



## **Atamyo Therapeutics announces Significant Milestones for ATA-100 and ATA-200, its Gene Therapy programs to Treat Limb-Girdle Muscular Dystrophy 2I/R9 and 2C/R5**

- *Additional approval of Clinical Trial Application for ATA-100 received from French National Medicines Health Agency (ANSM)*
- *Orphan Drug Designation awarded from European EMA for ATA-200*
- *Forthcoming presentation on ATA-200 at ASGCT annual meeting*
- *Appointment of Catherine Cancian as Chief Technical Officer*

Evry, France (May 16, 2022) - [Atamyo Therapeutics](#), a biotechnology company focused on the development of new-generation gene therapies targeting neuromuscular diseases, today announced multiple major milestones for ATA-100 and ATA-200, its one-time gene-replacement therapies for the treatment of limb-girdle muscular dystrophy types 2I/R9 and 2C/R5 (LGMD2I/R9 and LGMD2C/R5 respectively), as well as the reinforcement of its management team.

### **Authorization of Clinical Trial Application in France for ATA-100**

A third authorization of a Clinical Trial Application (CTA) in Europe was granted by the French National Medicines Health Agency (ANSM) for ATA-100. Two CTA approvals had previously been granted respectively by the United Kingdom Medicines & Healthcare products Regulatory Agency (MHRA) and by the Denmark Danish Medicines Agency (DKMA). ATA-100, a single-administration gene therapy candidate for LGMD2I/R9, delivers a normal copy of the gene for production of FKRP proteins. LGMD2I/R9 is a rare genetic disease caused by mutations in the gene that produces fukutin-related protein (FKRP) and affects an estimated 5,000 people in the US and Europe.

### **Orphan Drug Designation awarded from European EMA for ATA-200**

ATA-200, Atamyo's one-time gene replacement therapy for the treatment of LGMD 2C/R5, has been granted Orphan Drug Designation by the European Medicines Agency (EMA) for the treatment of LGMD. Orphan Drug Designations by the EMA grants a ten-year market exclusivity in Europe and provides with other benefits such as tax credits, protocol assistance and research grants.

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.

“We are thrilled by this additional approval for our first-in-human trial with ATA-100 and by the Orphan Drug Designation in Europe for ATA-200. These single-administration treatments bring hope to patients with LGMD2I/R9 and LGMD2C/R5”, said Stéphane Degove, CEO of Atamyo Therapeutics.

## **Forthcoming presentation of ATA-200 at ASGCT annual meeting on May 16, 2022**

The ATA-200 construct, as well as unveiled *in vivo* results and GLP-biodistribution and toxicology study, will be presented at the forthcoming ASGCT 25<sup>th</sup> Annual Meeting, Washington (DC) on May 16-19, 2022. The poster presentation details are:

- Abstract #173 and Poster Board Number M-54: Preclinical Development of a Gene Therapy for Gamma Sarcoglycanopathy, J Poupiot et al.
- Session Title: AAV Vectors - Preclinical and Proof-of-concept Studies I
- Location, Date and Start Time: Hall D – May 16, 2022 17:30 (EST)

“We are looking forward to presenting to the community ATA-200/GNT008 our first-in-class one-time gene replacement therapy for LGMD2C/R5,” said Isabelle Richard, Chief Scientific Officer at Atamyio Therapeutics.

LGMD 2C/R5 affects an estimated 2,000 people in the US and Europe. Symptoms usually appear during early childhood. Patients suffer from progressive muscular weakness leading to loss of ambulation before adulthood. They also are prone to respiratory impairment and myocardial dysfunction. There are currently no curative treatments for LGMD2C/R5.

### **Appointment of Catherine Cancian MSc, MEng, MBA, as CTO**

Atamyio also announced the appointment of Ms. Catherine Cancian, MSc, MEng, MBA as Chief Technical Officer overseeing all pharmaceutical development, clinical and future commercial supply. Ms Cancian has an extensive career as a senior manager in the industry, particularly in regard to pharmaceutical development of biologicals.

Before joining Atamyio, Ms. Cancian was Vice President Pharmaceutical Operations for GenSight Biologics where she developed and executed the strategy for CMC and Supply Chain activities to support the clinical development and commercial readiness of AAV-based gene therapies. Previously, Catherine was Industrialization Project Director for the setup of a new Gene and Cell Therapy manufacturing facility at YposKesi and served at Sanofi Pasteur during 18 years at various management positions including Manufacturing, Project Management, Process Science & Technology and Quality for various marketed vaccines.

### **About Atamyio Therapeutics**

Atamyio 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, Atamyio leverages unique expertise in AAV-based gene therapy and muscular dystrophies from the Progressive Muscular Dystrophies Laboratory at Genethon. Atamyio’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. Atamyio 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.atamyio.com](http://www.atamyio.com)

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