EMA/591490/2015
EMEA/H/C/000139

EPAR summary for the public

BeneFIX

nonacog alfa

This document is a summary of the European Public Assessment Report (EPAR) for BeneFIX. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for BeneFIX.

What is BeneFIX?

BeneFIX is a medicine that contains the active substance nonacog alfa. It is available as a powder and solvent that are mixed together to form a solution for injection.

What is BeneFIX used for?

BeneFIX is used for the treatment and prevention of bleeding in adults and children with haemophilia B (an inherited bleeding disorder). BeneFIX is intended for either short-term or long-term use.

The medicine can only be obtained with a prescription.

How is BeneFIX used?

BeneFIX should be started by a doctor who has experience in the treatment of haemophilia. During treatment, the doctor should regularly carry out blood tests to adjust the dose to be given.

BeneFIX is given by a slow injection into a vein, usually at up to 4 ml per minute and should not be mixed with other infusion solutions or given using a kit other than the one supplied with the medicine. The dose and the frequency of injection depend on whether BeneFIX is used to treat or prevent bleeding, or to reduce bleeding during surgery, and on the patient's condition. The dose is adjusted depending on the severity and location of the bleeding, or the type of surgery. Full details on how to calculate the dose are included in the summary of product characteristics.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Patients or their carers can give injections of BeneFIX, provided that they have been trained appropriately.

How does BeneFIX work?

The active substance in BeneFIX, nonacog alfa, is a blood coagulation factor protein (a substance that helps the blood to clot). Patients with haemophilia B lack a protein called factor IX, which is involved in blood clotting. The lack of factor IX causes blood-clotting problems, such as bleeding in the joints, muscles and internal organs. BeneFIX is used to replace the missing factor IX. It corrects the factor IX deficiency and gives temporary control of the bleeding disorder.

Nonacog alfa is not extracted from human blood but produced by a method known as 'recombinant DNA technology': it is made by cells that have received a gene (DNA), which makes them able to produce human coagulation factor IX.

How has BeneFIX been studied?

BeneFIX has been studied in previously treated patients with moderate or severe haemophilia B for prevention or to treat bleeding episodes during and after surgery. It has also been studied in patients who have not received any treatment for their haemophilia. The studies assessed the number of bleeding episodes that occurred and rated BeneFIX's effectiveness using a scale from 'no response' to 'excellent'.

What benefit has BeneFIX shown during the studies?

In previously treated patients, 82% of the 693 bleeding episodes treated resolved after a single infusion of BeneFIX. Out of 972 infusions, 84% were rated as bringing about 'good' or 'excellent' responses.

What is the risk associated with BeneFIX?

Hypersensitivity (allergic) reactions have been seen in patients treated with products containing factor IX and may sometimes be severe. They include angioedema (swelling of the face and limbs), burning and stinging at the injection site, chills, flushing, urticaria (itchy rash), headache, hives, hypotension (low blood pressure), lethargy, nausea (feeling sick), restlessness, tachycardia (fast heartbeat), tightness of the chest, tingling, vomiting and wheezing. Patients with haemophilia B may also develop neutralising antibodies (inhibitors) to factor IX. If antibodies develop, BeneFIX will not work effectively, which may result in a loss of bleeding control. For the full list of all side effects reported with BeneFIX, see the package leaflet.

BeneFIX must not be used in people who are hypersensitive (allergic) to recombinant coagulation factor IX, to any of the other ingredients, or to hamster proteins.

Why has BeneFIX been approved?

The CHMP decided that BeneFIX's benefits are greater than its risks and recommended that it be given marketing authorisation.

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

A risk management plan has been developed to ensure that BeneFIX 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 BeneFIX, including the appropriate precautions to be followed by healthcare professionals and patients.

Other information about BeneFIX

The European Commission granted a marketing authorisation valid throughout the European Union for BeneFIX on 27 August 1997.

The full EPAR for BeneFIX 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 BeneFIX, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 09-2015.