30 April 2020
EMA/CHMP/221210/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Reblozyl

luspatercept

On 30 April 2020, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Reblozyl[2], intended for the treatment of adults with transfusion-dependent anaemia associated with myelodysplastic syndromes (MDS) or beta-thalassaemia. The applicant for this medicinal product is Celgene Europe BV.

Reblozyl will be available as 25-mg and 75-mg powder for solution for injection. The active substance in Reblozyl is luspatercept, an erythroid maturation agent (ATC group B03X) that binds selected transforming growth factor-β superfamily ligands and thus inhibits Smad2/3 signalling, which is abnormally high in MDS and beta thalassaemia. Inhibition of Smad2/3 signalling results in differentiation of erythroid precursors (normoblasts) and maturation of red blood cells.

The benefits with Reblozyl are its ability to reduce the number of transfusions in patients with MDS or beta-thalassaemia. The most common side effects are bronchitis, urinary tract infection, upper respiratory tract infection, influenza, hypersensitivity, hyperuricaemia, dizziness, headache, syncope or presyncope, vertigo, hypertension, thromboembolic events, dyspnoea, diarrhoea, nausea, back pain, arthralgia, bone pain, fatigue, asthenia and injection site reactions.

The full indication is: "treatment of adult patients with transfusion-dependent anaemia due to very low, low and intermediate-risk myelodysplastic syndromes (MDS) with ring sideroblasts, who had an unsatisfactory response to or are ineligible for erythropoietin-based therapy and for the treatment of adult patients with transfusion-dependent anaemia associated with beta-thalassaemia."

Reblozyl should be prescribed by physicians experienced in treatment of haematological diseases.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion
[2] This product was designated an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained

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