Genethon announces expanded collaboration with Sarepta Therapeutics for continued development of an innovative gene therapy for Duchenne muscular dystrophy

Evry (France) – January 9th, 2020 - **Genethon, a French laboratory dedicated to the design and development of gene therapy products for rare diseases, and Sarepta Therapeutics, the leader in precision genetic medicine for rare diseases, today announced an expansion of their collaboration to develop a gene therapy approach for Duchenne muscular dystrophy. Microdystrophin has demonstrated significant efficacy in pre-clinical testing*. The agreement between Sarepta and Genethon paves the way for a clinical trial to begin in 2020.**

Gene therapy combining an AAV-type viral vector with a shortened version of the dystrophin gene (microdystrophin) has been developed by Genethon researchers in collaboration with Pr George Dickson's team (University of London) to treat patients suffering from Duchenne Muscular Dystrophy (DMD). This approach has demonstrated significant efficacy in dogs naturally affected by the disease with a high expression of microdystrophin level and a significant restoration of muscle function with a stabilization of clinical symptoms.

On the basis of these results, Genethon and Sarepta initiated a research collaboration in 2017 to finalize the preclinical development of the product. The agreement announced today reinforces and expands this collaboration with the co-development of the clinical gene therapy program using this microdystrophin approach. Under the terms of agreement, Genethon will be responsible for commercializing the product GNT0004 in Europe (excluding the UK) and Sarepta will be responsible for the rest of the world. Genethon is entitled to receive an upfront payment, development and commercial milestones and royalties from Sarepta.

YposKesi, one of Europe's largest GMP vector production sites, with 160 bio-production experts and a dedicated site of 5,000 sqm, has been entrusted with the production of clinical and large-scale commercial batches based on innovative suspension-based production methods.

To meet the needs, YposKesi has planned to expand its production capacity and increase the volume of its bioreactors.

"*This cutting-edge technology is the result of years of research and development based on the expertise of Genethon researchers, pioneers in the field of gene therapy for rare diseases. Combining this promising approach, which targets the majority of Duchenne patients, with the know-how of our partner Sarepta is a new opportunity for patients. We have just started a clinical study of pre-inclusion of patients ("baseline" study) to allow us to precisely evaluate the efficacy of the product. This is a very concrete step towards the injection of the first patient with the product in the coming months",* states Frédéric Revah, CEO of Genethon.

Nature Communications: Long-term microdystrophin gene therapy is effective in a canine model of Duchenne muscular dystrophy

About Duchenne muscular dystrophy

Duchenne muscular dystrophy is a rare, progressive genetic disease that affects all the muscles of the body and affects 1 in 3500 boys. It is the most common neuromuscular disease in children. It is linked to abnormalities in the DMD gene, which is responsible for the production of dystrophin, a protein that is essential for the proper functioning of the muscle. This gene has the characteristic of being one of the largest in our genome (2.3 million base pairs of which more than 11,000 are coding). Because of this size, it is technically impossible to insert the complete DNA of dystrophin in a viral vector (or even the only 11,000 coding base pairs), as is usually done for gene therapy.

About Genethon - www.genethon.fr

Created by the AFM-Telethon and located in Evry (France), Genethon is a non-profit research and development centre dedicated to the development of biotherapies for rare diseases, from research to clinical validation. Genethon specializes in the discovery and development of gene therapy drugs and has several ongoing clinical, preclinical and research programs for genetic diseases of muscle, blood, immune system and liver. A first product to which Genethon has contributed has obtained marketing authorization in the United States in 2019 and is currently undergoing approval in Europe for spinal muscular atrophy. 7 other products resulting from Genethon's R&D, alone or in collaboration, are currently in clinical trials, and several others are in the preparation phase for clinical trials in 2020 and 2021.

Accessing Genethon's pipeline

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