EMA/24814/2021
EMEA/H/C/005031

Elzonris (*tagraxofusp*)

An overview of Elzonris and why it is authorised in the EU

What is Elzonris and what is it used for?

Elzonris is a medicine used to treat adults with blastic plasmacytoid dendritic cell neoplasm (BPDCN), a type of blood cancer which can affect many organs including the skin, the bone marrow (the spongy tissue inside the large bones) and the lymph nodes.

BPDCN is rare, and Elzonris was designated an 'orphan medicine' (a medicine used in rare diseases) on 11 November 2015. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3151567.

Elzonris contains the active substance tagraxofusp.

How is Elzonris used?

Elzonris is available for infusion (drip) into a vein and can only be obtained with a prescription. It should only be given under the supervision of a doctor experienced in the use of cancer medicines, in a setting where resuscitation equipment is available.

The recommended dose is 12 micrograms per kilogram body weight once a day, infused over 15 minutes. Elzonris is given on the first 5 days of a 21-day cycle; cycles are repeated unless the disease gets worse or patients have unacceptable side effects.

The first cycle is given in a hospital, and patients should be monitored for side effects for at least 24 hours after the last infusion. Subsequent cycles can be given in an outpatient setting equipped for intensive monitoring of patients being treated for blood cancer.

Around one hour before each infusion, patients should be given antihistamine medicines, a corticosteroid and paracetamol to lower the risk of allergy-like reactions.

For more information about using Elzonris, see the package leaflet or contact your doctor or pharmacist.

How does Elzonris work?

The active substance in Elzonris, tagraxofusp, is made up of diphtheria toxin (a substance which is poisonous to cells), linked to a protein called interleukin 3. The interleukin-3 portion of the medicine

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attaches to receptors (targets) which are found in high number on BPDCN cells. Once attached to cancer cells, the medicine is taken up by them, allowing the toxin to be released inside, which kills them. This is expected to prevent spread of the cancer and reduce symptoms of the disease.

What benefits of Elzonris have been shown in studies?

Elzonris was investigated in one study involving 84 adults with BPDCN (65 previously untreated, and 19 previously treated with other medicines).

The study showed that 57% (37 out of 65) of previously untreated patients had no signs of disease or had minimal skin damage after treatment with Elzonris. Additionally, about a third (21 out of 65) of these patients were able to have stem cell transplantation (a procedure where the patient's bone marrow is replaced by stem cells from a donor to form new bone marrow that produces healthy cells), which contributed to patients living longer.

What are the risks associated with Elzonris?

The most common side effects with Elzonris (which may affect more than 1 in 5 people) are hypoalbuminemia (low blood albumin levels), increased blood levels of transaminases (a sign of liver problems), thrombocytopenia (low blood platelet counts), nausea (feeling sick), tiredness and fever.

The most serious side effect that may occur during treatment with Elzonris is capillary leak syndrome (an unpredictable, life-threatening side effect due to small blood vessels becoming leaky), which may affect up to 1 in 5 people.

For the full list of side effects and restrictions of Elzonris, see the package leaflet.

Why is Elzonris authorised in the EU?

Although the study was small and Elzonris was not compared with other treatments, the European Medicines Agency considered that conducting larger studies for this rare disease is challenging. After considering additional advice from experts in the field, the Agency concluded that the benefits of Elzonris outweigh its risks in patients with BPCDN who had not yet received other treatments and that the medicine can be authorised for use in the EU. However, the data were not sufficient to draw the same conclusion for patients in whom previous treatments did not work. The safety of Elzonris was considered acceptable with specific measures in place to minimise the risk of the most serious side effects.

Elzonris has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Elzonris due to the rarity of the disease. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Elzonris?

Since Elzonris has been authorised under exceptional circumstances, the company that markets Elzonris will provide the results of a study based on a registry of patients with BPCDN, in order to further characterise the effectiveness and safety of Elzonris.

What measures are being taken to ensure the safe and effective use of Elzonris?

The company that markets Elzonris will provide educational materials for healthcare professional and an 'alert card' for patients with important information about capillary leak syndrome.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Elzonris have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Elzonris are continuously monitored. Side effects reported with Elzonris are carefully evaluated and any necessary action taken to protect patients.

Other information about Elzonris

Elzonris received a marketing authorisation under exceptional circumstances valid throughout the EU on 7 January 2021.

Further information on Elzonris can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/elzonris.

This overview was last updated in 01-2021.