15 December 2022
EMA/CHMP/916178/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Hemgenix

etranacogene dezaparvovec

On 15 December 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a conditional[2] marketing authorisation for the medicinal product Hemgenix[3], intended for the treatment of severe and moderately severe Haemophilia B. As Hemgenix is an advanced therapy medicinal product, the CHMP positive opinion is based on an assessment by the Committee for Advanced Therapies.

The applicant for this medicinal product is CSL Behring GmbH.

Hemgenix will be available as 1 x 10^{13} genome copies/ml concentrate for solution for infusion. The active substance of Hemgenix is etranacogene dezaparvovec (ATC code: B06). Etranacogene dezaparvovec is a recombinant adeno-associated virus serotype 5 (AAV5)-based gene therapy vector that expresses a version of the Padua variant of human factor IX under the control of a liver-specific promoter. The expressed factor IX replaces the missing coagulation factor IX required for proper coagulation of the patient's blood.

The benefits of Hemgenix are the induction of relevant plasma levels of factor IX and the reduction of bleeding episodes. In the clinical trial presented, most patients treated with Hemgenix had a significant increase in factor IX activity levels and experienced less bleedings than before the treatment on standard FIX prophylaxis. Most patients no longer needed Factor IX replacement therapy up to two years after the administration. The most common side effects with Hemgenix are headache, hepatic laboratory abnormalities (increased ALT and AST) and flu-like illness.

The full indication is:

> Hemgenix is indicated for the treatment of severe and moderately severe Haemophilia B (congenital Factor IX deficiency) in adult patients without a history of Factor IX inhibitors.

[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] A conditional marketing authorisation is granted to a medicinal product that fulfils an unmet medical need when the benefit to public health of immediate availability outweighs the risk inherent in the fact that additional data are still required. The marketing authorisation holder is expected to provide comprehensive clinical data at a later stage.
[3] This product was designated as 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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Treatment with Hemgenix should be initiated under the supervision of a physician experienced in the treatment of Haemophilia and/or bleeding disorders. This medicinal product should be administered in a setting where personnel and equipment are immediately available to treat infusion-related reactions.

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.