**PRESS RELEASE**

**Evry, 2 March 2009**

# Gene therapy
# Production of the first batch of HIV-derived vectors in Europe for use in a human clinical trial

**Généthon**, the laboratory created and funded by the **AFM** (French Association against Myopathies) using donations from Téléthon, has announced the 20th of February that it has **produced, controlled and released a batch of lentiviral vectors derived from the human immunodeficiency (HIV) virus** for a gene therapy trial in humans in a rare immune deficiency. The *Etablissement de thérapie génique et cellulaire* (ETGC – gene and cell therapy unit) at Généthon thus becomes **the first organisation in Europe to have produced this type of vector in accordance with GMP (good manufacturing practice) standards**. Part of the safety controls were carried out by GenoSafe[1], a company created by the AFM and Généthon.

Since it was founded in 2005 and officially approved by the health authorities in 2006, the ETGC at Généthon has been working on a number of different vectors. In particular, it carried out sterile filling operations, quality control and batch release of AAV vectors used in the phase I gene therapy trial conducted for a neuromuscular disease: gamma-sarcoglycanopathy. It has also produced and released oncoretroviral vectors for future trials in graft-versus-host disease, inclusion body myositis and autoimmune diseases.

The release of this first batch of HIV-derived vectors, produced in accordance with GMP standards, is a crucial step in the preparation of the clinical trial authorisation application to be submitted to Afssaps (the French drug safety authority) during the course of March. Following approval by the health authorities, this batch will be used in a gene therapy trial for Wiskott-Aldrich syndrome, a rare immune deficiency. **Généthon will be the sponsor of this trial, which is expected to be launched during the last quarter of 2009.**

*"This first is good news for our Généthon laboratory, confirming its capacity to serve as a major European tool to demonstrate the effectiveness of gene therapy in the treatment of rare diseases",* states Laurence Tiennot-Herment, President of the AFM, and also President of the laboratory since 1 January 2009.

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[1] *Founded in 2003 by Généthon and the AFM, GenoSafe is a* ***service company specialising in evaluation of the efficacy and safety of biotherapeutic products****. From development of products until they are brought to market, GenoSafe conducts studies meeting the regulatory requirements and specifically tailored to its customers' needs. For more information: www.genosafe.com*

***About Généthon***

*The Généthon laboratory is unique in the world and, since its inception, has been a strong driving force accelerating genetic research and a source of exceptional innovation. After mapping the human genome and identifying the genes responsible for diseases, it then tackled the development of gene therapy tools and their application in humans for rare diseases. It thus became the first non-pharmaceutical laboratory to be authorised to produce batches of medicine vectors for trials in humans. To achieve this, Généthon has two clinical vector batch production units accredited by Afssaps (ETGC) and is committed to the development of a pre-industrial scale GMP production site (Gamma project) by 2010. In addition to the trial in Wiskott-Aldrich syndrome, Généthon is also continuing the gene therapy trial launched in 2006 for a neuromuscular disease.*

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