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Esperoct (*turoctocog alfa pegol*)

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

What is Esperoct and what is it used for?

Esperoct is a medicine used to treat and prevent bleeding in children, adolescents and adults with haemophilia A, an inherited bleeding disorder caused by the lack of factor VIII, a protein that helps the blood clot.

Esperoct contains the active substance turoctocog alfa pegol.

How is Esperoct used?

Esperoct can only be obtained with a prescription and treatment should be started by a doctor who has experience in the treatment of haemophilia.

Esperoct is given as an injection into a vein. The dose, frequency and duration of treatment depend on the age of the patient, whether it is used to treat or prevent bleeding, as well as on the severity of the haemophilia, the extent and location of the bleeding and the patient's condition and weight. Patients or their carers may be able to inject Esperoct themselves at home once they have been trained appropriately.

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

How does Esperoct work?

Patients with haemophilia A lack factor VIII, a protein needed for normal clotting of the blood, and as a result they bleed readily. The active substance in Esperoct, turoctocog alfa pegol, works in the body in the same way as human factor VIII. It replaces the missing factor VIII, thereby helping the blood to clot and giving temporary control of the bleeding disorder.

Part of the active substance is 'pegylated', whereby a chemical called polyethylene glycol (PEG), which is added to help the medicine remain in the body for longer and thereby prolong its action.

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What benefits of Esperoct have been shown in studies?

Studies have shown Esperoct to be effective at preventing and treating bleeding episodes in patients with severe haemophilia.

In a main study involving patients between 12 and 66 years of age, 175 patients given Esperoct every 4 days or twice weekly as preventive treatment had, on average, around 4 bleeding episodes a year, achieving the target of the study, which was less than 8.5 episodes a year. When bleeding episodes occurred, they were successfully treated with one or two further injections in 94% of cases.

Twelve further patients in this study did not receive preventive treatment but were given Esperoct for treating bleeding 'on demand'. These patients had on average around 32 bleeding episodes a year and Esperoct was successful at stopping the bleeds 97% of the time after 1 or 2 injections.

In a second study in 68 children aged below 12 years who were previously given other factor VIII medicines, Esperoct given as preventive treatment led to around 2 bleeding episodes a year during the main part of the study lasting 26 weeks. In a long-term extension of the second study, children experienced an estimated average of less than 1 bleeding episode per year; all bleeds were successfully treated with additional doses of Esperoct.

In a third main study, involving 81 children under 6 years of age who had not previously received factor VIII medicines, children experienced an estimated average of 1.8 bleeding episodes per year; 93% of bleeds were successfully treated with Esperoct.

What are the risks associated with Esperoct?

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

Hypersensitivity (allergic) reactions can occur with Esperoct; these are uncommon (affecting up to 1 in 100 people) and in some cases can become severe. These include swelling, burning and stinging at the injection site, chills, flushing, itchy rash, headache, hives, low blood pressure, lethargy, nausea and vomiting, restlessness, a rapid heartbeat, tightness of the chest, tingling and wheezing.

Very rarely, patients may develop antibodies against hamster protein in the medicine and have allergic reactions. Esperoct must not be used in patients with allergy to hamster proteins.

Following treatment with factor VIII products, including Esperoct, some patients may develop inhibitors (antibodies) against factor VIII, causing the medicine to stop working and resulting in a loss of bleeding control. In such cases, a specialised haemophilia centre should be contacted.

In clinical trials, about a third of previously-untreated patients starting Esperoct, who did not develop inhibitors against factor VIII, developed antibodies against the PEG part of the active substance. This led to decreased factor VIII activity, resulting in a loss of bleeding control in some of these patients. The factor VIII activity levels returned to normal in all patients who continued to receive treatment with Esperoct.

Why is Esperoct authorised in the EU?

Studies have found that Esperoct is effective at preventing and treating bleeding in adults and children with haemophilia A. Because Esperoct is 'pegylated', patients on this medicine could also have fewer or less frequent injections than they would on conventional FVIII products.

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

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

The company that markets Esperoct will carry out a study to investigate the potential effects of PEG accumulation in the choroid plexus of the brain and other tissues and organs.

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

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

Other information about Esperoct

Esperoct received a marketing authorisation valid throughout the EU on 20 June 2019.

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

This overview was last updated in 10-2024.