The Dion Foundation and Atamyo Therapeutics Announce a Partnership to Expand into the US Atamyo's Clinical Trial of ATA-200 Gene Therapy to Treat Limb-Girdle Muscular Dystrophy Type 2C/R5

- *The Dion Foundation for Children with Rare Diseases will finance expansion in the US of Atamyo's first-in-human trial of ATA-200.*
- *The Phase 1b, dose-escalation study to evaluate the safety and efficacy of ATA-200 in children has already received regulatory clearance in Europe.*
- *ATA-200 is a single-injection gene therapy aimed to treat limb-girdle muscular dystrophy Type 2C/R5 (LGMD2C/R5) caused by mutations in the γ-sarcoglycan gene*

Evry, France and Boston, MA (September 4, 2024) - Atamyo Therapeutics and The Dion Foundation for Children with Rare Diseases, today announced a key partnership to expand into the US a 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).

France-based Atamyo is a clinical stage biotechnology company focused on the development of new generation gene therapies targeting muscular dystrophies and cardiomyopathies. The US-based Dion Foundation is a non-profit organization devoted to increasing awareness and allocating funds towards research and development of potential treatments of rare genetic diseases, such as limb-girdle muscular dystrophy.

The Dion Foundation will finance deployment in the US of Atamyo's clinical trial of ATA-200 (NCT05973630). The study, sponsored by Atamyo, has already received regulatory clearance in France and Italy. This multicenter, Phase 1b, open-label, dose escalation study will evaluate safety, pharmacodynamics, efficacy, and immunogenicity in children of intravenous ATA-200, a single-dose Adeno-Associated Virus (AAV) vector carrying the human γ-sarcoglycan transgene.

"We are so grateful for the opportunity to establish a partnership with Atamyo to help facilitate bringing their groundbreaking research to a clinical site in the US for the very first clinical trial for children with LGMD2C/R5. This is a monumental step for the entire LGMD community," said Courtney Dion, Co-Founder and President of the Dion Foundation.

"We are thrilled by this key partnership and grateful to the Dion Foundation for their financial support which aims to include US patients in the first-in-human trial for ATA-200," said Stéphane Degove, CEO of Atamyo. "We are already engaged in the preparation of an IND filing in the US for ATA-200."

Atamyo is a spin-off of Genethon, a non-profit research organization that is a pioneer in developing gene therapies for rare diseases. ATA-200 is based on the research of Atamyo Chief Scientific Officer Isabelle Richard, Ph.D., Research Director at National Center of Scientific Research (CNRS) in France and head of the Progressive Muscular Dystrophies Laboratory at Genethon.

Genethon CEO Frederic Revah observed, "This partnership between the Dion Foundation and Atamyo, a spin-off from Genethon, represents another major milestone in our efforts over the past 30 years to develop gene therapies for rare diseases and bring them to patients worldwide."

LGMD2C/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. 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 presenting as dilated cardiomyopathy is reported in approximately half of patients. There is no curative treatment. The management of LGMD2C/R5 is solely supportive.

ATA-200, a gene therapy candidate for LGMD2C/R5, delivers a normal copy of the gene for production of γ-sarcoglycan protein. In preclinical models, a single systemic injection of ATA-200 demonstrated its tolerability and capability to correct symptoms and biomarkers of the pathology. ATA-200 has been granted Orphan Medicinal Product Designation by the European Medicines Agency (EMA) and it is the first treatment targeting LGMD-2C/R5 to enter clinical trials.

About The Dion Foundation for Children with Rare Diseases
The Dion Foundation is a non-profit organization established in 2023 with the mission to raise awareness of rare and ultra-rare genetic diseases affecting children, such as Limb-Girdle Muscular Dystrophy. We advocate for supportive legislation at both local and federal levels, and we are dedicated to allocating funds for research and the development of potential treatments and cures for these devastating conditions. At the Dion Foundation, we believe that no child should be left behind. Every child matters, and so do the countless others affected by rare genetic neuromuscular diseases.
For more information: https://www.thedionfund.org/contact-us

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 LGMD2I/R9 and LGMD2C/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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