Press release
Tuesday, April 20th 2021

Genethon announces First Patient dosed in Clinical Trial of Investigational Gene therapy GNT 0004 for Duchenne Muscular Dystrophy

A first participant was dosed at I-Motion, the pediatric clinical trial platform for neuromuscular diseases located at Trousseau hospital in Paris, as part of the gene therapy trial in Duchenne muscular dystrophy (DMD) conducted by Genethon.

A young boy suffering from Duchenne muscular dystrophy received a first dose of the investigational gene therapy GNT 0004 at I-Motion, the pediatric clinical trial platform for neuromuscular diseases in Paris. He was the first patient in an international phase I/II/III multicenter trial for which Genethon is the sponsor. The trial has been approved in France by the French National Agency for Medicines and Health Products Safety (ANSM) and in the UK by British Medicines & Healthcare products Regulatory Agency.

"***Dosing this first patient is a step that is profoundly symbolic for Genethon. This trial is the culmination of 30 years of pioneering research by Genethon. It embodies the quality of the research conducted in our laboratories, in collaboration with high-performing international teams. Duchenne is a very challenging disease and while we are cautious, we are hopeful and proud that the technologies developed at Genethon are today becoming drug candidates that could change the future for patients suffering from Duchenne muscular dystrophy***" explains CEO Frédéric Revah.

"***There remains a tremendous unmet need for treatments to help individuals affected by DMD. We put high hopes in this novel candidate*** " says Prof. Muntoni from the Dubowitz Neuromuscular Center (UCL Great Ormond Street Institute of Child Health & Great Ormond Street Hospital (London, UK)), the principal investigator for the trial. There is no cure for Duchenne muscular dystrophy, and the development of an effective treatment is exceptionally challenging. Duchenne Muscular Dystrophy has been a cornerstone of the fight led by AFM-Téléthon and Genethon.

The gene therapy (GNT 0004) is based on an adeno-associated virus (AAV) capsid and an optimized gene, a shortened version of the gene coding for dystrophin, the protein that is absent in patients with Duchenne muscular dystrophy. This micro-dystrophin, associated with a vector designed to be expressed in muscle tissues, was developed by Genethon, in partnership with the teams of Prof. Dickson (University of London, Royal Holloway) and the Institute of Myology (Paris). It is now developed jointly in the clinical phase with Sarepta Therapeutics.

About the trial

This phase I/II/III gene therapy trial is a multicenter dose determination trial, followed by the randomized efficacy part of the trial, to assess the product's efficacy versus placebo. The cross-over is planned after the one year following the treatment with placebo in order to allow all participants to potentially benefit from the treatment. The trial uses a single intravenous injection of GNT 0004. The trial aims to enroll boys aged 6 to 10 suffering from Duchenne muscular dystrophy who are still able to walk. The trial was approved in France, in the UK, and submissions are ongoing in the USA and Israel.

The main criterion for evaluation of efficacy is the change on the North Star Ambulatory Assessment (NSAA) score at one year. The NSAA is a validated 17-item rating scale that is used to measure functional motor abilities in ambulant children.

About Duchenne muscular dystrophy

Duchenne muscular dystrophy is a rare progressive genetic disease that affects all the muscles in the body, including the heart muscle, and affects 1 in 3,500 boys. It is connected to abnormalities in the gene responsible for producing dystrophin, a protein essential for the muscle to work. This gene is one of the largest in our genome (2.3 million base pairs, over 11,000 of which are coding). Because of its size, it is technically impossible to insert the complete coding DNA sequence for dystrophin into a viral vector (or even only the coding sequence), as is usually done in gene therapy.

About Genethon

Genethon was created in 1990 by the AFM-Telethon with the donations from the first Telethon. The stakes at that stage were huge: deciphering the human genome, tracking down the genes responsible for genetic diseases and using this knowledge to make innovative drugs. Thirty years later, a first gene therapy drug, to which Genethon contributed, has obtained marketing authorization in the United States, Europe and Japan for spinal muscular atrophy. In addition, 10 products resulting from Genethon research and developed alone or in collaboration, are today in clinical trials for rare diseases involving eyesight, the liver, blood, the immune system and the muscles. A further 8 products are in the preparation phase for clinical trials over the next five years.

Genethon's pipeline

https://www.genethon.fr/en/products/

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