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Innovative biotherapies and genetic eye diseases: the *Réseau Thérapie Génique Oculaire* (Ocular Gene Therapy Network) prepares to launch human trials

Tomorrow, around thirty innovative biotherapy and genetic eye disease experts* will gather together at Généthon (Evry) to review, for the first time, the studies carried out so far as part of the *Réseau Thérapie Génique Oculaire* (R-TGO – Ocular Gene Therapy Network).

The network was launched in 2009 by Généthon and is co-led by José-Alain Sahel (*Institut de la Vision*, Paris), Fabienne Rolling (Nantes) and Laurence Tiennot-Herment (Généthon/*Association Française contre les Myopathies*). Made up of project teams, representatives from centres of excellence in the field of eye diseases and gene therapy experts from the Généthon laboratory, its objective is to **accelerate the launch of gene therapy trials for genetic eye diseases in the next five years**. Around a dozen clinical projects for various genetic eye diseases (including Leber's congenital amaurosis, Leber's hereditary optic neuropathy (LHON), Wolfram syndrome (also called DIDMOAD - Diabetes Insipidus, Diabetes Mellitus, Optic Atrophy, and Deafness), Bardet-Biedl syndrome and other retinal dystrophies) have now been identified as targets by the network. The meeting to be held tomorrow, 4 June, at Généthon will be an opportunity to define strategies aimed at overcoming the common obstacles currently holding up these trials.

"Gene therapy has already proved effective in restoring sight in puppies blinded by Leber's congenital amaurosis, a genetic disease. A trial will soon be launched in young patients. To ensure this advance benefits all genetic eye diseases, we have played an active role in the creation of this network of specialists, to which Généthon will contribute its expertise as well as its scientific, technical and regulatory structures. Our objective is to work together to achieve the therapeutic successes now within our grasp as soon as possible", said Laurence Tiennot-Herment, President of Généthon and the AFM.

For Alain-José Sahel (*Institut de la vision*), a co-leader of this network: *"This remarkable AFM-Généthon initiative has already led us to identify several projects offering significant potential, to harness the skills liable to accelerate progress towards clinical trials without compromising patient safety, and to create a network of centres of excellence".*

The *Rétina France* and *Fédération Française des Aveugles de France* (French Blind People's Federation) patients associations will also be in attendance on 4 June. **In addition to the dozens of rare diseases concerned, advances made in the field of innovative biotherapies may potentially benefit some very common conditions, such as age-related macular degeneration, which currently affects 20% of the over 50s.**

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 centres 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*

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