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Lumoxiti (*moxetumomab pasudotox*)

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

What is Lumoxiti and what is it used for?

Lumoxiti is a cancer medicine used to treat adults with hairy cell leukaemia, a cancer of the white blood cells where too many B cells (a type of white blood cell) are produced. The term 'hairy cell' refers to the hair-like projections that can be seen on the surface of the B cells when they are examined under a microscope.

Lumoxiti is used when the disease has not responded to or has come back (relapsed) after at least two other treatments, including treatment with a type of cancer medicine called purine nucleoside analogue (PNA).

Lumoxiti contains the active substance moxetumomab pasudotox.

Hairy cell leukaemia is rare, and Lumoxiti was designated an 'orphan medicine' (a medicine used in rare diseases) on 5 December 2008. Further information on the orphan designation can be found here.

How is Lumoxiti used?

Lumoxiti can only be obtained with a prescription. Treatment should be started and supervised by a doctor who has experience in cancer treatment.

The medicine is given by infusion (drip) into a vein. The recommended dose depends on the patient's weight. Infusions last 30 minutes and are given on days 1, 3 and 5 of a 4-week cycle. Treatment can continue for up to 6 cycles, unless the medicine no longer works or the patient has severe side effects. The doctor may also stop treatment if the patient no longer has signs of the disease.

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

How does Lumoxiti work?

The active substance in Lumoxiti, moxetumomab pasudotox, is made up of an antibody (a type of protein) attached to part of a toxin from the *Pseudomonas* bacteria. The antibody in this medicine has been designed to recognise and attach to an antigen (target) called CD22, which is found on the surface of B cells in hairy cell leukaemia. After attaching to CD22, moxetumomab pasudotox is taken

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up into the cells, where the toxin is released. The toxin then stops the cancerous cells from making new proteins, eventually killing them.

What benefits of Lumoxiti have been shown in studies?

A main study involving 80 patients showed that Lumoxiti was effective against hairy cell leukaemia that had not responded to or had relapsed after at least 2 other treatments, including treatment with PNA. The results showed that 36% (29 out of 80) of the patients had no signs of the cancer for at least 6 months after treatment ended.

In addition, blood cell counts returned to normal in 80% (64 out of 80) of patients and stayed within normal range for an average of 46 months.

What are the risks associated with Lumoxiti?

The most common side effects with Lumoxiti (which may affect more than 1 in 5 people) are oedema (swelling), nausea (feeling sick), infusion-related reactions, hypoalbuminaemia (low levels of albumin, a blood protein), and increased transaminases (a possible sign of liver problems).

The most serious side effects (which may affect up to 1 in 10 people) are haemolytic uraemic syndrome (a serious condition causing blood clots and kidney failure) and capillary leak syndrome (leakage of fluid from blood vessels causing tissue swelling and a drop in blood pressure).

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

Why is Lumoxiti authorised in the EU?

Lumoxiti has been shown to be effective at treating hairy cell leukaemia and keeping patients cancer-free for sustained periods. It also corrects abnormal blood cell counts and keeps them within the normal range for long periods. The effect on blood cell counts is of benefit to patients as low counts cause disease symptoms that require treatment. As for the medicine's safety, the side effects are consistent with those expected for an immunotoxin-based medicine and are manageable.

The European Medicines Agency therefore decided that Lumoxiti's benefits are greater than its risks and it can be authorised for use in the EU.

Lumoxiti has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about the medicine 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 Lumoxiti?

Since Lumoxiti has been authorised under exceptional circumstances, the company that markets the medicine will carry out a study based on data from a disease registry of patients with hairy cell leukaemia to provide further information on the safety and effectiveness of the medicine.

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

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

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

Other information about Lumoxiti

Lumoxiti received a marketing authorisation valid throughout the EU on 8 February 2021.

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

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