Genethon to Present Efficacy Data from 3 Major Clinical Trials of Its Gene Therapy Products at the 2026 American Society of Gene & Cell Therapy Annual Meeting May 11-15 in Boston, MA

- *Duchenne muscular dystrophy (DMD) trial data will be presented from patients treated with Genethon's GNT0004 gene therapy in the early stages of its all-in-one Phase 1/2/3 trial, which currently is in the pivotal phase.*
- *Promising results also will be presented from the first patients treated with ATA-200 gene therapy in a clinical trial targeting LGMD-R5 limb-girdle muscular dystrophy.*
- *Data presented from the first patient treated with GNT-018-IDES trial demonstrates the feasibility of imlifidase as pretreatment in gene therapy for patients with Crigler–Najjar syndrome who are immune to AAV vectors.*
- *In addition, Genethon scientists will make 5 other oral and 13 poster presentations at the conference.*

PARIS, FRANCE (May 5, 2026) - Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Telethon), announced today its research and clinical trial data will be featured in eight oral presentations and 13 posters at the American Society of Gene & Cell Therapy Annual Meeting May 11-15 in Boston, MA.

Three members of Genethon's leadership team - Angela Columbano, Gerald Perret and Giuseppe Ronzitti – will be available for one-on-meetings during the conference. Dr. Columbano, PharmD, Ph.D, MBA, is Director of Business Development and Partnership. Dr. Perret, Ph.D., MBA, is Director of Development. Dr. Ronzitti, Ph.D. and DR2 at Inserm, is Director of Scientific Forecasting and Head of the Immunology and Liver Disease Laboratory. Contacts for the three are: Dr. Columbano (acolumbano@genethon.fr); Dr. Perret (gperret@genethon.fr); and Dr. Ronzitti (gronzitti@genethon.fr).

Barry Byrne, M.D., Ph.D., of the Powell Gene Therapy Center at University of Florida, will present promising of phase I/II results from four patients in the ongoing clinical trial of ATA-200 gene therapy for LGMD-R5 (gamma-sarcoglycanopathy, formerly LGMD-2C). In the first two patients treated by Dr. Byrne and his team, more than 90% of muscle fibers expressed 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). The clinical trial is led by Atamyo Therapeutics, a Genethon company.

Dr. Byrne's presentation, "*Gene therapy for LGMDR5: preliminary safety data from a Phase 1 study of ATA-200,*" will be in the Clinical Trials: In vivo gene transfer and gene-editing therapies session, May 13, 4.45 pm. to 5 pm.

Gerald Perret will review DMD patient data in an oral presentation titled "*Long-term follow-up of ambulatory boys treated with GNT0004, Genethon's AAV-based gene therapy for Duchenne muscular dystrophy: Results from the phase 1/2 part of the GNT-016-MDYF clinical trial*." The presentation will be in the Late Breaking Abstracts II session, May 15, 8 am to 9:45 am.

Giuseppe Ronzitti will present results of using imlifidase as a pre-treatment for GNT0003 gene therapy in a patient with a severe form of Crigler–Najjar syndrome, who is naturally immune to the AAV8 vector. This is the first time gene therapy has been successfully administered to a Crigler–Najjar syndrome patient with antibodies against AAV8. If the results are confirmed in the next stages of the trial, this approach could become a promising option for patients with antibodies to AAVs, who are currently ineligible for clinical trials and existing gene therapy treatments.

Dr. Ronzitti's presentation will be in the Advancing clinical trials and redosing session. (Organized by the International Coalition of Cell and Gene Therapy Societies) May 15, 10:15 am to 10:41 am.

Following are five other oral presentations by Genethon scientists:

May 13

- "*AAV gene therapy for Glycogen Storage Disease type III with a functional mini-GDE transgene rescues muscle impairment in aged mice and supports clinical translation through dose optimization, NHP and organoid model"* - Youssef Krimi Benchekroun; 10:15 am. Session: Translational advances in genetic and cellular therapies for muscle and skeletal disorders.
- *Liver-directed AAV delivery of an optimized C-terminal FGF23 transgene improves phosphate homeostasis and skeletal mineralization in an X-linked hypophosphatemia mouse model"* - Louisa Jauze; 10:30 am to 10:45 am. Session : Next-generation liver-directed genetic therapies for inherited metabolic diseases.
- *"Targeted gene addition of lentiviral vector in human hematopoietic stem cell by homology-mediated end joining pathway"* – Giulia Scalisi; 12:25 pm. Session: Poster talks II session.

May 14

- *"Challenges and solutions in the development of gene therapy for muscular dystrophies"* – Isabelle Richard, PhD; 9:00 am. Session: Emerging molecular therapeutic strategies for muscular dystrophies.

May 15

- *"CRISPR activation of utrophin as a mutation-independent approach for Duchenne muscular dystrophy therapy"* - Paola Galbiati; 4:45 pm. Session: Expanding cell and gene therapy through therapeutic RNA and epigenome engineering.

The following are 13 poster presentations from Genethon scientists:

- *"GLP-compliant six-month toxicity and biodistribution study following single intravenous administration of ATA-200 in Sprague-Dawley rats"* - Estelle Creoff
- *"Systemic GNT0004 Gene Therapy: Dose-Finding Study in the DMDmdx Rat Model"* - Estelle Creoff
- "*Mtm1 deficient rats as a new preclinical model for myotubular myopathy gene therapy"* - Badih Salman
- *"Intra-CSF administration of an AAV9 vector expressing human acid ceramidase prevents neurological signs in P361R-Farber mice"* - Marion Derome
- *"Internal optimization of the AAV5 capsid for improved transduction"* - Adrian Westhaus
- *"Integrated Immune Profiling Reveals Transgene-Directed B Cell and Inflammatory Responses in a DMD Patient Experiencing Severe Immune-Mediated Myositis"* - Philippe Veron
- "*Prime and base editing for the correction of pathogenic valosin-containing protein (VCP) mutations"* - Lydie Debaize
- *"Safety and Biodistribution of rAAV9-Mediated ASAH1 Gene Therapy in Nonhuman Primates"* - Jerome Denard
- "*Genotoxicity study of CRISPR/Cas9-based genome editing and AAV-transgene targeted integration in HSPC using Long Read-sequencing*" - Alexandra Tachtsidi
- *"Novel AI-designed AAV capsids targeting Integrin alpha V beta 6 shows superior muscle transduction, especially in diaphragm and enables effective low-dose gene therapy in muscular dystrophy models"* - Isabelle Richard
- *"Next-generation IgG degrading enzymes with improved specific activity, broader specificity across species and reduced antigenicity in humans"* - Rim Harb
- "*Therapeutic challenges in Glycogen Storage Disease type III: how proliferation and inflammation influence rAAV gene transfer stability in a mild fibrotic background"* - Youssef Krimi Benchekroun
- *"Impact of pre-existing neutralizing antibodies and IdeS treatment on AAV9 biodistribution in liver, heart and muscle in non-human primates"* - Rim Harb

About Genethon
A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a non-profit laboratory created by the AFM-Telethon. The first gene therapy drug, to which Genethon contributed, has been approved for marketing for spinal muscular atrophy. With more than 240 scientists and experts, Genethon's goal is to develop innovative therapies that change the lives of patients suffering from rare genetic diseases. Fifteen gene therapy products resulting from Genethon's research, or to which Genethon has contributed, are currently undergoing clinical trials for diseases of the liver, blood, immune system, muscles, and eyes. Others are preparing for clinical trials over the next five years. Discover the Genethon's pipeline: https://www.genethon.com/our-pipeline/

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