PRESS RELEASE

Généthon, the French AFM-Telethon laboratory, becomes the first not-for-profit to obtain authorization from ANSM to be a pharmaceutical manufacturer

Evry, June 27, 2013. Généthon, the AFM-Telethon laboratory, has received the authorization delivered by the National Agency for Drug Safety (ANSM) to become a pharmaceutical manufacturer. Its production center, Généthon BioProd, is now authorized to produce drugs for innovative treatments. This is a first for a laboratory created by an association of patients and financed thanks to the generosity of the public, and a new stage in the emergence of innovative treatments for rare diseases.

With Généthon BioProd, the AFM-Telethon laboratory has the greatest capacity for drugs for gene therapy in the world. From proof of concept to clinical development and in compliance with Good Manufacturing Practices (GMP) regulations, Généthon, which received the Prix Galien France 2012, strengthens its position as a world leader in the domain of biotherapies for rare diseases. It is the first not-for-profit association laboratory to obtain this pharmaceutical establishment status in accordance with the law of March 22, 2011.

With 5000 m^2 of high-tech laboratories and four L3 biological containment suites, Généthon BioProd has a production capacity of more than 20 batches of lentivirus or AAV vector-drugs for clinical trial phases in humans in order to make these biologics available to patients. Already sponsor of two international clinical trials for immune deficits, Généthon will now be able to continue its clinical development projects for rare diseases of vision, muscles, blood, the liver, and the brain.

"This authorization delivered by ANSM marks a major step in the history of AFM-Telethon and its Généthon laboratory. For the first time, a not-for-profit association, created by patients and their families, financed through public generosity, becomes a pharmaceutical manufacturer. This excellent tool will allow us to accelerate our development programs for innovative biotherapies for rare diseases, in the service of the general interest," says Laurence Tiennot-Herment, President of AFM-Telethon and Généthon.

For Frederic Revah, CEO of Généthon, "*With the Prix Galien France 2012, this authorization as a pharmaceutical manufacturer confirms the role of Généthon as a world leader in the domain of gene*

therapy. Thanks to Généthon BioProd, we will manufacture drugs at large scale for innovative therapy for trials in humans and thereby pursue our objective: making treatments available to patients with rare diseases for which no therapy is available."

The construction costs for Généthon BioProd were 28.5 million euros, of which 5.5 million euros were financed by AFM-Telethon, 8 million euros by the Ile de France Regional Council, 7 million euros by the Essonne General Council, and 8 million by the Evry Genopole. Its annual operating costs (about 10 million euros) are integrally financed by AFM-Telethon thanks to donations to the Telethon.

Généthon BioProd, a high-tech site
with a production capacity that is unique in the world

- Concept satisfies High Quality Environmental objectives (HQE®)
- 5 000 m^2 dedicated to GMP-manufacturing and testing of gene therapy products including 2500 m2 of confined and classified containment laboratories
- 4 production suites for a total of 500 m²
- 2 suites for aseptic fill and finish operations
- 120 m² of pilot laboratories dedicated to industrialization of optimized manufacturing procedures
- 500 m² of laboratories for quality control under GMP norms
- 15 units for air treatment –interior air in the containment zones is 100 000 to 500 000 times cleaner than ambient air
- 3 km of circular ducts

Production capacities
- Up to 1 000 liters of culture in bioreactors for AAV-type products, per batch
- Up to 100 liters of cultures of lentivirus-type vectors, per batch
- More than 20 batches of vector-drugs per year at full capacity

Groups
60 bioproduction experts: pharmacists, engineers, technicians

About the AFM-Telethon: The French Muscular Dystrophy Association (AFM) federates patients with neuromuscular diseases (genetic diseases that causing progressive irreversible muscle atrophy lead to death) and their parents. Thanks in great part to donations from France's annual Telethon (€94.1 million in 2011), the AFM-Telethon has become a major player in biomedical research for rare diseases in France and worldwide. It currently funds 36 clinical trials for about 30 different genetic diseases affecting the eye, the blood, the brain, the 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: Généthon, located in Evry, France, is a non-profit organisation dedicated to the development of biotherapies for orphan genetic diseases. Généthon has unique experience in the international research community in gene therapy and muscle disorders, and has several clinical trials ongoing or in preparation for neuromuscular, blood, liver and eye diseases. Généthon has one of the largest viral vector production facilities in the world, and has unique expertise in assessing the quality and efficacy of viral vectors for clinical application.

Press Contacts

Stéphanie Bardon / Géraldine Broudin / Gaëlle Monfort
01 69 47 12 78 / 25 64 / 28 59 - presse@afm.Généthon.fr