bluebird bio Awarded up to $4.2 Million from the French Muscular Dystrophy Association for Continued Development of Gene Therapy for Beta-Thalassemia and Sickle Cell Anemia

March 16, 2011 3:24 PM ET

FOR IMMEDIATE RELEASE

Media Contact:
Sheryl Seapy
Pure Communications Inc.
(949) 608-0841

bluebird bio Awarded up to $4.2 Million from the French Muscular Dystrophy Association for Continued Development of Gene Therapy for Beta-Thalassemia and Sickle Cell Anemia

CAMBRIDGE, Mass., March 16, 2011 – bluebird bio, an emerging leader in the development of innovative gene therapies for severe genetic disorders, today announced that it has entered into an agreement with the French Muscular Dystrophy Association (AFM), a French non-profit entity, whereby the company will receive an initial amount of approximately $1.4 million in cash to support the development of LentiGlobin®, the company's development-stage program for the treatment of beta-thalassemia and sickle cell anemia. As part of the research agreement, bluebird bio has the option to draw upon an additional amount of up to $2.8 million in credit toward the manufacturing of cGMP clinical trial material at Généthon. In December 2010, bluebird bio entered into an agreement with Généthon designed to enable substantial advances in the existing manufacturing process of lentiviral vectors for the benefit of both partners.

bluebird bio's LentiGlobin introduces a fully functional human beta-globin gene, under the control of the beta-globin promoter and locus control regions, into the patient's own hematopoietic stem cells. bluebird bio is currently conducting a Phase 1/2 trial examining the feasibility, safety and efficacy of LentiGlobin in the treatment of beta-thalassemia and sickle cell anemia. Based on clinical data published in the September 2010 issue of *Nature*, LentiGlobin therapy has shown the potential to eliminate the need for monthly blood transfusions in patients with beta-thalassemia, without the risk of graft-versus-host disease.

"This funding from the AFM will not only support our ongoing thalassemia and sickle cell clinical trial, but also signifies the beginning of an important collaboration with the AFM," said Nick Leschly, president and chief executive officer of bluebird bio. "We are grateful for the AFM's commitment to the advancement of a treatment that has the potential to greatly improve the lives of patients."

Beta-thalassemia is an inherited blood disorder that is named for defects in production of the beta-globin chain of hemoglobin, the protein in red blood cells that carries oxygen. Approximately 60,000 children are diagnosed with the disease each year throughout the world. Patients typically require monthly supportive red blood cell transfusions to treat their severe anemia for life. Sickle cell anemia is characterized by clotting of improperly shaped red blood cells, which leads to a wide variety of serious health problems including chronic pain and high risk for stroke. Sickle cell anemia affects millions throughout the world, including approximately 95,000 people in the United States.

About the AFM
The French Muscular Dystrophy Association (Association Française contre les Myopathies, AFM) was founded for the purpose of serving patients with genetic neuromuscular diseases (diseases that cause muscle wasting) 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 for rare diseases both in France and worldwide. It is currently funding 36 clinical trials for 30 different genetic diseases, including diseases of the eye, blood, brain, immune system and muscle. Thanks to its Généthon research lab, the AFM stands out through its unique ability to produce and test its own gene-based therapeutics.

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.

###