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31 July 2018 12:41

Gene therapy from bluebird bio gets accelerated assessment by CHMP

A gene therapy for the treatment of adolescent and adult patients with transfusion-dependent β-Thalassemia (TDT) is granted an accelerated assessment by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA).

With this accelerated assessment, LentiGlobin, which is a potential one-time gene therapy that may address the underlying genetic cause of TDT, is on track for bluebird bio's upcoming marketing authorisation application (MAA) — planned for submission this year.

"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 Dr David Davidson, 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."

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

A priority medicines (PRIME) eligibility and orphan medicinal product designation was previously granted to LentiGlobin by the EMA for the treatment of TDT. Additionally, the therapy forms part of the EMA's Adaptive Pathways pilot programme — an initiative by the agency to improve timely patient access to new medicines.

Furthermore, the US Food and Drug Administration (FDA) has granted orphan drug status and breakthrough therapy designation for the treatment of TDT.

accelerated assessment transfusion-dependent β-Thalassemia (TDT)
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