bluebird bio's LentiGlobin™ Gene Therapy Granted Accelerated Assessment by European Medicines Agency for the Treatment of Transfusion-Dependent β-Thalassemia

– Company on Track to Submit Marketing Authorization Application (MAA) in European Union in 2018 –

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--bluebird bio, Inc. (Nasdaq: BLUE) today announced that its investigational LentiGlobin™ gene therapy for the treatment of adolescent and adult patients with transfusion-dependent β-thalassemia (TDT) and a non-β^0/β^0 genotype, was granted an accelerated assessment by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA), for its upcoming marketing authorization application (MAA). LentiGlobin is a potential one-time gene therapy that may address the underlying genetic cause of TDT.

"Transfusion-dependent β-thalassemia is a severe genetic disease that requires a lifetime of chronic blood transfusions for survival, and while these transfusions are life-saving, they are also associated with serious medical complications such as organ failure from iron overload," said David Davidson, M.D., chief medical officer, bluebird bio. "Receiving accelerated assessment for LentiGlobin helps support our goal of delivering the first gene therapy to patients with TDT. We look forward to working in collaboration with the regulatory authorities on this potentially transformative treatment option."

bluebird bio intends to file an MAA for LentiGlobin in TDT with the EMA in 2018. Accelerated assessments can reduce the active review time of an MAA from 210 days to 150 days once it has been validated by the EMA. An accelerated assessment is granted to products deemed by the CHMP to be of major interest for public health and represent therapeutic innovation.

The accelerated assessment for LentiGlobin is supported by data from clinical studies, including the completed Phase 1/2 Northstar (HGB-204) study, the ongoing Phase 1/2 HGB-205 study as well as available data from the Phase 3 Northstar-2 (HGB-207) study and the long-term follow-up study LTF-303.

The EMA previously granted Priority Medicines (PRIME) eligibility and Orphan Medicinal Product designation to LentiGlobin for the treatment of TDT. LentiGlobin is also part of the EMA's Adaptive Pathways pilot program, which is part of the EMA's effort to improve timely access for patients to new medicines.

The U.S. Food and Drug Administration (FDA) also granted LentiGlobin Orphan Drug status and Breakthrough Therapy designation for the treatment of TDT.

About Transfusion-Dependent β-Thalassemia

Transfusion-dependent β-thalassemia (TDT) is an inherited blood disorder caused by a mutation in the β-globin gene, which causes ineffective red blood cell production leading to severe anemia. TDT is the most severe clinical presentation of β-thalassemia and includes patients who receive blood transfusions as frequently as every two to four weeks.

Supportive care for people with TDT consists of a lifelong regimen of chronic blood transfusions to enable survival and suppress symptoms of the disease, and iron chelation therapy to manage iron overload that results from the transfusions. Despite the availability of supportive care, many people with TDT experience serious complications and organ damage due to underlying disease and iron overload.

By eliminating or reducing the need for blood transfusions, the long-term complications associated with TDT may be reduced.

Allogeneic hematopoietic stem cell transplantation (HSCT) is currently the only available option with the potential to correct the genetic deficiency in TDT. Complications of allogeneic HSCT include a risk of treatment-related mortality, graft failure, graft-versus-host disease (GvHD) and opportunistic infections, particularly in patients who undergo non-sibling matched allogeneic HSCT.

Clinical Development Program for LentiGlobin

bluebird bio's clinical development program for LentiGlobin includes ongoing studies around the world with sites in Australia, Germany, Greece, France, Italy, Thailand, the United Kingdom and the United States. For more information visit: www.northstarclinicalstudies.com or clinicaltrials.gov using identifier NCT01745120.

In addition, bluebird is conducting a long-term safety and efficacy follow-up study (LTF-303) for people who have participated in bluebird bio-sponsored clinical studies of LentiGlobin for treatment-dependent β-thalassemia and sickle cell disease.

About bluebird bio, Inc.

With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built a pipeline with broad potential application in severe genetic diseases and cancer.

bluebird bio's gene therapy clinical programs include investigational treatments for cerebral adrenoleukodystrophy, transfusion-dependent β-thalassemia, also known as β-thalassemia major, and severe sickle cell disease.

bluebird bio's oncology pipeline is built upon the company's lentiviral gene delivery and T cell engineering, with a focus on developing novel T cell-based immunotherapies, including chimeric antigen receptor (CAR T) and T cell receptor (TCR) therapies. The company's lead oncology programs are anti-BCMA CAR T programs partnered with Celgene.

bluebird bio's discovery research programs include utilizing megaTAL/homing endonuclease gene editing technologies with the potential for use across the company's pipeline.

bluebird bio has operations in Cambridge, Massachusetts; Seattle, Washington; Durham, North Carolina and Zug, Switzerland.

LentiGlobin is a trademark of bluebird bio, Inc.

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 Company's development and regulatory approval plans for its LentiGlobin product candidate to treat transfusion-dependent ß-thalassemia. Any forward-looking statements 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 risks that the preliminary positive efficacy and safety results from our prior and ongoing clinical trials of LentiGlobin will not continue or be repeated in our ongoing or planned clinical trials of LentiGlobin, the risks that the changes we have made in the LentiGlobin manufacturing will not result in improved patient outcomes, risks that the current or planned clinical trials of LentiGlobin will be insufficient to support regulatory submissions or marketing approval in the US and EU, and the risk that any one or more of our product candidates, will not be successfully developed, approved or 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 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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