



Atamyo Therapeutics Announces First Three Patients Dosed with ATA-200 Gene Therapy in on-going LGMD-R5 Clinical Trial, and Extension of Partnership with the Dion Foundation

- *First three patients dosed with ATA-200 gene replacement therapy*
- *Extension of Partnership with The Dion Foundation for Children with Rare Diseases to support financing of the Phase 1b, dose-escalation study evaluating the safety and efficacy of ATA-200 in children with LGMD-R5*
- *First clinical results from the ongoing study conducted by Dr. B. Byrne (U. Florida) expected to be published in the coming months*

Evry, France and Boston, MA (February 3, 2026) - [Atamyo Therapeutics](#) and [The Dion Foundation for Children with Rare Diseases](#), today announced the expansion of their partnership to support financing of the first-in-human clinical trial of ATA-200, Atamyo's gene therapy for the treatment of the γ -sarcoglycan related limb-girdle muscular dystrophy Type 2C/R5 (LGMD2C/R5), and the dosing of a third US pediatric patient.

The Phase1b/2 open-label dose escalation study ([NCT05973630](#)) evaluates safety, pharmacodynamics, efficacy, and immunogenicity in children receiving intravenous ATA-200, a single-dose Adeno-Associated Virus (AAV) gene therapy carrying the human γ -sarcoglycan transgene. The study is being conducted at the Powell Gene Therapy Center, University of Florida, by Dr. Barry Byrne.

Additional results from this ongoing study are expected to be published in the coming months as further follow-up data become available.

"We are delighted to expand our partnership with Atamyo to treat more U.S. children in the very first clinical trial for children with LGMD2C/R5," said Courtney Dion, Co-Founder and President of the Dion Foundation. *"The Dion Foundation fully supports Atamyo's ATA-200 program and the promise it holds to bring real, lasting impact to children living with LGMD2C and their families."*

"We are deeply grateful of the collaboration with The Dion Foundation and with Dr. Barry Byrne at the Powell Gene Therapy Center." said Angela Columbano, Chief Executive Officer of Atamyo Therapeutics. *With the support of Atamyo's shareholder, Genethon, we are delighted of the progress of this first-in-human trial in LGMD-R5. Beyond the children currently enrolled, this study contributes important scientific and clinical knowledge that helps advance the field of therapies for LGMD for patients around the world, and we are*

proud of giving children affected by LGMD-R5 the opportunity to receive a treatment that could be life-changing.”

“We have been delighted to work with Atamyo and the Dion Foundation in the design and conduct of the ATA-200 study.” said Dr. Barry Byrne, Associate Chair of Pediatrics and Director of the Powell Gene Therapy Center, University of Florida, in Gainesville, Florida, where the first three patients were dosed, and principal investigator of this trial. *“We look forward to continuing assessing the potential benefit of ATA-200 in the first children to receive the product.”*

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 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 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. For more information visit www.atamyo.com

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