Genethon's Scientists Featured in Multiple Presentations at American Society of Gene & Cell Therapy Annual Meeting May 7-11, 2024, in Baltimore, MD

- *Genethon is a global leader in developing gene therapies for rare diseases*
- *Organization aims to make gene therapy a staple of 21st century medicine*

PARIS, FRANCE (May 7, 2024) - Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Téléthon), announced today its research will be featured in two oral presentations and five posters at the 27th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), May 7-11, in Baltimore, MD. The international convention attracts thousands of scientists, physicians, patient advocates, and government officials.

"Gene therapy is a unique medical opportunity to treat currently untreatable diseases. Our presentations and posters demonstrate the breadth of Genethon's gene therapy research and the dedication of our scientists to create new treatments for patients suffering from rare genetic diseases," said Frederic Revah Ph.D., Genethon CEO. "With more than 200 scientists and professional staff, Genethon is a global leader in gene and cell therapies and is dedicated to helping make gene therapy a staple of 21st century medicine."

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

Oral presentations:

Tuesday, May 7 – 2.15 pm - Lysosomal storage disorders – Room 309-310

"Rescue of lysosomal acid lipase deficiency in mice by AAV liver gene transfer" – Marine Laurent, a Ph.D. student in the Genome Editing Team led by Mario Amendola, Ph.D. The presentation will describe the development of a liver-based in vivo gene therapy for lysosomal acid lipase deficiency (LAL-D), a progressive genetic metabolic disease, using rAAV8. Results of the study demonstrated stable LAL enzyme expression able to correct the toxic lipid accumulation and extend lifespan in a LAL-D mouse model.

Wednesday, May 8 – 1.30 pm – CAR T and Other Genetically Modified Immune Cells – Room 339-342

"Development of a FAP-CAR T cell protocol to reduce skeletal muscle fibrosis in a murine model of Duchenne muscular dystrophy" – Anne Galy, Ph.D., will introduce the design of tools that generate fibrosis-specific killer CAR T cells to reduce fibrosis of the muscle tissues and improve the Duchenne muscular dystrophy gene therapy treatment.

Five Genethon posters will be presented during the Congress:
Wednesday, May 8; 12pm – 7pm, Exhibit Hall

- ***Identification of AAV2-13 hypervariable regions mediating liver transduction and detargeting across species*** **– Tiziana La Bella**

- ***Disease exacerbation in 3D MYOtissues derived from Duchenne muscular dystrophy iPSC reveals muscle strength loss and enables gene therapy screening*** – **Laura Palmieri**

Thursday, May 9; 12pm – 7pm, Exhibit Hall

- ***Determinants of AAV persistence in a mouse model of Glycogen storage disease type III*** **–** Tiziana La Bella

- ***Innovative IDLV design for transgene targeted integration via microhomology-mediated end joining pathway*** **–** Giulia Scalisi

- ***Fibrosis restricts rAAV gene transfer and can be relieved by FAP-specific CAR T cells in a murine model of Duchenne muscular dystrophy*** **–** Anne Galy

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 Généthon 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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