23 June 2022
EMA/CHMP/584501/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Roctavian

valoctocogene roxaparvovec

On 23 June 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 Roctavian[3], intended for the treatment of severe haemophilia A.

As Roctavian 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 BioMarin International Limited.

Roctavian will be available as a 2 x 10^{13} vg/mL solution for infusion. The active substance of Roctavian is valoctocogene roxaparvovec, an antihemorrhagic agent (ATC code: B02). Valoctocogene roxaparvovec is an adeno-associated virus serotype 5 (AAV5)-based gene therapy vector that expresses a recombinant version of human factor VIII under the control of a liver-specific promoter. The recombinant FVIII replaces the missing coagulation factor VIII needed to restore the coagulatory ability of the patient's blood.

The benefit of Roctavian is the induction of relevant plasma levels of FVIII. In the clinical trial presented, the therapy significantly increased factor VIII activity levels in the majority of patients and most patients no longer needed factor VIII replacement therapy two years after the administration.

The most common side effects are hepatic laboratory abnormalities (increased ALT and AST), nausea and headache.

The full indication is:

> ROCTAVIAN is indicated for the treatment of severe haemophilia A (congenital factor VIII deficiency) in adult patients without a history of factor VIII inhibitors and without detectable

[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

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

antibodies to adeno associated virus serotype 5 (AAV5).

Roctavian should be prescribed by physicians 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.