press release

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bluebird bio Announces Launch in Germany of ZYNTEGLO™ (autologous CD34+ cells encoding βA-T87Q-globin gene) Gene Therapy for Patients 12 Years and Older with Transfusion-Dependent β-Thalassemia Who Do Not Have $\beta^0/\beta 0$ Genotype

First agreements with statutory health insurances utilize bluebird's innovative value-based payment model and provide coverage for ZYNTEGLO for up to 50% of patients in Germany

First qualified treatment center established at University Hospital of Heidelberg to provide ZYNTEGLO to patients

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 13, 2020-- **bluebird bio, Inc.** (Nasdaq: BLUE) announced the launch in Germany of ZYNTEGLO™ (autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene), a one-time gene therapy for patients 12 years and older with transfusion-dependent β-thalassemia (TDT) who do not have a β^0/β^0 genotype, for whom hematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available. This is the first time ZYNTEGLO is commercially available.

TDT is a severe genetic disease caused by mutations in the β-globin gene that result in significantly reduced or absent adult hemoglobin (HbA). In order to survive, people with TDT maintain hemoglobin (Hb) levels through lifelong chronic blood transfusions. These transfusions carry the risk of progressive multi-organ damage due to unavoidable iron overload. ZYNTEGLO is a one-time gene therapy that addresses the underlying genetic cause of TDT and offers patients the potential to become transfusion independent, which, once achieved, is expected to be lifelong.

Due to the highly technical and specialized nature of administering gene therapy in rare diseases, bluebird bio is working with institutions that have expertise in stem cell transplant as well as in treating patients with TDT to create qualified treatment centers that will administer ZYNTEGLO. bluebird bio has established a collaboration with University Hospital of Heidelberg as the first qualified treatment center in Germany.

In addition, bluebird has entered into value-based payment agreements with multiple statutory health insurances in Germany to help ensure patients and their healthcare providers have access to ZYNTEGLO and that payers only pay if the therapy delivers on its promise. bluebird's proposed innovative model is limited to five payments made in equal installments. An initial payment is made at the time of infusion. The four additional annual payments are only made if no transfusions for TDT are required for the patient.

"For patients with TDT, lifelong chronic blood transfusions are required in order to survive. We are thrilled to announce that ZYNTEGLO will now be available for patients in the EU living with this severe disease," says Alison Finger, chief commercial officer, bluebird bio. "In addition to confirming manufacturing readiness of our partner, apceth Biopharma GmbH, bluebird has also submitted a dossier to the Joint Federal Committee (G-BA) in Germany for drug benefit assessment. We would like to thank our collaborators for their commitment in helping us transform the healthcare system by accepting innovative payment models, and we look forward to treating our first commercial patient soon."

About LentiGlobin for β-Thalassemia (autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene)

The European Commission granted conditional marketing authorization for LentiGlobin for β-thalassemia, to be marketed as ZYNTEGLO™ (autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene) gene therapy, for patients 12 years and older with TDT who do not have a β^0/β^0 genotype, for whom hematopoietic stem cell (HSC) transplantation is appropriate, but a human leukocyte antigen (HLA)-matched related HSC donor is not available.

TDT is a severe genetic disease caused by mutations in the β-globin gene that result in reduced or significantly reduced hemoglobin (Hb). In order to survive, people with TDT maintain Hb levels through lifelong chronic blood transfusions. These transfusions carry the risk of progressive multi-organ damage due to unavoidable iron overload.

LentiGlobin for β-thalassemia adds functional copies of a modified form of the β-globin gene ($\beta^{A\text{-}T87Q}$-globin gene) into a patient's own hematopoietic (blood) stem cells (HSCs). Once a patient has the $\beta^{A\text{-}T87Q}$-globin gene, they have the potential to produce HbA^{T87Q}, which is gene therapy-derived hemoglobin, at levels that may eliminate or significantly reduce the need for transfusions.

Non-serious adverse events (AEs) observed during the HGB-204, HGB-207 and HGB-212 clinical studies that were attributed to LentiGlobin for β-thalassemia were hot flush, dyspnoea, abdominal pain, pain in extremities, thrombocytopenia, leukopenia, neutropenia and non-cardiac chest pain. One serious adverse event (SAE) of thrombocytopenia was considered possibly related to LentiGlobin for β-thalassemia for TDT.

Additional AEs observed in clinical studies were consistent with the known side effects of HSC collection and bone marrow ablation with busulfan, including SAEs of veno-occlusive disease.

The conditional marketing authorization for ZYNTEGLO is valid in the 28 member states of the EU as well as Iceland, Liechtenstein and Norway. For details, please see the **Summary of Product Characteristics (SmPC)**.

The U.S. Food and Drug Administration (FDA) granted LentiGlobin for β-thalassemia Orphan Drug status and Breakthrough Therapy designation for the treatment of TDT. LentiGlobin for β-thalassemia is not approved in the United States.

bluebird bio has initiated the rolling BLA submission for approval in the U.S., and is engaged with the FDA in discussions regarding the requirements and timing of the various components of the rolling BLA submission. Subject to these ongoing discussions, the company is currently planning to complete the BLA submission in the first half of 2020.

LentiGlobin for β-thalassemia continues to be evaluated in the ongoing Phase 3 Northstar-2 and Northstar-3 studies. For more information about the ongoing clinical studies, visit **www.northstarclinicalstudies.com** or **clinicaltrials.gov** and use identifier NCT02906202 for Northstar-2 (HGB-207) or NCT03207009 for Northstar-3 (HGB-212).

bluebird bio 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 β-thalassemia. For more information visit: **https://www.bluebirdbio.com/our-science/clinical-trials** or **clinicaltrials.gov** and use identifier NCT02633943 for LTF-303.

About bluebird bio, Inc.

bluebird bio is pioneering gene therapy with purpose. From our Cambridge, Mass., headquarters, we're developing gene therapies for severe genetic diseases and cancer, with the goal that people facing potentially fatal conditions with limited treatment options can live their lives fully. Beyond our labs, we're working to positively disrupt the healthcare system to create access, transparency and education so that gene therapy can become available to all those who can benefit.

bluebird bio is a human company powered by human stories. We're putting our care and expertise to work across a spectrum of disorders including cerebral adrenoleukodystrophy, sickle cell disease, β-thalassemia and multiple myeloma, using three gene therapy technologies: gene addition, cell therapy and (megaTAL-enabled) gene editing.

bluebird bio has additional nests in Seattle, Wash.; Durham, N.C.; and Zug, Switzerland. For more information, visit **bluebirdbio.com**.

Follow bluebird bio on social media: **@bluebirdbio**, **LinkedIn**, **Instagram** and **YouTube**.

ZYNTEGLO, LentiGlobin, and bluebird bio are trademarks of bluebird bio, Inc.

The full common name for ZYNTEGLO: A genetically modified autologous CD34+ cell enriched population that contains hematopoietic stem cells transduced with lentiviral vector encoding the $\beta^{A\text{-}T87Q}$-globin gene.

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 plans and expectations for the commercialization for ZYNTEGLO™ (autologous CD34+ cells encoding $\beta^{A\text{-}T87Q}$-globin gene, formerly LentiGlobin™ in TDT) to treat TDT, and the potential implications of clinical data for patients. 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 risk that the efficacy and safety results from our prior and ongoing clinical trials of ZYNTEGLO will not continue or be repeated in our ongoing or planned clinical trials of ZYNTEGLO; the risk that the current or planned clinical trials of ZYNTEGLO will be insufficient to support regulatory submissions or marketing approval in the US, or for additional patient populations in the EU; the risk that the production of HbA^{T87Q} may not be sustained over extended periods of time; the risk that we may not secure adequate pricing or reimbursement to support continued development or commercialization of ZYNTEGLO; the risk that our collaborations with qualified treatment centers will not continue or be successful; and that the risk that commercial patients treated with ZYNTEGLO will not achieve or maintain transfusion independence. 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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