30 March 2023
EMA/CHMP/130783/2023
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Pedmarqsi

sodium thiosulfate

On 30 March 2023, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Pedmarqsi, intended for the prevention of ototoxicity induced by cisplatin chemotherapy in children with solid tumours.

The applicant for this medicinal product is Fennec Pharmaceuticals (EU) Limited.

Pedmarqsi will be available as an 80 mg/ml solution for infusion. The active substance of Pedmarqsi is sodium thiosulfate (ATC code not yet assigned), an antidote with a mechanism of action that is not fully understood but is thought to include increasing levels of endogenous antioxidants, inhibition of intracellular oxidative stress and direct interaction with cisplatin to prevent cisplatin-induced damage to cochlear cells and associated hearing loss.

The benefit of Pedmarqsi is preventing cisplatin-induced hearing loss, as evaluated in a phase 3, randomised, open-label study in children with standard-risk hepatoblastoma and treated with cisplatin alone or in combination with Pedmarqsi. The most common side effects are vomiting, nausea, hypernatraemia, hypophosphataemia and hypokalaemia.

The full indication is:

> Pedmarqsi is indicated for the prevention of ototoxicity induced by cisplatin chemotherapy in patients 1 month to < 18 years of age with localised, non-metastatic, solid tumours.

Pedmarqsi is intended for hospital use only and must be used under the supervision of an appropriately qualified physician.

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

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