Press Release
October 1, 2025

# Genethon to Present Long-Term Follow-Up of Duchenne Muscular Dystrophy Patients Treated in Its Phase 1/2/3 Trial at the European Society of Gene & Cell Therapy 32nd Annual Congress October 7 – 10, 2025

- *The oral presentation October 9 will focus on patients treated in the dose escalation portion of Genethon's all-in-one Phase1/2/3 clinical trial.*
- *The Phase 3 trial is under way in Europe with the first patients included in France with Genethon's low-dose micro-dystrophin gene therapy GNT0004.*

**PARIS, France** (October 1, 2025) – Genethon, a worldwide pioneer and leader in research and development in gene therapy for rare genetic diseases, announced today it will detail long-term follow-up of Duchenne muscular dystrophy (DMD) patients treated in the dose escalation portion of the Phase 1/2/3 trial of GNT0004 in an oral presentation at the European Society of Gene & Cell Therapy 32nd Annual Congress in Seville, Spain, October 7 – 10, 2025.

Dr. Arnaud Valent, M.D., Genethon's Head of Medical Activities and Pharmacovigilance, will make the presentation, titled **GNT0004, Genethon's AAV-based gene therapy for Duchenne muscular dystrophy: long-term follow-up of ambulatory boys enrolled in the dose-escalation phase of GNT-016-MDYF**, on Thursday, October 9 at 7:15 pm CEST in Session 10d: *Late breaking news from the clinic*; Rm Parallel D.

Genethon CEO Frederic Revah said, "We have begun including patients in our Phase 3 trial, which was cleared by regulatory authorities based on the results of the Phase1/2 dose escalation portion. One of the strengths of our product is the dose selected for the pivotal phase, which is lower than those used in other gene therapy trials for DMD."

Dr. Revah also noted three other major oral presentations will be made at the conference, along with 19 poster presentations from Genethon scientists showcasing the latest results of their work in the field of gene therapy for patients with rare diseases, illustrating Genethon's advances in terms of novel therapy development, innovative vectors, and improved biomanufacturing methods.

"The oral presentations and posters reflect not only the expertise of our teams, but also Genethon's pioneering role and recognized leadership in this sector," Dr. Revah said.

One of the presentations involves a Phase 2 trial collaboration between Genethon and Hansa Biopharma. The study is evaluating the effectiveness of Hansa's antibody cleaving enzyme,

imlifidase, in breaking down Crigler-Najjar syndrome patients' pre-existing anti-AAV antibodies before they receive Genethon's gene therapy, GNT0003.

The presentation by Dr. Jeremy Do Cao of the Antoine-Beclere Hospital, APHP, titled **Overcoming AAV8 Immunity: First Seropositive Crigler-Najjar Patient Treated with GNT0003 Following Imlifidase Pretreatment (GNT-018-IDES clinical trial) is** on Friday, October 10 at 10 am CEST in Session 11a: *Metabolic Diseases II*; Rm Parallel A.

A third presentation, titled **Harnessing the potential of Immunoglobulin G degrading enzymes (Ide) for the treatment of AAV-seropositive patients** will be made by Dr. Giuseppe Ronzitti, the leader of Genethon's Immunology and Liver Diseases Team, on Thursday, October 9 at 8:30 am CEST in Session 7a: *Immune Responses to GT*; Rm Parallel A.

The fourth presentation, titled **Durability of AAV-based gene therapy in patients with Crigler Najjar syndrome at 4 years of follow up after dosing (the GNT-012-CRIG Study)**, will be made by Dr. Lorenzo D'Antiga of the Hospital Papa Giovanni XXIII, Bergamo**,** Italy, on October 10, at 10 am CEST in Session 11b: *AAVs/Non Integrative Vectors III, Translation;* Rm Parallel B.

Genethon scientists will make 19 poster presentations.

**AAVs/non integrative vectors**

P0004: A second-generation myotropic capsid targeting integrin alpha V beta 6 with enhanced transduction efficacy in skeletal and cardiac muscles: Ai Vu Hong
P0019: Therapeutic advantage of a Dual AAV-Split Intein MIDI Dystrophin revealed by dynamic muscle function assay in animal and human DMD models: Maxime Ferrand
P0020: Optidys: a dual-AAV gene therapy strategy for Duchenne muscular dystrophy: Sonia Albini
P0021: Systemic AAV vector readministration by combination of natural and bioengineered capsids: Edith Renaud-Gabardos
P0022: Comparison study of intravenous and intracisternal administration in NHP of a rAAV9 gene therapy for acid ceramidase deficiency related disorders: Michael Hocquemiller
P0041: Shuffling of HVR in AAV capsid reveals a context-dependent role for HVR5 in peripheral tissue detargeting while ensuring CNS targeting: Christian Leborgne
P0047: 11-year of efficacy of a single administration of AAV8 microdystrophin gene therapy in a GRMD dog: Estelle Creoff
P0063: AAV8-mediated gene replacement corrects the metabolic pathology in a liver-specific mouse model of Glycogen-storage disorder type 1A: Michael Blatzer
P0183: Same vectors, different contaminants: tracking HCPs across rAAV serotypes: Gregory Rouby
P0189: Accelerating small scale development in gene therapy: Fast and robust full capsids quantification using Mass Photometry Technology from Refeyn (SamuxMP): Clotilde Ciesla

**Cardiovascular & muscular diseases**

P0274: Therapeutic perspective of metabolic normalization in Duchenne muscular dystrophy: Elise Lachiver

P0291: The DMDmdx rat: a representative model of Duchenne muscular dystrophy skeletal, cardiac, and respiratory deficiencies: Estelle Creoff

P0300: Quantitative MRI as a Valuable Surrogate Biomarker for Monitoring Disease Progression in DMD: Insights from Genethon's Multicenter Natural History Study with Standardized Follow-Up: Arnaud Valent

**Disease models**

P0375: Mtm1-deficient rats as a new preclinical model for myotubular myopathy gene therapy: Badih Salman

**Gene editing**

P0437: Novel homology-mediated end joining-IDLV precise integration for Therapeutic Genome Editing in Hematopoietic Stem Cells: Giulia Scalisi

P0472: CRISPR activation of utrophin as a mutation-independent approach for Duchenne muscular dystrophy therapy: Paola Galbiati

**Skin, pulmonary and skeletal Diseases**

P1141: Preclinical development of GNT0008, a Gene therapy product to treat LGMDR1: Anthony Brureau

**Immune responses to GT**

P0613: Muscle-specific expression reduces early antigen presentation and promotes CD8 T cell tolerance after rAAV gene transfer: Lindsay Jeanpierre

**Manufacturing**

P0956: Improvement of AAV quality, quantity and scale-up focusing on the transfection step and dissolved CO2: Tom Nocerra