PRESS RELEASE

Evry (France), March 8th, 2011

Généthon and Wake Forest University School of Medicine (North Carolina) announce their collaboration for a preclinical gene therapy trial

Généthon (the not-for-profit biotherapy lab funded by the French Muscular Dystrophy Association (*Association Française contre les Myopathies*, AFM) with donations from France's annual Telethon) and Wake Forest University School of Medicine (Winston Salem, North Carolina, USA) **today announced that they are to collaborate on the preclinical development of a gene therapy for myotubular myopathy.** This rare genetic disease is a very severe congenital myopathy which affects skeletal muscles and leads to respiratory failure from birth.

Généthon/INSERM researcher Dr Anna Buj Bello has been working on murine models of myotubular myopathy for many years, in order to understand the disease mechanism and design therapeutic strategies. Her research group has developed an adeno-associated virus (AAV) vector for transferring a healthy copy of the myotubularin gene into muscle cells.

The collaborative work will test this approach in a canine disease model characterized by Martin Childers, D.O., Ph.D., a professor in the Department of Neurology and the Institute for Regenerative Medicine at Wake Forest. Généthon will transfer its method for local-regional perfusion of AAV vectors, supply expert advice and produce the batches of vectors to be used in the work in the United States.

This transatlantic collaboration will help the researchers to understand the mechanisms involved in the disease and complement the data generated in mice. If the results are confirmed in dogs, the therapy could then be clinically tested in humans.

"We are very pleased to be working with Dr Childers' group at Wake Forest. The great match between his group's skills and those at Généthon will enable us to accelerate the development of a gene therapy for myotubular myopathy", commented Généthon Chief Executive Frédéric Revah.

"Myotubular myopathy is an extremely severe muscle disease and we urgently need a treatment. The AFM is delighted with this collaboration between its operational arm Généthon and Wake Forest. Patients and their families should ultimately gain significant benefit from this work", added Laurence Tiennot-Herment, Chairperson of AFM and Généthon.

"Généthon has developed unique skills and know-how in the field of gene therapy for rare diseases in general and neuromuscular diseases in particular. For our research group, this collaboration with Généthon represents an opportunity to turn academic work into therapeutic reality", stated Dr Martin Childers, the trial's lead investigator.

About Généthon

Généthon is a not-for-profit research centre that was created by the French Muscular Dystrophy Association (AFM) and is funded almost exclusively by donations from France's annual Telethon. Its goal is to deliver innovative therapies to patients with rare diseases.
After having played a pioneering role in the sequencing of the human genome and 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 for rare diseases. Généthon also has a biomanufacturing platform for clinical-grade vectors and is involved in building the world's largest industrial pilot plant: the 5000 m^2 Généthon BioProd site will open in Evry (France) in early 2011. www.genethon.fr

About the AFM

The French Muscular Dystrophy Association (*Association Française contre les Myopathies,* 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 (€95 million raised in 2009), the AFM has become a major player in biomedical research into rare diseases in France and worldwide. It is currently funding 36 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.

Wake Forest University Baptist Medical Center (www.wfubmc.edu) is an academic health system comprised of North Carolina Baptist Hospital, Brenner Children's Hospital, Wake Forest University Physicians, and Wake Forest University Health Sciences, which operates the university's School of Medicine and Piedmont Triad Research Park. The system, which includes Lexington Memorial Hospital, comprises 1,004 acute care and rehabilitation beds and has been ranked as one of "America's Best Hospitals" by *U.S. News & World Report* since 1993. Wake Forest Baptist also holds the Gold Seal of Approval™ from The Joint Commission, the nation's esteemed standards-setting and accrediting body for health care quality. Wake Forest Baptist has more than 200 physicians listed in Best Doctors in America®. The institution is in the top third in funding by the National Institutes of Health and fourth in the Southeast in revenues from its licensed intellectual property.

About the Wake Forest Institute for Regenerative Medicine

The Wake Forest Institute for Regenerative Medicine (www.wfirm.org), part of Wake Forest University Baptist Medical Center, is an established center dedicated to the discovery, development and clinical translation of regenerative medicine technologies by leading faculty. The institute has used biomaterials alone, cell therapies, and engineered tissues and organs for the treatment of patients with injury or disease. It's scientists were the first in the world to engineer laboratory organs in the lab that were implanted in human patients.

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