



NEWS RELEASE

bluebird bio and Généthon Announce Manufacturing Research Collaboration Centered on Lentiviral Vectors for Gene Therapy

Cambridge, Mass. And Evry, France, December 14, 2010 – [bluebird bio](#) (formerly Genetix Pharmaceuticals Inc.), an emerging leader in the development of innovative gene therapies for severe genetic disorders, and Généthon, a leader in the field of gene therapy treatments for rare diseases, today announced a research collaboration focused on process development and scale up efforts for the manufacturing of lentiviral vectors. This agreement is designed to enable substantial advances in existing manufacturing process for the benefit of both partners. It will also strengthen bluebird bio's manufacturing platform to enable robust, commercial-scale manufacturing and broaden the company's capabilities in Europe. Specific terms of the agreement were not disclosed.

"Généthon has strong interest and focus on the development and manufacturing of gene therapy products and is an excellent strategic fit for bluebird bio," said Nick Leschly, president and chief executive officer of bluebird bio. "Généthon has a distinguished reputation in gene therapy, and over the years has helped identify the genes responsible for hundreds of diseases, developed vectors for gene transfer therapy and established a GMP manufacturing organization. We look forward to collaborating with the project team on this research and manufacturing project."

The *Etablissement de thérapie génique et cellulaire* (ETGC – gene and cell therapy unit) at Généthon was the first organization in Europe to have produced HIV-derived lentiviral vectors in accordance with GMP (good manufacturing practice) standards. Since it was founded in 2005 and officially approved by the health authorities in 2006, the ETGC at Généthon has released GMP-compliant batches of a number of different vector types.

"We are delighted to work with bluebird bio, a leader in the development of innovative gene therapies for severe genetic disorders," said Frédéric Revah, Ph.D., chief executive of Généthon. "As a non-profit R&D organization for biotherapy, Généthon has the mission of providing patients affected with rare genetic disease access to gene therapy treatments. We believe bluebird bio is at the forefront of one of the most promising advances in gene therapy, and we look forward to being able to combine our expertise with that of bluebird bio and achieve significant improvements in the production of lentiviral vectors for gene therapy through this collaboration."

bluebird bio's approach uses stem cells harvested from the patient's bone marrow into which a healthy version of the disease causing gene is inserted.

After being grown in culture, those cells are given back to the patient. This approach represents a true paradigm shift in the treatment of severe genetic diseases by eliminating the potential complications associated with donor cell transplantation and potentially presenting a one-time transformative therapy. bluebird bio has two distinct product candidates in clinical development: one for [childhood cerebral adrenoleukodystrophy](#) (CCALD), and another program (LentiGlobin[®]) for [beta-thalassemia and sickle cell anemia](#). In addition to these initial disease targets, the company's gene therapy platform is expected to be applicable to the treatment of other severe genetic diseases.

About bluebird bio

bluebird bio is developing innovative gene therapies for severe genetic disorders. At the heart of bluebird bio's product creation efforts is its broadly applicable gene therapy platform for the development of novel treatments for diseases with few or no clinical options. The company's novel approach uses stem cells harvested from the patient's bone marrow into which a healthy version of the disease causing gene is inserted. After being grown in culture, those cells are given back to the patient. bluebird bio's approach represents a true paradigm shift in the treatment of severe genetic diseases by eliminating the potential complications associated with donor cell transplantation and presenting a one-time transformative therapy. bluebird bio has two later stage clinical products in development for childhood cerebral adrenoleukodystrophy (CCALD) and beta-thalassemia/sickle cell anemia. Led by a world-class team, bluebird bio is privately held and backed by top-tier life sciences investors, including Third Rock Ventures, TVM Capital, Forbion Capital Partners, Easton Capital and Genzyme Ventures. Its operations are located in Cambridge, Mass. and Paris, France. *For more information, please visit www.bluebirdbio.com.*

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 in general and neuromuscular diseases in particular. With over 200 scientists, physicians, engineers and regulatory affairs specialists and after having played a pioneering role in the global effort to decipher the human genome, Généthon is now a world-leading centre 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. Généthon has two ongoing clinical trials: a multicenter international study focusing on Wiskott Aldrich syndrome (a rare primary immune deficiency disease) and a second trial focusing on gamma-sarcoglycanopathy (a rare neuromuscular disease). *To know more: www.genethon.fr*

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