 2030 supports the entire life cycle of innovation until its industrialization.
More information on : france2030.gouv.fr

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