16 December 2021
EMA/CHMP/622322/2021
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Oxbryta

voxelotor

On 16 December 2021, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Oxbryta[2], intended for the treatment of haemolytic anaemia due to sickle cell disease. The applicant for this medicinal product is Global Blood Therapeutics Netherlands B.V.

Oxbryta will be available as 500 mg film-coated tablets. The active substance of Oxbryta is voxelotor (ATC code: B06AX03), a small molecule which binds reversibly to haemoglobin, stabilizing the oxygenated haemoglobin state and preventing HbS polymerization by increasing haemoglobin's affinity for oxygen.

The benefits of Oxbryta are an increase in haemoglobin and a decrease in laboratory markers indicative of haemolysis. The most common side effects were headache, diarrhoea, and abdominal pain.

The full indication is:

> Oxbryta is indicated for the treatment of haemolytic anaemia due to sickle cell disease (SCD) in adults and paediatric patients 12 years of age and older as monotherapy or in combination with hydroxycarbamide.

Oxbryta should be prescribed by physicians experienced in the management of sickle cell disease.

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 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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