MDA Conference 2026

Atamyo Therapeutics presents promising results in the first patients treated with its ATA-200 gene therapy in the clinical trial targeting LGMD-R5 limb-girdle muscular dystrophy

- *Four patients with LGMD-R5* (gamma-sarcoglycanopathy, formerly LGMD-2C) *have received the ATA-200 gene therapy as part of the ongoing Phase 1b/2 trial evaluating the safety, pharmacodynamics, and efficacy of ATA-200*.
- *Atamyo Therapeutics' clinical trial is currently being conducted at the Powell Gene Therapy Center, University of Florida, with Dr. Barry Byrne, MD, PhD, as the principal investigator.*
- *Nine-month follow-up data (including muscle biopsies performed at six months) for the first two patients treated have been reported: the safety of the product has been demonstrated and the efficacy results for these first two patients treated are very encouraging*.

Evry, France (March 9, 2026) - Atamyo Therapeutics, a biotechnology company specializing in the development of next-generation gene therapies for limb-girdle muscular dystrophy (LGMD), announced at the *MDA (Muscular Dystrophy Association) Conference 2026* the first safety, pharmacodynamics, and efficacy results for its ATA-200 gene therapy in LGMD-2C/R5 limb-girdle muscular dystrophy associated with γ-sarcoglycan deficiency (*SGCG,* gamma-sarcoglycanopathy**).** The results are from the first patients treated in the clinical trial conducted at the Powell Gene Therapy Center at the University of Florida by Dr. Barry Byrne and supported by The Dion Foundation for Children with Rare Diseases.

LGMD-2C/R5 is a severe form of muscular dystrophy that appears in childhood and causes loss of walking ability before adulthood, respiratory and heart failure, and premature death. This Phase 1b/2 clinical trial (NCT05973630) is a single-center study evaluating the safety, pharmacodynamics, efficacy, and immunogenicity of ATA-200 in children aged 6 to 13 years. ATA-200 is an adeno-associated virus (AAV) gene therapy carrying a normal copy of the human *SGCG* gene and administered as a single intravenous injection at a dose of 1.0E+14 vg/kg. This gene therapy product was developed by Isabelle Richard, a pioneer in the study of limb-girdle muscular dystrophies and the development of innovative therapies at Genethon.

At a dose of 1.0E+14 vg/kg, the following was observed in the first two patients treated with ATA-200:

- More than 90% of muscle fibers expressing the *SGCG* protein—demonstrating that almost all muscle fibers had received the therapeutic gene (90.2% for patient 1 and 92.1% for patient 2, biopsies at 6 months).

- A significant and sustained reduction in CPK levels (a biomarker of muscle damage) and a decrease in transaminases 9 months after treatment, demonstrating the significant efficacy of ATA-200 gene therapy.

- Also 9 months post-treatment, clinical benefits were observed on several other important parameters in ambulatory patients, particularly in timed functional tests.

No serious side effects were observed in the four patients treated, confirming the safety of the product.

"These initial results are very encouraging and demonstrate the potential of our product with biological data rarely seen in neuromuscular diseases and at such an early stage of the trial. I would like to commend the quality of the work done by the teams at Atamyo Therapeutics and, in particular, the commitment and determination of Isabelle Richard, which has made it possible to offer this hope to patients and their families. We are deeply grateful for the collaboration with the Dion Foundation and Dr. Barry Byrne of the Powell Gene Therapy Center and proud to offer children with LGMD-R5 the opportunity to receive a treatment that could change their lives." — Angela Columbano, CEO, Atamyo Therapeutics.

Further results from this ongoing study are expected to be published in the coming months, when new longer-term follow-up data becomes available.

We are grateful to the Dion Foundation for Children with Rare Diseases and CureSCG for their financial contributions that made it possible to launch the trial.

About the ATA-200 program in LGMD-2C/R5

About the ATA-200 program in LGMD-2C/R5 LGMD-2C/R5 is a rare genetic disease caused by mutations in the gene that produces γsarcoglycan, a transmembrane protein that is involved in the connection between muscle fibers and their environment. It affects an estimated 2,000 people in Europe and in the US. 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 presented as dilated cardiomyopathy, is reported in approximately half of patients and will eventually impact life expectancy. There is no curative treatment. The management of LGMD-2C/R5 is solely supportive. ATA-200, Atamyo's gene therapy for LGMD-2C/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.

ATA-200 has been awarded Orphan Drug Designation in the US and Europe, and Rare Pediatric Disease Designation by the US FDA.

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 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.

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