Press Release For Immediate Release

Clinical Milestones, Vector Innovation Key to Gene Therapy, GenoTher Biocluster's First International Summit, Atamyo Therapeutics' Progress for LGMDs Highlight Genethon's Most Recent Newsletter

PARIS, FRANCE (June 19, 2025) – Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Telethon), and its spin-off Atamyo Therapeutics made significant advances in their gene therapies for multiple rare diseases as highlighted in Genethon's most recent Newsletter.

Chief among them are key clinical milestones reached, including those reflected in Genethon's Phase 3 ready Duchene muscular dystrophy gene therapy (GNT0004); and Atamyo's significant milestones in clinical trials of gene therapies (ATA-100 and ATA-200) for treatment of two subtypes of limb-girdle muscular dystrophy (LGMD2I/R9 and LGMD2C/R5).

In addition, the GenoTher Biocluster, a public-private partnership serving as a leading ecosystem for generation of innovative genetic medicines' approaches, held its first international summit attended by 350 researchers, investors, biotech executives and public decision-makers representing more than 100 organizations.

In his CEO Commentary, Genethon's Frederic Revah discusses new research involving the organization's AAV platform to increase safety, efficacy and disease applications of gene therapies. Dr. Revah reports Genethon also is exploring non-viral based delivery systems, such as Lipid Nano Particles (LNPs), for their potential in gene editing.

Atamyo in April completed the dose escalation phase of a Phase 1b/2b trial in Europe of ATA-100 for LGMD2I/R9 and announced in June dosing in the US of the first two patients in a Phase 1b/2 study of ATA-200 for LGMD2C/R5. Both subtypes lead to loss of ambulation and other complications in children and there are no cures.

Read the full Newsletter to learn about these gene therapy advances and others.

About Genethon
As a pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit laboratory that was established by AFM-Telethon. A first gene therapy for spinal muscular atrophy to which Genethon contributed has obtained a product license. With more than 200 scientists and professional staff, Genethon is pursuing its aim to develop therapies which

change the lives of patients suffering from rare genetic diseases. Thirteen products stemming from Genethon's R&D or from collaborations are in clinical trial for diseases of the liver, blood, immune system, muscles and eyes. Seven other products could enter clinical trials over the next five years. More information at www.genethon.com

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