Press release
December 1st 2020

Genethon gets the green light from the ANSM to start an innovative gene therapy trial for Duchenne muscular dystrophy

Genethon, dedicated to designing and developing gene therapy products for rare diseases, received this Monday 30th of November the authorisation from the ANSM, the French National Agency for Medicines and Health Products Safety, to start in France a multicentre international clinical trial for the treatment of Duchenne muscular dystrophy with product GNT 004. This trial's goal is to assess the safety and efficacy of an innovative gene therapy designed at Genethon, combining an AAV-type viral vector with a shortened version of the dystrophin gene, "microdystrophin".

Duchenne muscular dystrophy is a rare, progressive genetic disease that impacts all the muscles in the body and affects 1 in 3500 boys. It is associated with abnormalities in the gene responsible for the production of dystrophin, a protein that is essential to the proper functioning of the muscles. This gene has the characteristic of being one of the largest in our genome (2.3 million base pairs, more than 11,000 of which are coding). Due to its size, it is technically impossible to insert the complete DNA sequence coding for dystrophin into a viral vector (or even the 11,000 coding base pairs alone), as is usually done in gene therapy.

GNT 0004: An innovative technology

The innovative technology used for this clinical trial is GNT 0004, a gene therapy combining an AAV-type viral vector and a shortened version of the dystrophin gene, microdystrophin, designed by researchers and experts at Généthon, in collaboration with Prof. George Dickson's team (University of London). Following demonstrated safety and efficacy in preclinical animal studies (*Nature Communications*, July 2017), Généthon and Sarepta Therapeutics, the leader in precision gene medicine for rare diseases, announced a collaboration to complete preclinical development, then to co-develop the clinical program.

A multicentre international trial

The international trial of boys with Duchenne muscular dystrophy, which will be coordinated by Prof. Francesco Muntoni (Great Ormond Street Hospital, London), and clinical sites are planned in the United Kingdom, the United States and Israel.
In France, it will be conducted at the following investigating centres: the Hautepierre Hospital in Strasbourg (Prof. Vincent Laugel), I-Motion, Trousseau Hospital, in Paris (Prof. Odile Boespflug-Tanguy), and hospitals in Brest, Bordeaux, Lyon, Marseille and Lille

Pre-inclusion study already underway

An international pre-inclusion study has already started at the trial investigating centres in France. This study, whose objective is to follow the natural progression of the disease using endpoints that will define the therapeutic trial endpoints, will follow up, over a period of 3 months to 3 years, around 100 young boys aged 5 to 9 years who are still able to walk. This study will help to identify children who could be included in the gene therapy trial.

"From translational research to clinical application, Généthon has supported this candidate drug, which is the concrete result of years of cutting-edge research conducted at our laboratory. Beyond France, Généthon plans to extend this study, to the United Kingdom, the United States and Israel. This trial for this devastating disease, marks a new decisive stage for Généthon, an institution created 30 years ago by AFM-Téléthon" explains Frédéric Revah, CEO of Généthon.

About Genethon - genethon.fr

Genethon, a pioneer in gene therapy for rare diseases, was created in 1990 by AFM-Téléthon, thanks to donations from the first French Telethons. The challenge at the time was huge: deciphering the human genome, tracking the genes responsible for genetic diseases and using this knowledge to create innovative medicines. Today, a product incorporating patents and the results of research work conducted at Généthon is available on the market for spinal muscular atrophy, a neuromuscular disease, and a second product is at the marketing authorisation application stage, for a rare disease affecting vision. A total of 9 other products resulting from R&D at Généthon, developed alone or in collaboration, are at the clinical trial stage around the world for rare diseases of the muscle, vision, liver, immune system and blood, and a further 6 products are at the preclinical stage.
Accessing the Généthon pipeline
Follow Genethon on LinkedIn

Press contact:

Stéphanie Bardon / Marion Delbouis – Tel: +33 (0)1 69 47 29 01 –
communication@genethon.fr