

Press Release
Evry, avril 28, 2015

Généthon, Winner of the World Innovation Competition 2030

Tuesday April 28, during a ceremony at the Elysee Palace, Généthon, the laboratory of the AFM-Telethon, was named recipient of the Global Innovation Competition 2030 in the category "Risk lift." A prize that rewards the expert laboratory and world leader in the field of gene therapy for the development of an industrial production process of gene therapy vectors.

The Global Innovation Competition 2030 aims to "*bring out the talents and future champions of the French economy by identifying and supporting the growth of French and foreign entrepreneurs whose innovative project has a particularly strong potential for the French economy*" said the Elysee at launch.

Gene therapy is an area with high medical and economic potential. Indeed, this innovative therapy, which involves replacing a diseased gene with a functional gene, has increased the therapeutic success for rare diseases (affecting 3 million people in France, 30 million in Europe) than for common diseases. Future needs can therefore involve tens of thousands of patients a year. The challenge is to industrialize the manufacturing processes of gene therapy vectors, these "carriers" of gene-drug complex to produce. **The objective of Généthon program called IGT, aims to develop a generic method for the production of lentiviral vectors to target industrial.** Clinical studies have indeed demonstrated the reliability of these vectors, their efficiency and enhanced security compared to their precursors retroviral (see press release of April 21, 2015 "[New gene therapy success in a rare disease of the immune system: Wiskott-Aldrich syndrome](#)"). Therefore, this process will produce a large scale, in pharmaceutical terms that is to say, according to Good Manufacturing Practices (GMP), developing a specific business model. It is with this objective that the World Innovation has allocated \$ 1.298 million euros at Genethon IGT program that responds to **public health issues and economic development on high value-added industry competition and that with a view to mastering the production of innovative biotherapies in France.**



"This award is a real pride and recognition of the expertise of our laboratory in the field of gene therapy and its industrial applications. Généthon represents the excellence of French research and plays a major role in the development of a essential sector of innovative biotherapies for the benefit of patients, and high economic value" said Frédéric Revah, CEO of Généthon.



A pride widely shared by Laurence Tiennot-Herment, President of AFM-Telethon and Généthon: *"Since the first hour, the AFM-Telethon has chosen innovation. Because everything had to be invented, because for our patients there was urgency, rare diseases have been the catalyst for therapeutic innovation for the benefit of the greatest number. We are very proud to receive this award today and, through it, the trust and State involvement (ou the State's commitment) in this difficult and costly fight (ou action) against rare diseases."*

About AFM-Téléthon - www.afm-telethon.fr

The French Muscular Dystrophy Association (AFM) federates patients with neuromuscular diseases and their parents. Thanks in great part to donations from France's annual Telethon (€89,3 million in 2013), the AFM-Telethon has become a major player in biomedical research for rare diseases in France and worldwide. It currently funds about 30 clinical trials in different genetic diseases affecting the eye, blood, brain, immune system, and muscles... Thanks to its Généthon research lab, the AFM-Telethon stands out through its unique ability to produce and test its own gene-based medicines.

About Généthon - www.genethon.fr

Created by the AFM-Telethon, Généthon's mission is to make available to patients innovative gene therapy treatments. Having played a pioneering role in deciphering the human genome, Généthon is today, with more than 200 scientists, physicians, engineers and regulatory affairs specialists, an international research and development center for preclinical and clinical gene therapy treatments for rare diseases. Généthon has the largest site in the world for GMP production of gene therapy products, Généthon Bioprod. In 2012, Généthon was the first associative laboratory to receive the 2012 Prix Galien for Pharmaceutical Research (France). As part of a therapeutic program on genetic diseases of the blood and immune system, Généthon working for over 10 years on gene therapy of Wiskott-Aldrich syndrome. The laboratory is currently conducting clinical trials for this disease in Europe, Paris and London, and the United States with the Children's Hospital Boston.

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