Genethon will introduce its latest advances in gene therapy research in multiple presentations at the International Myology 2024 Congress

PARIS, FRANCE (April 22, 2024) - Genethon, a unique non-profit gene therapy R&D organization, announced today its research will be featured in 3 oral presentations and 18 posters of their latest research on neuromuscular diseases at the 8th International Myology 2024 Congress in Paris, April 22 to 25, 2024.

Dedicated to advances in research and therapeutics on muscle and its diseases, Myology 2024 is organized by Genethon's founder AFM-Téléthon and will bring together more than 1,000 international scientists and experts over four days. More than 70 Genethon researchers and experts will participate.

For Genethon, Myology 2024 will be an occasion to announce the first clinical results of its gene therapy trial for Duchenne muscular dystrophy. They will be presented by Professor Francesco Muntoni (London), principal investigator of the trial, on Tuesday April 23 from 3:00 to 3:20 pm during a "Late breaking news" session.

"The Myology 2024 Congress is an opportunity for our scientists to present Genethon's latest advances in gene therapy innovations," said Genethon CEO Frederic Revah. "Our participation this year will be marked by exceptional announcements that reflect the research excellence and expertise of our teams in designing and developing gene therapy treatments and bringing them to patients."

Oral presentations:

Tuesday, April 23 – 2:30 pm - Young investigators symposium

"*The damaged lysosome is a therapeutic target for combined therapy in Duchenne muscular dystrophy* " - Abbass Jaber, PhD student in Isabelle Richard's team, will demonstrate the existence of dysfunctions in muscle cell lysosomes in Duchenne muscular dystrophy patients, and present how this discovery could enable the development of combined therapies with gene therapy.

Tuesday, April 23 – 3 pm - Late Breaking News

« First data of the phase I/II part of the clinical trial of GNT0004 AAV8-µdystrophin gene therapy in ambulant DMD boys » - Professor Francesco Muntoni, principal investigator (Great Ormond Street Hospital for Children in London) will introduce the first clinical results of gene therapy clinical trial for Duchenne Muscular Disease.

Wednesday, April 24 – 11 am - Parallel session 2 // LGMD
"*Development of gene therapy for three prevalent forms of LGMD* " – Evelyne Gicquel, PhD, Researcher in Isabelle Richard's Progressive Muscular Dystrophies team, will introduce the latest advances in the development of gene therapy drug candidates for three different limb-girdle muscular dystrophies (LGMD).

18 Genethon posters will be presented during the Congress:

- ***Deep Learning algorithm for determination of muscle fiber type on Hematoxylin Eosin (HE) stained histology slices*** **–** J**érémie Cosette**

- ***Quantitative muscle MRI for predicting disease progression in thigh muscle of Duchenne muscular dystrophy patients*** **– Fei Cao**

- ***Lipid metabolism is disrupted in mouse and human models of FKRP deficiency, and rescued after FKRP gene transfer*** **– Evelyne Gicquel**

- ***FKRP related Limb-Girdle Muscular Dystrophy: a biomarker identification study*** **– Stephany Campuzano**

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

- ***Evaluation of gene transfer efficiency in a model of dystrophic muscle disorder performed by machine learning and linear discriminant analysis*** **– Anthony Brureau**

- ***Evaluation of muscle fiber membrane impairment in isolated fibers of a dysferlin mouse model*** **– Anthony Brureau**

- ***Occurrence of lysosomal damage in the dystrophic muscle and its evaluation by Galectin-3*** **– David Israeli**

- ***The SGCG KI mouse: a new animal model for sarcoglycanopathies*** **– Valeria Agostini**

- ***Prime editing as a potential therapeutic for p97 mutations derived neurodegeneration and neuromuscular diseases*** **– Tao Wang**

- ***An Integrin-targeting AAV developed by a novel computational rational design methodology presents an improved targeting to the skeletal muscle and reduced tropism to the liver*** **– Laurence Suel-Petat**

- ***Optimization of gene therapy vectors for alpha -sarcoglyconapathies*** **– Elise Lachiver**

- ***The engineered AAVpo1.A1 vector transduces efficiently murine and human skeletal muscle fibers with liver detargeting*** **– Edith Renaud-Gabardos**

- ***Spastin, a protein involved in hereditary spastic paraplegia, regulates ER-mitochondrial contact sites and mitochondrial homeostasis* – Andrea Burgo**

- ***Understanding neuromuscular affectations in acid ceramidase deficient mice and correction by gene therapy* – Marion Derome**

- ***Heart toxicity can be induced in rat after injection of high level of AAV expressing Gamma–sarcoglycan using the Desmin and MHCK7 but not the tMCK promoter* – Jérôme Poupiot**

- ***Immunotherapy treatment with FAP-specific CAR-T cells can reduce skeletal muscle fibrosis and promote AAV gene therapy efficiency in a murine model of Duchenne muscular dystrophy* – Sonia Albini**

- ***Treatment of myotonic dystrophy type 1 with CRISPR/Cas9* – Paloma Navas-Navarro**

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 Genethon 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.fr

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