Genethon's R&D to be Featured in Multiple Presentations at American Society of Cell & Gene Therapy Annual Meeting May 16-19, 2022, in Washington, DC

PARIS, FRANCE (May 11, 2022) – Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Telethon), announced today its research will be featured in six presentations at the 25th Annual Meeting of the American Society of Cell and Gene Therapy (ASCGT), May 16-19, 2022, at the Walter E. Washington Convention Center in Washington, D.C. The international convention attracts thousands of scientists, physicians, patient advocates and government officials. Attendees can participate in-person or virtually.

"Our scientists not only are presenting some of the most significant advances to date in gene therapy research, but they also are serving as co-chairs for several portions of the program at this distinguished global convention," said Frederic Revah, Genethon CEO. "Genethon has pioneered development of gene therapy for rare diseases for more than 30 years and thousands of patients around the world already are benefitting from our research."

Genethon's Anne Galy, Ph.D., Director of the Integrare Research Unit, is co-chair of the Hematopoietic Stem Cell Gene Therapy oral abstract session and the Inborn Metabolic Issues scientific symposium, and Mario Amendola, Ph.D., Genethon's Head of the Gene Editing Laboratory, is co-chair of the RNA Virus Vectors oral abstract session.

Following are Genethon's oral abstract and poster session presentations (all times are U.S. EDT).

Monday, May 16, 3:45 pm – 5:30 pm, Salon G

Tools and Approaches for Inborn Errors of Metabolism Oral Abstract Presentations

- Ex Vivo Editing of Hematopoietic Stem Cells for Erythroid Expression of Therapeutic Proteins In Vivo for LAL-D Therapy, Monday, May 16; 5:15 pm - 5:30 pm. - Marine Laurent

Monday, May 16, 5:30 pm – 6:30 pm, Hall D

AAV Vectors Preclinical and Proof-of-concept Studies I Poster Session

- Bioengineered AAV Vectors with Increased Skeletal Muscle Potency and Specificity for Systemic Gene Therapy [Board No. M-29] – Edith Renaud-Gabardos
- Preclinical Development of a Gene Therapy for Gamma Sarcoglycanopathy [Board No. M-54], – Isabelle Richard

Tuesday, May 17, 5:30 pm – 6:30 pm, Hall D

Pharmacology/Toxicology Studies or Assay Development Poster Session

- Novel Cytometry Based Characterization of Lysosomal Disease Affected and Gene Corrected Patient's Cells [Board No. Tu-290] – Marine Laurent

Wednesday, May 18, 5:30 pm – 6:30 pm, Hall D
Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases II Poster Session

- AAV-Mediated Gene Therapy of Spinal Muscular Atrophy with Progressive Myoclonic Epilepsy (SMA-PME) and Farber Disease [Board No. W-137] – Jérôme Denard

Musculo-skeletal Diseases Poster Session

- AAV Gene Transfer of Dlk1-Dio3 miRNAs Indicates That the miRNA Cluster Regulates Mitochondrial Activities in Duchenne Muscular Dystrophy [Board No. W-179] – Ai Vu Hong

About Genethon
A pioneer in the discovery and development of gene therapies for rare diseases, Genethon is a unique non-profit organization created by a patient association, the AFM-Telethon. A first gene therapy drug, to which Genethon contributed, has obtained marketing for spinal muscular atrophy. With 200+ scientists and professionals, Genethon is pursuing its mission to bring life-changing therapies to patients suffering from rare genetic diseases. 12 products resulting from Genethon's research are in clinical trials for eye, liver, blood, immune system and muscle diseases. A further 7 products are in the preparation phase for clinical trials over the next five years. Find out more: genethon.com

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