bluebird bio's LentiGlobin™ for Sickle Cell Disease Gene Therapy (bb1111) Granted Priority Medicines (PRIME) Designation by European Medicines Agency

EMA's PRIME programme designed to optimise development and expedite evaluation of innovative medicines for patients with high unmet need

ZUG, Switzerland— **23 September 2020** — **bluebird bio, Inc.** announced today that its investigational treatment for sickle cell disease (SCD), LentiGlobin™ for SCD gene therapy (bb1111), was granted eligibility to the Priority Medicines (PRIME) programme by the European Medicines Agency (EMA).

The EMA's PRIME initiative provides enhanced support and increased interaction to companies, with the goal of optimising development plans and speeding regulatory evaluations to potentially bring innovative medicines to patients more quickly. To be accepted for PRIME, a therapy must demonstrate potential to benefit patients with unmet medical need through early clinical data. Clinical data from the completed Phase 1/2 HGB-205 study, the ongoing Phase 1/2 HGB-206 and ongoing long-term safety and efficacy follow-up study LTF-303 supported the PRIME application for LentiGlobin for SCD.

"Even with recent progress to deliver new medicines for sickle cell disease, there remains unmet need for people living with SCD. SCD is a progressive disease that frequently leads to organ damage and early death, and whose hallmarks are haemolytic anaemia, painful vaso-occlusive crises (VOCs) and stroke," said Anne-Virginie Eggimann, M.Sc., senior vice president regulatory science of bluebird bio. "The PRIME designation shows the importance of expediting the development and review of treatment options for patients with SCD."

SCD is a serious, progressive and debilitating genetic disease caused by a mutation in the β-globin gene that leads to the production of abnormal sickle haemoglobin (HbS). HbS causes red blood cells (RBCs) to become sickled and fragile, which can result in chronic haemolytic anaemia, vasculopathy and unpredictable, painful VOCs[i] [ii] [iii] [iv]. For adults and children living with SCD, this can mean painful crises and other life altering or life-threatening acute complications—such as ACS, stroke and infections – and chronic end-organ complications, which can contribute to morbidity and early mortality in these patients[v] [vi] [vii] [viii].

LentiGlobin for SCD was designed to add functional copies of a modified form of the β-globin gene ($\beta^{A\text{-}T87Q}$-globin gene) into a patient's own haematopoietic (blood) stem cells. The expected outcome of this experimental treatment is that, once patients have the betaAT87Q globin gene, their red blood cells can produce anti-sickling haemoglobin, HbA^{T87Q}, which decreases the proportion of HbS, with the goal of reducing sickled red blood cells, haemolysis and other complications.

LentiGlobin for SCD received orphan medicinal product designation from the European Commission for the treatment of SCD.

The U.S. FDA granted orphan drug designation, fast track designation, regenerative medicine advanced therapy (RMAT) designation and rare paediatric disease designation for LentiGlobin for SCD.

LentiGlobin for SCD is investigational and has not been approved in any geography.

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-thalassaemia 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.; Zug, Switzerland Munich, Germany; Milan, Italy; Utrecht, the Netherlands; Hampshire, United Kingdom; and Paris, France.

For more information about bluebird bio in the UK, visit **bluebirdbio.co.uk**.

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