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Altuvoct (*efanesoctocog alfa*)

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

What is Altuvoct and what is it used for?

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

Haemophilia A is rare, and Altuvoct was designated an 'orphan medicine' (a medicine used in rare diseases) on 28 June 2019. Further information on the orphan designation can be found on the EMA website.

Altuvoct contains the active substance efanesoctocog alfa.

How is Altuvoct used?

Altuvoct can only be obtained with a prescription and treatment should be started and supervised by a doctor experienced in treating haemophilia.

Altuvoct is available as a powder and a liquid to be made into a solution and given by injection into a vein, over 1 to 10 minutes. The dose depends on the patient's body weight and whether the medicine is used to prevent or treat bleeding.

To prevent bleeding (prophylaxis), Altuvoct is given once a week. To treat active bleeding (on-demand treatment), the patient is first given a single injection of Altuvoct, followed by additional injections every 2 to 3 days if necessary. The duration of the on-demand treatment depends on the severity of the disease, the location and extent of the bleeding and the patient's health condition.

Patients or their carers may be able to administer Altuvoct themselves at home once they received appropriate training.

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

How does Altuvoct work?

Patients with haemophilia A lack factor VIII, a protein in the body that helps the blood clot. The active substance in Altuvoct, efanesoctocog alfa, resembles and replaces the missing factor VIII, thereby

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helping the blood to clot and giving temporary control of the bleeding disorder. Efanesoctocog alfa was designed to remain active in the body for longer than the natural factor VIII.

What benefits of Altuvoct have been shown in studies?

Altuvoct has been shown to be effective at preventing and treating bleeding in patients with severe haemophilia A.

In a main study involving 159 patients aged 12 years and above with severe haemophilia A, 133 patients received a weekly injection of Altuvoct to prevent bleeding (prophylaxis). After 52 weeks of treatment, patients had an average of around 0.70 bleeding episodes per year. For 77 patients, data on previous treatments were available; in this group, the average number of bleeding episodes was 0.69 with Altuvoct compared with around 3 with previous treatments. During the study, most bleeding episodes were successfully treated with a single injection of Altuvoct (on-demand treatment).

In a study involving 74 children under 12 years of age with haemophilia A, treatment with Altuvoct yielded similar results to those in older patients. Altuvoct was therefore considered effective for the treatment of haemophilia A in younger children.

Combined data from 3 studies involving 41 patients with haemophilia A who underwent major surgery showed that Altuvoct is effective at preventing bleeding episodes during and after surgery.

What are the risks associated with Altuvoct?

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

The most common side effects with Altuvoct (which may affect more than 1 in 10 people) include headache and joint pain. Other side effects with Altuvoct (which may affect up to 1 in 10 people) include vomiting, eczema (itchy, red and dry skin), rash, urticaria (itchy rash), pain in the extremities (arms and legs), back pain and fever.

As with all factor VIII medicines, patients may develop antibodies against efanesoctocog alfa, causing the medicine to stop working and resulting in a loss of bleeding control. In such cases, a specialised haemophilia centre should be contacted.

Why is Altuvoct authorised in the EU?

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

Altuvoct has been shown to prevent bleeding in adults and children with haemophilia A. At the time of authorisation, most authorised factor VIII preventive treatments required an injection every 2 to 4 days. Altuvoct is given once a week which is more convenient for patients and may help patients stick to their treatment. On-demand treatment with Altuvoct is effective at treating bleeding episodes, most of which resolve after a single injection. Altuvoct is also effective at preventing bleeding in patients undergoing surgery. The side effects with Altuvoct are similar to those with other factor VIII medicines and are considered manageable. Due to how factor VIII works, there is a low risk of thromboembolic events (formation of blood clots in blood vessels) with factor VIII medicines. The company marketing Altuvoct will further investigate this risk with Altuvoct.

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

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

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

Other information about Altuvoct

Altuvoct received a marketing authorisation valid throughout the EU on 17 June 2024.

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

This overview was last updated in 05-2024.