IND for ATA-100, a Gene Therapy for the Treatment of Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9), cleared to proceed by FDA

- **LGMD 2I/R9 is a rare muscle dystrophy with no approved treatment**
- **ATA-100 is being evaluated in Europe in a multi-center phase 1b/2b**

Evry, France (September 6, 2023) - Atamyo Therapeutics, a biotechnology company focused on the development of new-generation gene therapies targeting muscular dystrophies and cardiomyopathies, today announced that the U.S. Food & Drug Administration (FDA) has cleared its Investigational New Drug (IND) application for ATA-100 to proceed in a 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).
ATA-100 (formerly known as GNT0006) is being evaluated in a multi-center phase 1b/2b in Denmark, France, and United-Kingdom.

"This IND clearance in an important step to bring ATA-100 to US patients suffering from this highly debilitating LGMD-R9 disease" said Dr. Sophie Olivier, Atamyo's Chief Medical Officer. "After the initiation in 2022 of a phase 1b/2b in Europe, we are looking forward to opening US centers in the near future for this clinical trial."

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. 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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