



NEWS RELEASE

bluebird bio Announces First Patient Transplanted in Phase 1/2 HGB-205 Study for the Treatment of Beta-Thalassemia and Sickle Cell Anemia

CAMBRIDGE, MA, December 2, 2013 – bluebird bio, Inc. (Nasdaq: BLUE) a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic and orphan diseases, today announced that the first subject with beta-thalassemia major has been enrolled in its phase 1/2 HGB-205 study in France and has undergone infusion with bluebird bio's LentiGlobin drug product in an autologous hematopoietic stem cell transplantation.

"We believe gene therapy represents a potentially new and exciting treatment option for patients with severe forms of beta-thalassemia and sickle cell disease," stated Marina Cavazzana, MD, Professor of Medicine at Paris Descartes University and Research Director at the Centre for Clinical Research in Biotherapy, Necker Hospital, Paris France. "The beta-hemoglobinopathies are the most prevalent inherited disorders worldwide, and they result in substantial morbidity and mortality. The HGB-205 study builds on early and encouraging clinical data from the LG001 study based on the pioneering work of Professor Philippe Leboulch and colleagues at the Universities of Paris and Harvard, Inserm and the French Atomic Energy and Alternative Energies Commission (CEA) research center in Paris."

"It is very gratifying for our research, manufacturing and development teams to see their efforts to improve the potency and scalability of our product platform finally reach the clinic for patients with this life threatening disease. This milestone brings us closer towards realizing our vision of making hope a reality for patients with limited therapeutic options," stated Dave Davidson, MD, bluebird bio's Chief Medical Officer. "

About the HGB-205 Study

The phase 1/2 study is designed to evaluate the safety and efficacy of LentiGlobin drug product in the treatment of subjects with beta-thalassemia major and severe sickle cell disease. The study is designed to enroll up to seven subjects. Subjects will be followed to evaluate safety and transfusion requirements post-transplant. In sickle cell disease patients only, efficacy will also be measured based on the number of vaso-occlusive crises or acute chest syndrome events.

About beta-thalassemia major and sickle cell disease

Beta-thalassemia major is a rare hereditary blood disorder caused by a genetic abnormality of the beta globin gene resulting in defective red blood cells. Symptoms of beta-thalassemia include severe anemia, splenomegaly and iron overload in major organs. It is estimated that about 288,000 patients with beta-thalassemia major are alive, of which an estimated 15,000 live in the United States and Europe.

Sickle cell disease (SCD) is also a hereditary blood disorder resulting from a mutation in the beta globin gene that causes polymerization of hemoglobin proteins and abnormal red blood cell function. The symptoms of SCD include anemia, vaso-occlusive crisis and strokes. The global incidence of SCD is estimated to be 250,000 to 300,000 births annually, and the global prevalence of the disease is estimated to be about 20 to 25 million.

About bluebird bio, Inc.

bluebird bio is a clinical-stage company committed to developing potentially transformative gene therapies for severe genetic and orphan diseases. bluebird bio has two clinical-stage programs in development. The most advanced product candidate, Lenti-D, is in a recently-initiated phase 2/3 study for the treatment of childhood cerebral adrenoleukodystrophy (CCALD), a rare, hereditary neurological disorder affecting young boys. The next most advanced product candidate, LentiGlobin, is currently in a phase 1/2 study in France for the treatment of beta-thalassemia major and severe sickle cell disease. A second phase 1/2 study with LentiGlobin in the United States has been initiated for the treatment of beta-thalassemia major.

bluebird bio also has an early-stage chimeric antigen receptor-modified T cell (CAR-T) program for oncology in partnership with Celgene Corporation.

bluebird bio has operations in Cambridge, Massachusetts and Paris, France. For more information, please visit www.bluebirdbio.com

Forward-Looking Statements

This release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the advancement of the Company's clinical studies and the potential safety and clinical benefits of the Company's product candidates. Any forward-looking statements in this press release are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to, the risk of cessation or delay of any of the ongoing or planned clinical studies and/or our development of our product candidates, the risk of a delay in the enrollment of patients in the Company's clinical studies, the risk that the results of previously conducted studies involving similar product candidates will not be repeated or observed in ongoing or future studies involving current product candidates, the risk that our collaboration with Celgene will not continue or will not be successful, and the risk that any one or more of our product candidates will not be successfully developed and commercialized. For a discussion of other risks and uncertainties, and other important factors, any of which could cause our actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in our most recent quarterly report on Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. All

information in this press release is as of the date of the release, and bluebird bio undertakes no duty to update this information unless required by law.

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