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EPAR summary for the public

Rixubis

nonacog gamma

This is a summary of the European public assessment report (EPAR) for Rixubis. 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 Rixubis.

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

What is Rixubis and what is it used for?

Rixubis is a medicine used to treat and prevent bleeding in patients with haemophilia B, an inherited bleeding disorder caused by lack of factor IX. It can be used in patients of all ages, and for short-term or long-term use. Rixubis contains the active substance nonacog gamma.

How is Rixubis used?

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

Rixubis 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 Rixubis is used to treat or prevent bleeding, as well as the seriousness of the haemophilia, the extent and location of the bleeding and the patient's age and health. For further information, see the summary of product characteristics (also part of the EPAR).

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

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How does Rixubis work?

Patients with haemophilia B lack factor IX, which is needed for blood to clot properly. This lack causes blood clotting problems, such as bleeding in the joints, muscles or internal organs. The active substance in Rixubis, nonacog gamma, is a version of human factor IX and helps the blood to clot in the same way. Rixubis can therefore be used to replace the missing factor IX, giving temporary control of the bleeding disorder.

Nonacog gamma is not extracted from human blood but is made by a method known as 'recombinant DNA technology': it is made by hamster cells into which a gene (DNA) has been introduced that makes the cells able to produce the human clotting factor.

What benefits of Rixubis have been shown in studies?

The benefits of Rixubis in treating and preventing bleeding episodes have been shown in three main studies involving patients with severe or moderately severe haemophilia B. None of the studies compared the effectiveness of Rixubis directly with another medicine. Effectiveness in stopping bleeding was measured on a standard scale in which 'excellent' meant complete relief of pain and no signs of bleeding after a single dose of the medicine, and 'good' meant relief of pain and signs of improvement with a single dose, although further doses might be needed for complete resolution.

In the first study, involving 73 patients aged 12 to 59 years, 249 bleeding episodes were treated with Rixubis. The effect of treatment in stopping bleeding episodes was rated excellent in 41% of cases and good in a further 55%. With respect to prevention of bleeding, the average bleeding rate during treatment was 4.26 bleeds a year, compared with an average of about 17 per year before enrolment. A second study involved 23 children aged from just under 2 to nearly 12 years of age, who experienced 26 bleeding episodes during the study: treatment of bleeding episodes was rated as excellent in 50% of cases and good in another 46%, and the average bleeding rate was reduced from 6.8 to 2.7 bleeds per year. In a third study, Rixubis was given to 14 patients undergoing surgery; treatment with Rixubis kept blood loss during the surgery to levels expected in patients without haemophilia B.

The evaluation of these studies also indicated that Rixubis was distributed in the body similarly to another approved factor IX product.

What are the risks associated with Rixubis?

The most common side effects with Rixubis (which may affect up to 1 in 10 people) are dysgeusia (taste disturbances) and pain in the limbs. Hypersensitivity (allergic) reactions may occur rarely, and can include angioedema (swelling of tissues under the skin), burning and stinging at the injection site, chills, flushing, itchy rash, headache, hives, hypotension (low blood pressure), feeling tired or restless, nausea (feeling sick) or vomiting, tachycardia (rapid heartbeat), tightness of the chest, wheezing and tingling sensations. In some cases, reactions become severe (anaphylaxis) and may be associated with dangerously steep falls in blood pressure. For the full list of all side effects with Rixubis see the package leaflet.

Rixubis must not be used in patients who are hypersensitive (allergic) to nonacog gamma or any of its other ingredients, or who are known to be allergic to hamster protein.

Why is Rixubis approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Rixubis's benefits are greater than its risks and recommended that it be approved for use in the EU. The Committee considered that Rixubis had been shown to be effective in preventing and treating bleeding episodes in

adults and children with haemophilia B, and was also effective in allowing them to undergo surgery safely. The safety profile was considered acceptable and was outweighed by the beneficial effects.

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

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

Further information can be found in the summary of the risk management plan.

Other information about Rixubis

The European Commission granted a marketing authorisation valid throughout the European Union for Rixubis on 19 December 2014.

The full EPAR and risk management plan summary for Rixubis 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 Rixubis, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 01-2015.