



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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EPAR summary for the public

Alprolix

eftrenonacog alfa

This is a summary of the European public assessment report (EPAR) for Alprolix. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Alprolix.

For practical information about using Alprolix, patients should read the package leaflet or contact their doctor or pharmacist.

What is Alprolix and what is it used for?

Alprolix is a medicine used to treat and prevent bleeding in patients with haemophilia B, an inherited bleeding disorder caused by lack of a clotting protein called factor IX. It can be used in patients of all ages.

Because the number of patients with haemophilia B is low, the disease is considered 'rare', and Alprolix was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 June 2007.

Alprolix contains the active substance eftrenonacog alfa.

How is Alprolix used?

Alprolix can only be obtained with a prescription and treatment should be under the supervision of a doctor who has experience in the treatment of haemophilia.

Alprolix is available as a powder and solvent that are mixed together to make a solution for injection into a vein. The dose and frequency of treatment depend on the patient's bodyweight and whether Alprolix is used to treat or prevent bleeding, as well as the severity of the patient's factor IX deficiency, the extent and location of the bleeding and the patient's age and health. For further information on how to use this medicine, see the summary of product characteristics (also part of the EPAR).



Patients or their carers may be able to inject Alprolix themselves at home once they have been trained appropriately. For full details, see the package leaflet.

How does Alprolix work?

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

What benefits of Alprolix have been shown in studies?

Alprolix has been shown to be effective at both preventing and treating bleeding episodes in 2 main studies in patients with haemophilia B.

In the first study involving 123 adults and adolescents aged 12 years or above, patients given Alprolix as a weekly preventive treatment had around 3 bleeding episodes a year, patients given Alprolix as an every 10 days preventive treatment had around 2 bleeding episodes per year, and patients given Alprolix for treating bleedings on demand had around 18 bleeding episodes a year. In addition, when bleeding did occur, around 90% of bleeding episodes resolved with one injection of Alprolix.

In the second study in 30 children aged below 12 years, Alprolix was similarly effective: 2 bleeding episodes occurred per year on average and around 75% of bleeding episodes resolved with one injection.

What are the risks associated with Alprolix?

Hypersensitivity (allergic) reactions are seen rarely with Alprolix and include: swelling, burning and stinging at the injection site, chills, flushing, itchy rash, headache, hives, low blood pressure, lethargy, nausea and vomiting, restlessness, a fast heartbeat, tightness of the chest and wheezing. In some cases these reactions can become severe.

Some patients taking factor IX medicines may develop inhibitors (antibodies) against factor IX, causing the medicine to stop working and resulting in a loss of bleeding control. Factor IX medicines can also potentially cause problems due to the formation of blood clots in the blood vessels.

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

Why is Alprolix approved?

Studies show that Alprolix is effective at preventing and treating bleeding episodes in patients with haemophilia B and its safety is comparable to that of other factor IX products. The Agency's Committee for Medicinal Products for Human Use (CHMP) therefore decided that Alprolix's benefits are greater than its risks and recommended that it be approved for use in the EU.

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

A risk management plan has been developed to ensure that Alprolix is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Alprolix, including the appropriate precautions to be followed by healthcare professionals and patients.

Other information about Alprolix

The European Commission granted a marketing authorisation valid throughout the European Union for Alprolix on 12 May 2016.

The full EPAR for Alprolix can be found on the Agency's website: ema.europa.eu/Find_medicine/Human_medicines/European_public_assessment_reports. For more information about treatment with Alprolix, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Alprolix can be found on the Agency's website: ema.europa.eu/Find_medicine/Human_medicines/Rare_disease_designation.

This summary was last updated in 05-2016.