EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

30 May 2024
EMA/CHMP/232716/2024
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Durveqtix

fidanacogene elaparvovec

On 30 May 2024, 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 Durveqtix, intended for the treatment of severe and moderately severe haemophilia B.

As Durveqtix 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 Pfizer Europe MA EEIG.

Durveqtix will be available as 0.79-1.21 × 10^{13} vector genomes/mL concentrate for solution for infusion. The active substance of Durveqtix is fidanacogene elaparvovec, a blood coagulation factor gene therapy (ATC code: not yet assigned). Fidanacogene elaparvovec uses a recombinant adeno-associated viral serotype Rh74 (AAVRh74var) to deliver a functional copy of the Padua variant of human factor IX transgene. The expressed factor IX replaces the missing coagulation factor IX required for proper coagulation of the patient's blood.

The benefit of Durveqtix is a reduction in bleeding episodes. In the clinical trial presented, most patients treated with Durveqtix experienced fewer bleeding episodes than before treatment, when they were taking standard factor IX prophylaxis. Following the administration of Durveqtix, most patients no longer needed factor IX replacement therapy, and this benefit was maintained for at least two years. The most common side effects with Durveqtix are hepatic laboratory abnormalities (elevated ALT and AST).

The full indication is:

> DURVEQTIX 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 and without detectable antibodies to variant AAV serotype Rh74.

[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.

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Treatment with Durveqtix should be administered in a qualified treatment centre by a physician experienced in the treatment of haemophilia. This medicinal product should be administered in a setting where personnel and equipment are available to treat possible 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.