Press Release

The Latest Research of 14 Genethon Scientists to be Featured at the European Society of Gene & Cell Therapy's 30th Annual Congress, Oct. 24-27, 2023, in Brussels, Belgium

The gene therapy pioneering organization's research, aimed at curing rare and ultra-rare genetic diseases, is featured in 3 oral and 11 poster presentations

PARIS, FRANCE (October 24, 2023) – Genethon, a unique non-profit gene therapy R&D organization founded by the French Muscular Dystrophy Association (AFM-Telethon), announced today 14 scientists will make oral and poster presentations at the European Society of Gene & Cell Therapy's 30th Annual Congress October 24 – 27, 2023 in Brussels, Belgium.

"Our scientists will be presenting Genethon's latest advances in gene therapy research, product development and biomanufacturing," said Frederic Revah, Genethon CEO. "The breadth of this significant research is testament to the growth in importance of gene therapy in modern medicine. I am proud of the worldwide leadership role of our 200 scientists and professionals who are dedicated to developing new therapies for rare diseases."

In addition to the presentations, two of Genethon's scientists from the Immunology and Liver Diseases Team are serving as co-chairs of oral presentation sessions: **Giuseppe Ronzitti**, leader of the team, is co-chair of Session 3c on *Gene Therapy for metabolic diseases at preclinical stage* Wednesday, October 25, from 8:30 to 10:30; and **Sylvain Fisson** is co-chair of Session 11c on *Update on immune responses to gene & cell therapy* Friday, October 27, 11:00 to 13:00. All times are CEST.

The following are Oral Presentations by Genethon scientists:

Wednesday, October 25
Session 3b: AAV, non-integrative vectors I (8:30 to 10:30)

Ai Vu Hong - *An Integrin-targeting AAV developed by a novel computational rational design methodology presents an improved targeting to the skeletal muscle and reduced*

Thursday, October 26
Session 8b: AAV, non-integrative vectors II (15:00 to 17:00)

Giuseppe Ronzitti - *Innate immunity to AAV vectors: the devil's in the details*

Friday, October 27
Session 10b: Gene editing: Preclinical development (8:30 to 10:30)

Maelle Ralu - *CRISPR-Cas9 mediated endogenous utrophin upregulation improves Duchenne Muscular Dystrophy*

The following 11 Genethon scientists will make Poster Presentations :

AAV and Non-Integrative Vectors

Evelyne Gicquel - *A micro-RNA regulated AAV vector prevents the cardiotoxicity induced by transgene overexpression following FKRP gene transfer* - P060

Christian Leborgne - *Development of an alternative barcoding method to assess in vivo transduction efficiency of AAV vectors in rodent and non-human primate* - P082

Joelle Cheuzeville - *Screening of transfection reagents using a Design of Experiment approach* - P165

Nicolas Beard - *Multiple method determination of AAV integrity for gene therapy* - P121

Nadia Halzoun - *Long-term and accelerated stability of an AAV8-based gene therapy* - P175

Jessica Cartigny - *Development of full capsid enrichment step in AAV9 purification process with CIMmutus PrimaT monolithic column* - P204

Metabolic Diseases

Jeremy Rouillon - *Single AAV gene therapy with mini-Glycogen Debranching Enzyme for glycogen storage disease type III* - P559

Gene Editing

Laurie Lacombe - *NHEJ inhibition increases homology mediated AAV integration in hematopoietic cells* - P654

Immune Responses to Gene Therapy

Novella Tedesco - *Unequivocal detection of AAV-mediated gene doping: a two-step approach to the identification of vector transduction events* - – P763

Lindsay Jeanpierre - *Early T-cell activation after recombinant Adeno-Associated Virus delivery* - P766

Sylvain Fisson - *Extreme inter-individual variability of subretinal AAV-induced immune response even in a highly standardized context* - P767

About Genethon

As a pioneer in the discovery and development of gene therapies for rare diseases, Généthon 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 developed by Genethon 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.fr.

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