23 June 2022
EMA/CHMP/585580/2022
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Pepaxti

melphalan flufenamide

On 23 June 2022, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Pepaxti[2], intended for the treatment of multiple myeloma. The applicant for this medicinal product is Oncopeptides AB.

Pepaxti will be available as a 20 mg powder for concentrate for solution for infusion. The active substance of Pepaxti is melphalan flufenamide, an antineoplastic agent (ATC code: L01AA10). Melphalan flufenamide is a lipophilic derivative of melphalan designed to enhance cell penetration. Once inside the cells, melphalan inhibits DNA and RNA synthesis, causing tumour cell death.

The benefit of Pepaxti is its ability to bring about a response in patients with relapsed and refractory multiple myeloma. The most common side effects are thrombocytopenia, neutropenia and anaemia.

The full indication is:

> Pepaxti is indicated, in combination with dexamethasone, for the treatment of adult patients with multiple myeloma who have received at least three prior lines of therapies, whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one anti-CD38 monoclonal antibody, and who have demonstrated disease progression on or after the last therapy. For patients with a prior autologous stem cell transplantation, the time to progression should be at least 3 years from transplantation.

Pepaxti should be prescribed by physicians experienced in the treatment of multiple myeloma.

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

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