12 November 2020
EMA/CHMP/597095/2020
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Elzonris

tagraxofusp

On 12 November 2020, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion following a re-examination procedure, recommending the granting of a marketing authorisation for the medicinal product Elzonris[2], intended for the treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN).

The applicant for this medicinal product is Stemline Therapeutics B.V.

Elzonris will be available as a 1-mg/ml concentrate for solution for infusion. The active substance of Elzonris is tagraxofusp, an antineoplastic agent (ATC code: L01XX67). Tagraxofusp comprises a truncated diphtheria toxin (DT) fusion protein that is linked with recombinant human interleukin-3 (IL 3) to target CD123-expressing cells. Tagraxofusp irreversibly inhibits protein synthesis of target cells by inactivating elongation factor 2 (EF2), resulting in apoptosis (cell death).

The benefits of Elzonris are that it can lead to complete response, with a rate (CR/Crc)[3] of 53.8% as shown in a study involving 13 previously untreated patients. The most common side effects are hypoalbuminaemia, increased transaminases, thrombocytopenia, nausea, fatigue and pyrexia observed in >20% pf patients. The most serious adverse reaction that may occur during treatment with Elzonris is capillary leak syndrome which was reported in 17% of patients with a median time to onset of 6 days.

The full indication is:

ELZONRIS is indicated as monotherapy for the first-line treatment of adult patients with blastic plasmacytoid dendritic cell neoplasm (BPDCN) (see section 5.1). Elzonris should be administered under the supervision of a physician experienced in the use of anti-cancer agents.

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
[3] complete resolution of the disease (CR) or CR with residual skin abnormality not indicative of active disease (CRc)

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