Dr. Giuseppe Ronzitti to Present Genethon's Latest Clinical Data and Gene Therapy Advances at the Hope for Rare Science Conference 2026 in Shanghai, China.

- *Dr. Ronzitti, Director of Research Strategy, will present two-year efficacy and safety data from patients treated in the early stages of Genethon's all-in-one Phase 1/2/3 trial of GNT0004, a low dose micro-dystrophin gene therapy, currently in Phase 3 trial in Europe.*
- *A second presentation will focus on expanding gene therapy indications to largest populations possible usingsafe and efficient gene transfer, including a Crigler-Najjar syndrome gene therapy (GNT0003) clinical trial exploring the use of imlifidase to overcome patients' pre-existing anti-AAV immunity.*

PARIS, FRANCE (June 22, 2026) - Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Telethon), announced today Giuseppe Ronzitti, Ph.D., Director of Research Strategy and Director of Research (DR2) at Inserm, will make two oral presentations at the 3rd Hope for Rare Science Conference in Shanghai, China, June 25 – 27, 2026.

Dr. Ronzitti's presentation, titled "Two-year results from the Phase 1/2 study of GNT0004, an AAV-based gene therapy for Duchenne muscular dystrophy," details significant efficacy in treatment of three ambulatory boys aged 6–10 years with the selected GNT0004 pivotal dose of micro-dystrophin (3×10^{13} vg/kg), which is lower than doses used in other DMD gene therapies. The patients sustained reductions in serum creatine kinase (CK), a key marker of muscle damage, and showed stabilization or improvement in motor function. GNT0004 also was well tolerated, with no serious adverse events or unexpected safety signals reported. The presentation will be on June 26th at 8.30am (local time).

In the second oral presentation, titled "Overcoming gene therapy limitations to achieve safe and efficient gene transfer in genetic rare diseases," Dr. Ronzitti discusses Genethon's progress in addressing key challenges, including immune responses to viral vectors, tissue targeting specificity, durability and manufacturing scalability.

In a clinical trial involving Crigler-Najjar syndrome patients, naturally immune to AAV vectors, pretreatment with imlifidase, an enzyme capable of temporarily inhibiting the immune response, successfully "cut" the patient's antibodies and enabled treatment with Genethon's GNT0003 gene therapy for this rare liver disorder. No severe side effects related to GNT0003 or imlifidase were reported. The presentation will be on June 26th at 4 pm (local

time).

"We are honored to participate for the first time in the Hope for Rare Science Conference in Shanghai, a major international event for innovation in gene therapy. Being invited as a guest speaker is a strong recognition of the work carried out by our teams and of Genethon's unique model, which integrates cutting-edge academic research, translational development, and bioproduction and scale-up capacities.

This congress represents a valuable opportunity to share our clinical advances, particularly with GNT0004 for Duchenne muscular dystrophy, and to present our approaches to overcoming key challenges in gene therapy and expand patient's access to transformative therapies.

Beyond the scientific discussions, our participation to this meeting represents an important opportunity to strengthen dialogue with the global scientific community and reinforce collective strategies to advancing innovative therapies for patients with rare diseases" – said Dr Giuseppe Ronzitti.

The Hope for Rare Science Conference features 22 parallel sessions and several satellite meetings with more than 80 speakers from more than 10 countries, including representatives from academia, industry, regulatory agencies, and foundations.

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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