PRESS RELEASE

Evry, France - July 21st, 2011

Genethon and Children's Hospital Boston get FDA approval for a Wiskott Aldrich gene therapy trial

The US Food & Drug Administration (FDA) approved the launching in the U.S. of a clinical trial for gene therapy for a rare immunodeficiency, Wiskott-Aldrich syndrome (WAS). After its implementation in Paris and London, this trial based on preclinical research performed at Genethon (Evry, France) which also manufactures the GMP gene therapy product, is now going to be launched in Boston. It's one of the first international clinical trials using a gene therapy treatment for a rare disease.

Earlier this year (Press release - Jan 5, 2011), 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 announced that they have initiated a partnership to conduct a gene therapy clinical trial for Wiskott-Aldrich Syndrome (WAS), a severe immunodeficiency disease leading to death before adulthood. 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 is supplying the vector for all clinical sites. In total, this trial will involve fifteen patients, five for each site, who will be treated by 2013-2014. The sites in London and Paris have already started treating patients under this protocol. 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 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 gene insertion of patient cells will be accomplished in the Connell O'Reilly Family Good Manufacturing facility at the Dana-Farber Cancer Institute in collaboration with the Center for Human Cell Therapy at Harvard Medical School.

Wiskott Aldrich Syndrome (WAS) is a rare primary immune deficiency disease causing significant bleeding due to low platelet count and increased incidence 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.

"This authorization constitutes a new key stage for the project WAS initiated in 2002 by Anne Galy and her team at the Généthon. It is also an international recognition of the quality of the

work of Généthon. Without the donations collected during the French Telethon, this international gene therapy trial could not exist" states Laurence Tiennot-Herment, Chairperson, AFM and Généthon.

"We are very happy with the approval given by the FDA for the extension of the WAS clinical trial by 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.

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 2012.
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 2010), 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 test its own gene-based medicines.
More information at www.afm-telethon.fr

About Children's Hospital Boston

Children's Division of 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 Hematology service treats children with all types of non-malignant blood disorders. The 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 9 members of the National Academy of Sciences, 11 members of the Institute of Medicine and 9 members of the Howard Hughes Medical Institute comprise Children's research community. Children's Hospital Boston has been ranked as one of the nation's best pediatric hospitals by U.S.News & World Report for the past 21 years. Founded as a 20-bed hospital for children, Children's Hospital Boston today is a 396 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 research and clinical innovation at Children's,
visit: http://vectorblog.org

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