NEWS RELEASE

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NATURE Publishes Promising Results on Treatment of First Patient in bluebird bio's Phase 1/2 Beta-Thalassemia Study

Successful Clinical Response Achieved in beta-Thalassemia Patient with Single Treatment of Lentiviral Gene Therapy

Cambridge, Mass., September 15, 2010 – bluebird bio (formerly Genetix Pharmaceuticals Inc.) an emerging leader in the development of innovative gene therapies for severe genetic disorders, today announced publication in the journal *Nature* of its promising Phase 1/2 data highlighting positive results of LentiGlobin™ gene therapy treatment in a young adult with severe beta-thalassemia, a blood disorder that is one of the most frequent inherited diseases.

The patient, who had been transfusion dependent since early childhood, has become transfusion independent for the past 21 months – more than two years after treatment with the LentiGlobin vector. The study also identified a subset of cells with the corrected beta-globin gene that overexpressed a truncated form of a gene called HMGA2. The patient has not experienced any adverse events. The data show that while early on, the HMGA2 clone was a significant portion of the corrected cells, the clone levels had declined at the time the paper was prepared, and further follow up indicates the decline is continuing.

"Although based on the first treated patient, we believe these results are impressive and illustrate for the first time the significant potential for treatment of beta hemoglobinopathies using lentiviral beta-globin gene transfer in the context of autologous stem cell transplant," said Philippe Leboulch, M.D., senior author of the study and head of the Institute of Emerging Diseases and Innovative Therapies of CEA and INSERM; professor of medicine, University of Paris; and visiting professor, Harvard Medical School. "For beta-thalassemia, we have worked intensely for almost 20 years to design, develop and manufacture LentiGlobin to provide a sustained high level hemoglobin production, resulting in a major clinical benefit. It has been very rewarding to follow this patient as his life has dramatically improved since receiving our treatment."

"For the first time, a patient with severe beta-thalassemia is living without the need for transfusions over a sustained period of time," said Marina Cavazzana-Calvo, M.D., first co-author of the study and professor of hematology, University of Paris and chief of Cell and Gene Therapy Department, Necker-Enfants Malades Hospital in Paris. Salima Hacein-Bey-Abina, Ph.D., professor of immunology, University of Paris, added, "These results are not only

important due to the tremendous medical need that exists for thalassemia patients around the world, but also represents a significant step forward for the field of autologous stem cell therapy as an emerging therapeutic modality."

Dr. Françoise Bernaudin, the clinical hematologist who has followed this patient since early childhood, said, "It is wonderful to see that this young man is for now free of transfusions and injections for iron chelation. He is happy to have a normal life back, and for the first time has a full-time job as a cook in a main restaurant in Paris. We are now even able to bleed him regularly to help remove toxic iron that had accumulated over the years because of blood transfusions."

The paper, titled "Transfusion independence and HMGA2 activation after gene therapy of human beta-thalassemia," is available in the online publication of *Nature* at www.nature.com.

"We believe the human findings in beta-thalassemia, as well as the recently published data in *Science* on two patients with childhood cerebral adrenoleukodystrophy (CCALD), highlight the significant opportunity for bluebird bio's gene therapy platform to help patients with severe genetic disorders," said Nick Leschly, president and CEO of bluebird bio. "We are committed to building a world-class company in gene therapy led by outstanding people as we move aggressively forward with multiple clinical studies, including our ongoing clinical trials for the development of LentiGlobin for beta-thalassemia and our product for CCALD."

About Beta-Thalassemia

Thalassemias are inherited blood disorders that cause the body to have an inadequate amount of hemoglobin, the protein in red blood cells that carries oxygen. Beta-thalassemia is named for defects in production of the beta-globin chain of hemoglobin and mostly affects people of Mediterranean, Middle Eastern, South Asian, Southeast Asian and Chinese descent. Approximately 60,000 children are diagnosed with the disease each year throughout the world. The severe form of beta-thalassemia, also known as thalassemia major or Cooley's anemia, occurs when both beta-globin genes (one from each parent) are mutated. In beta E /beta 0-thalassemia, as in the study's treated patient, one of the mutated genes is completely silent while the other expresses low levels of a mutated protein (beta E). This form is especially common in Southeast Asian countries and their diasporas. Immigration patterns have made this form the most frequent in the West coast of the United States. Patients typically require monthly supportive red blood cell transfusions to treat their severe anemia for life. Unfortunately, over time these transfusions lead to severe iron overload that causes serious damage to many organs. Patients must regularly take chelating drugs to remove the excess iron to stay alive. The other option – allogeneic stem cell transplantation – carries a significant risk of morbidity and mortality.

About LentiGlobin®

bluebird bio's LentiGlobin introduces a fully functional human beta-globin gene, under the control of the beta-globin enhancer and locus control region, into the patient's own hematopoietic stem cells in the bone marrow. bluebird bio is conducting a Phase 1/2 trial examining the feasibility, safety and efficacy of LentiGlobin in the treatment of beta-thalassemia and sickle cell anemia. By adding a single transduction step to the routine practice of autologous bone-marrow

transplantation, LentiGlobin may extend the benefit of bone-marrow transplantation to the 75 percent of patients lacking matched sibling donors. Additionally, not only would autologous bone marrow transplantation avoid the mortality and morbidity associated with graft-versus-host disease and immune suppression, but a one-time gene transfer could replace lifetime monthly transfusions and daily overnight iron chelation therapy associated with thalassemia.

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

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