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EPAR summary for the public

NovoThirteen

catridecacog

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

What is NovoThirteen?

NovoThirteen is a medicine that contains the active substance catridecacog. It is available as a powder and solvent to be made up into a solution for injection.

What is NovoThirteen used for?

NovoThirteen is used for the long-term prevention of bleeding in patients with a condition called 'congenital factor XIII A-subunit deficiency'. This is an inherited blood clotting disorder characterised by bleeding episodes.

The medicine can only be obtained with a prescription.

How is NovoThirteen used?

Treatment with NovoThirteen should be started under the supervision of a doctor experienced in treating rare bleeding disorders. The condition must be confirmed by suitable tests before starting treatment.

The recommended dose is 35 international units (IU) per kilogram body weight once per month. This dose is given in a single, slow injection into a vein. In certain situations the doctor may decide to adjust the dose required to prevent bleeding, based on the patient's factor XIII levels in the blood.

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How does NovoThirteen work?

Factor XIII is a protein involved in the blood clotting process. A specific component of factor XIII called the A-subunit is involved in stabilising blood clots and increasing the strength of the clot. Patients with congenital factor XIII A-subunit deficiency do not have enough of the factor XIII A-subunit, or it does not work correctly, which makes them prone to bleeding. The active substance in NovoThirteen, catridecacog, is structurally the same as the human factor XIII A-subunit. NovoThirteen works by providing the factor XIII A-subunit, which helps to prevent bleeding in these patients. NovoThirteen does not work in patients lacking the factor XIII B-subunit.

The factor XIII A-subunit in NovoThirteen is made by a method known as 'recombinant DNA technology': it is produced by yeast cells that have received the gene (DNA) which makes the cells able to produce it.

How has NovoThirteen been studied?

NovoThirteen was investigated in a main study involving 41 adults and children above 6 years of age with congenital factor XIII A-subunit deficiency who had been treated in the past with medicines containing factor XIII. The patients were given NovoThirteen for one year for preventative use. The study looked at the number of bleeding episodes requiring treatment with a factor XIII-containing medicine in these patients, compared with patients who had not received NovoThirteen, based on earlier data from 16 patients with congenital factor XIII A-subunit deficiency. The study was extended for a further year in 33 of the patients, to look at the long-term safety of treatment with NovoThirteen.

The safety and effectiveness of NovoThirteen in children below 6 years of age was supported by preliminary data from an ongoing long-term study during which the patients were given NovoThirteen to prevent bleeding episodes.

What benefit has NovoThirteen shown during the studies?

The rate of bleeding episodes in patients receiving NovoThirteen preventatively was lower than the rate seen in the earlier data from patients treated on demand with another medicine containing factor XIII. On average, there were approximately 0.15 bleeding episodes requiring factor XIII treatment per year per patient treated preventatively with NovoThirteen. This compared with around 2.9 episodes per year per patient treated on demand with another medicine containing factor XIII.

During the long-term study, no bleeding episodes were observed in children below 6 years of age treated with NovoThirteen.

What is the risk associated with NovoThirteen?

The most common side effects with NovoThirteen (seen in between 1 and 10 patients in 100) are headache, leucopenia (low white blood cell counts), aggravated neutropenia (low levels of neutrophils, a type of white blood cell), pain in the extremities, injection site pain, and the presence in the blood of antibodies that attach to factor XIII and of small protein fragments called 'fibrin D-dimer'.

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

Why has NovoThirteen been approved?

The CHMP concluded that the main study provided satisfactory results in terms of effectiveness, since no severe or life-threatening bleedings took place during treatment with NovoThirteen. Moreover, no major side effects were seen with long term use of NovoThirteen. The CHMP decided that

NovoThirteen'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 NovoThirteen?

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

Additionally, the company that markets NovoThirteen will provide all doctors who are expected to prescribe it with an information pack for doctors and patients containing important information on the correct use of the medicine. This includes information on storage procedures, since incorrect storage after reconstitution can cause increased levels of activated NovoThirteen, thereby increasing the risk of thrombosis (formation of blood clots in the blood vessels), and information on administration procedures, since the factor XIII concentration in NovoThirteen is different from other factor XIII-containing medicines.

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

Other information about NovoThirteen

The European Commission granted a marketing authorisation valid throughout the European Union for NovoThirteen on 3 September 2012.

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

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