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ELZONRIS® (active substance: tagraxofusp)

First authorisation in Switzerland: 3 February 2023

Medicine (concentrate for solution for infusion) for first-line treatment of blastic plasmacytoid dendritic cell neoplasm (BPDCN) in adults.

About the medicine

The medicine ELZONRIS with the active substance tagraxofusp is used for first-line treatment of adults with blastic plasmacytoid dendritic cell neoplasm (BPDCN).

This disease is a very rare and aggressive type of blood cancer that frequently appears as skin changes. It develops as a result of increased formation of malignant plasmacytoid dendritic cells[1]. It can occur at any age but usually affects adults, and men more often than women.

There is no standard therapy for BPDCN, and no therapy specifically for BPDCN has been authorised in Switzerland. Intensive chemotherapy is frequently used. However, there is a high risk of relapse. The survival time can be increased substantially with a stem cell transplantation. ELZONRIS is an additional treatment option that helps to kill BPDCN cells.

Since BPDCN is a rare and life-threatening disease, the medicine has been authorised as an orphan drug. The term "orphan drug" is used to refer to important medicines for rare diseases.

Mode of action

Plasmacytoid dendritic cancer cells have certain characteristics on their surface, among them “CD123 antigens”. ELZONRIS is a protein that specifically targets cells with this CD123 trait. It acts by transporting toxins into the cancer cells, resulting in their death.

[1]Dendritic cells: Dendritic cells are highly specialised blood cells that play a role in the body’s immune response.

Indication

ELZONRIS, containing the active substance tagraxofusp, is a prescription-only medicine.

ELZONRIS is available in vials containing a 1 mg/ml concentrate to prepare a solution for infusion.

The recommended dosage is 12 µg tagraxofusp/kg bodyweight, administered once daily as an infusion into a vein. Treatments are given on days 1 to 5 of a 21-day treatment cycle. The treatment cycles are continued until the disease progresses or unacceptable side effects or another reason to discontinue treatment (e.g. transition to stem cell transplantation) occurs.

ELZONRIS is administered by a healthcare professional. Patients are monitored for signs and symptoms of hypersensitivity or capillary leak syndrome (CLS) [2] for at least 24 hours after the 1st cycle of ELZONRIS has been administered.

Efficacy

The efficacy of ELZONRIS was investigated in the 0114 study. The study subjects were adult patients with BPDCN.

The study was conducted in the USA. The patients were given ELZONRIS as an infusion over five days of a 21-day cycle until the disease progressed or another reason to discontinue the treatment occurred.

The effect was considered to be statistically significant if more than 10% of the patients had a complete response.

A total of 65 patients with BPDCN received first-line therapy with ELZONRIS, and 56.9% had a complete response. Furthermore, 32% of the patients underwent stem cell transplantation after treatment with ELZONRIS.

Precautions, undesirable effects & risks

ELZONRIS must not be used in those who are hypersensitive to the active substance or any of the excipients.

The most serious undesirable effect that can occur during treatment with ELZONRIS is CLS[2], which 18% of patients experienced.

Other undesirable effects that occurred in more than 30% of patients are high levels of liver enzymes (elevated transaminases), a reduced level of protein in the blood (hypoalbuminaemia), a low level of blood platelets (thrombocytopenia), nausea, fever (pyrexia), fatigue, and swelling due to fluid accumulation in the body (oedema).

All precautions, risks, and other possible undesirable effects are listed in the Information for healthcare professionals.

Why the medicinal product has been authorised

BPDCN is a very rare and aggressive disease with a poor prognosis for patients. To date, no therapy has been authorised in Switzerland specifically for BPDCN. Treatment usually involves intensive chemotherapy, followed by stem cell therapy if the patient re-

[2]Capillary leak syndrome (CLS): a life-threatening side effect caused by leaking capillaries (the smallest blood vessels).

sponds. The efficacy of ELZONRIS investigated in the study is considered to be sufficient and clinically relevant. The side effect profile of ELZONRIS varies, but safety is similar overall to that of the intensive chemotherapies currently in use.

Taking all the risks and precautions into account, and based on the available data, the benefits of ELZONRIS outweigh the risks. Swissmedic has therefore authorised the medicinal product ELZONRIS, containing the active substance tagraxofusp, for use in Switzerland.

Further information on the medicinal product

Information for healthcare professionals: Information for healthcare professionals ELZONRIS®

Healthcare professionals can answer any further questions.

The date of revision of this text corresponds to that of the SwissPAR. New information concerning the authorised medicinal product in question will not be incorporated into the Public Summary SwissPAR.

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