PRESS RELEASE

Evry, January 5th, 2011

Partnership for a gene therapy Clinical trial signed between Généthon and Children's Hospital Boston

A unique multicenter international study of gene therapy for a rare genetic disease (Wiskott-Aldrich syndrome) led in London, Paris and Boston.

Généthon, the not-for-profit biotherapy laboratory funded by the French Muscular Dystrophy Association (AFM) thanks to the donations collected during the French Telethon, and Children's Hospital Boston announce that they have initiated a partnership to conduct a gene therapy clinical trial for Wiskott-Aldrich Syndrome (WAS). This rare genetic condition is a life-threatening immunodeficiency disease. Genethon is sponsoring parallel trials at Great Ormond Street Hospital in London and Hopital Necker-Enfants Malades in Paris (Press release – Feb 24, 2010) and will be supplying the vector for the trial at the US site. The vector is a latest-generation lentiviral vector that incorporates several safety features designed to avoid the complications caused by early-generation vectors. The vector is manufactured by Genethon in Evry. Altogether, **the WAS gene therapy trials in London, Paris and Boston will constitute a unique multicenter collaboration to accelerate the testing of new advanced therapies for rare conditions.**

The **Wiskott Aldrich Syndrome (WAS)** is a rare primary immune deficiency disease causing significant bleeding due to low platelets and increased numbers of serious infections. Most patients have mild to severe eczema and are also at a higher risk of developing autoimmune disorders and malignancies such as lymphoma.

The forthcoming clinical trial results from a research program initiated in 2002 by the group of Anne Galy at Genethon (Inserm UMR951/Généthon, Université d'Evry Val d'Essonne, EPHE), which has developed an *ex vivo* approach that uses an HIV-derived lentiviral vector to transfer genes into autologous CD34+ hematopoietic stem cells from WAS patients.

The Boston trial will be funded by the National Heart, Lung and Blood Institute (NHLBI) through its Gene Therapy Resource Program (GTRP). Principal investigators at Children's are Sung-Yun Pai, MD, Division of Hematology/Oncology, and Luigi Notarangelo, MD, director, Research and Molecular Diagnosis Program on Primary Immunodeficiencies, Division of Immunology. David A. Williams, MD, chief of the Division of Hematology/Oncology and director of Translational Research for Children's Hospital Boston, will serve as sponsor-investigator.

The London WAS trial ongoing at Great Ormond Street Hospital/University College of London Institute of Child Health is conducted by Adrian J Thrasher MD, Professor of Paediatric Immunology and H. Bobby Gaspar MD Professor of Paediatrics and Immunology.

The trial ongoing at Necker-Enfants Malades in Paris is conducted by Marina Cavazzana-Calvo MD, Head of Clinical Investigation Center Biotherapy, Necker, Alain Fischer MD, Professor of

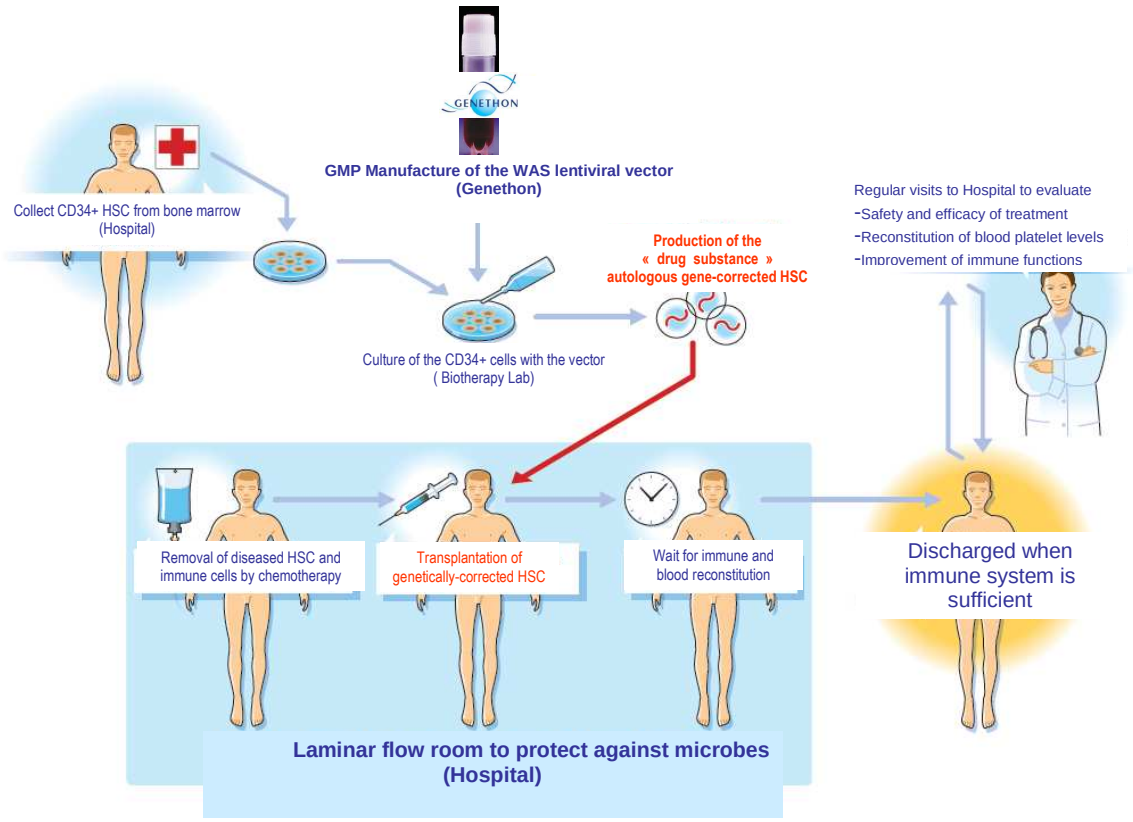
Pediatric Immunology, Scientific Director of the Imagine Foundation and Salima Hacein-Bey Abina PhD, Professor of Immunology and Head of the Gene Therapy Laboratory.

"This gene therapy trial (being run simultaneously in London, Paris and Boston) wouldn't have been possible without the Telethon. The AFM is taking on a huge challenge – biotherapeutics. This means new hope for millions of patients and new medical tools for the good of all." comments Laurence Tiennot-Herment, Chairperson, AFM and Généthon

"We are delighted with this new collaboration with the group headed by Prof. Williams. It demonstrates again the ability of Genethon to bring its research projects to the clinic in collaboration with the best clinical teams worldwide, for the benefit of patients. It reflects our cutting edge expertise in the field of translational research, bioproduction and preparation and implementation of clinical trials" emphasizes *Frederic Revah, CEO Genethon.*

"At Children's Hospital Boston, we are committed to utilizing state-of-the-art cell and molecular therapies to treat devastating pediatric diseases. The trial in WAS is particularly noteworthy as it represents a continuing transatlantic scientific and clinical research collaborative effort in gene therapy and huge multi-disciplinary team effort at Children's" comments Dr. David A. Williams, Sponsor-Investigator of study.

Schematic representation of the gene therapy trial for Wiskott Aldrich



About Généthon

Généthon is a not-for-profit research centre that was created by the French Muscular Dystrophy Association (AFM) and funded almost exclusively by donations from France's annual Telethon. Its goal is to deliver gene therapies to patients with rare diseases. 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 (Genethon BioProd), which will open in Evry in early 2011. www.genethon.fr
More information at www.afm-telethon.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 (€95 million 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.

About the Children's Hospital Boston

Children's Division of Pediatric Hematology/Oncology is internationally-known for treating young children and adolescents with all types of cancer and blood disorders. Its services are offered through two distinct services, Hematology and Oncology. The Pediatric Hematology service treats children with all types of non-malignant blood disorders. The Pediatric Oncology service, provided via the Dana-Farber/Children's Hospital Cancer Center treats patients with cancer as well as those requiring stem cell transplantation.
The Division of Immunology at Children's Hospital Boston is a leading center worldwide for the diagnosis and treatment of patients affected with immune deficiency diseases and for the identification of the underlying genetic defects.
Children's Hospital Boston is home to the world's largest research enterprise based at a pediatric medical center, where its discoveries have benefited both children and adults since 1869. More than 1,100 scientists, including nine members of the National Academy of Sciences (USA), 12 members of the Institute of Medicine and 13 members of the Howard Hughes Medical Institute comprise Children's research community. Founded as a 20-bed hospital for children, Children's Hospital Boston today is a 392-bed comprehensive center for pediatric and adolescent health care grounded in the values of excellence in patient care and sensitivity to the complex needs and diversity of children and families. Children's also is the primary pediatric teaching affiliate of Harvard Medical School. For more information about the hospital and its research visit: www.childrenshospital.org/newsroom.

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