PRESS RELEASE

Evry, February 24, 2010

Gene therapy of rare diseases

Généthon initiates a new clinical trial for a severe immune deficiency (Wiskott-Aldrich syndrome)

Généthon, the not-for-profit biotherapy laboratory operated by the French Muscular Dystrophy Association (AFM) with funding from the country's annual Telethon*, has just obtained approval from the French and British health authorities for a Phase I/II clinical trial of gene therapy for a rare immunodeficiency, Wiskott-Aldrich syndrome (WAS). The Généthon-sponsored trial will be performed both in France (led by Professors Alain Fischer (1) and Marina Cavazzana-Calvo (2) at Necker Children's Hospital, Paris) and in the UK (led by Professor Adrian Thrasher at London's Great Ormond Street Hospital). **With just a few days to go to Rare Disease Day 2010* (February 28th, with a focus on "Bridging Patients and Researchers"), initiation of this trial marks a new step towards curing rare diseases with innovative biotherapies.**

Wiskott Aldrich syndrome (WAS) is a severe, X-linked immunodeficiency which results in hemorrhage, recurrent infection and eczema. It is almost always fatal in affected children. Although a bone marrow transplant can reestablish the patient's immune system, this operation requires a compatible donor and carries a non-negligible risk of failure. Gene therapy (which has already enabled the treatment of other hereditary immune deficits) therefore represents a major source of hope for patients waiting for therapy.

The forthcoming clinical trial is the end result of research initiated in 2002 by Anne Galy's group (UMR951) (3) in the Généthon laboratory. The lab's researchers have developed an *ex vivo* approach that uses an HIV-derived lentiviral vector to transfer genes into autologous $CD34^+$ hematopoietic stem cells from the patient. Généthon has developed, produced and quality-controlled the trial's batches of gene-based medicines under GMP conditions.

The trial also demonstrates the extent of the AFM lab's know-how, ranging from preclinical research and the GMP production of gene-based drugs through to trial logistics and regulatory approval.

"This trial is enabling the Généthon laboratory (funded almost exclusively by donations from France's annual Telethon) to demonstrates its world-class expertise in the production of gene-based medicines and the preparation and implementation of clinical trials. It confirms Généthon's ability to become a major international player in demonstrating the efficacy of gene therapy for rare diseases" emphasized AFM and Généthon President Laurence Tiennot-Herment.

About Généthon

Généthon is a not-for-profit research centre that was created by the French Muscular Dystrophy Association (AFM) and is now funded almost exclusively by donations from France's annual Telethon. Its goal is to deliver gene therapies to patients with rare diseases in general and neuromuscular diseases in particular. With over 200 scientists, physicians, engineers and regulatory affairs specialists, Généthon is one of the world's leading centers for preclinical and clinical research and development in the field of gene therapy. Généthon also has a biomanufacturing platform for clinical-grade vectors and is involved in building the world's largest facility for pre-industrial pilot production. A 5000 m^2 facility will open in Evry in early 2011. www.genethon.fr

About the AFM

The French Muscular Dystrophy Association (AFM) federates patients with neuromuscular diseases (genetic diseases that kill muscle after muscle) and their parents. Thanks in great part to donations from France's annual Telethon (€90 million in 2009), the AFM has become a major player in biomedical research into rare diseases in France and worldwide. It is currently funding 34 clinical trials on 30 different genetic diseases affecting the eyes, the blood, the brain, the immune system, the muscle... Thanks to its Généthon research lab, the AFM stands out through its unique ability to produce and trial its own gene-based medicines.

* More information at www.afm-telethon.fr and www.rarediseaseday.org

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