# Atamyo Therapeutics Submits CTA in Europe for ATA-100, Its Gene Therapy to Treat Limb-Girdle Muscular Dystrophy Type 2I/R9

*Company also appoints Sophie Olivier, M.D., Chief Medical Officer, joining co-founders Stephane Degove, Chief Executive Officer, and Isabelle Richard, Ph.D., Chief Scientific Officer*

*Atamyo is a spin-off company from Genethon, a pioneer and world leader in R&D for gene therapies targeting rare diseases*

Evry, France (October 26 2021) - 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-100, its gene therapy targeting fukutin-related protein (FKRP) limb-girdle muscular dystrophy Type 2I/R9 (LGMD2I/R9).

"We are thrilled to file our first CTA in France, Denmark and the U.K. for the devastating LGMD2I/R9 disease," said Stephane Degove, Co-Founder and Chief Executive Officer of Atamyo. "This is a first step in our mission to bring to patients suffering from limb-girdle muscular dystrophies (LGMD) a new generation of safe and effective gene therapies."

"This CTA filing is the achievement of 30 years of research at Genethon, first on the search for genes responsible of LGMDs, then on the development of gene therapies for these complex diseases. It was made possible by the work of many people at Genethon, the support of patients' representatives, and the participation in our LGMD2I/R9 program of key hospitals in Europe, for many years," said Isabelle Richard, Ph.D., Co-founder and Chief Scientific Officer of Atamyo.

Atamyo is a spin-off from Paris-based Genethon, a pioneer and world leader in the research and development of gene therapies for neuromuscular diseases over the past 30 years. The company is leveraging Genethon's unique expertise in AAV-based gene therapy, muscular dystrophies and breakthrough technologies for the safety and efficacy of AAV-based gene therapies.

"The creation of Atamyo by Genethon stems from its ambition to create a leader of gene therapy applied to LGMDs. It benefits from all our breakthroughs in this field, and its mission will be to develop these treatments for the benefit of the largest number of patients who have no therapeutic options," said Frédéric Revah, Ph.D., CEO of Genethon. "Atamyo will raise the funds necessary to develop its LGMD programs up to the market."

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 currently are no curative treatments.

ATA-100, the gene therapy for LGMD21/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.

In preclinical mice models, ATA-100 demonstrated its tolerability and capability to correct symptoms and biomarkers of the pathology at unprecedented low doses for systemic AAV-mediated gene transfer addressing muscle diseases. Atamyo plans to initiate dosing in patients for GNT006 in the first half of 2022.

In addition to its LGMD2I/R9 gene therapy, Atamyo is developing gene therapies for LGMD2C/R5, related to deficiencies in γ-sarcoglycan protein; and LGMD2A/R1, related to deficiencies in calpain protein.

**Appointment of Dr. Sophie Olivier as CMO**

Atamyo also announced the appointment Sophie Olivier, M.D., as Chief Medical Officer overseeing all clinical development and medical affairs strategies. Dr. Olivier has an extensive career as a senior manager in the industry, particularly in regard to pediatric development and rare diseases.

Before joining Atamyo, Dr. Olivier was Chief Medical Officer at Lysogene and Genticel. She also worked as Scientific Officer in the Pediatric Team at the European Medicines Agency in London (2009-2014) and led clinical developments in Women's Health diseases at Wyeth Pharmaceutical US.

"We are delighted to welcome Dr. Olivier to the Atamyo senior leadership team as we enter an important period with multiple programs advancing towards clinical development," said Mr. Degove.

Dr. Olivier commented that "joining Atamyo when entering clinical phase for its first program, with other CTA filings planned in a near future is very stimulating. Knowing that our work has the potential to improve the life of patients with LGMDs is very motivating."

**About Atamyo Therapeutics**
Atamyo Therapeutics is 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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