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Hemgenix (*etranacogene dezaparvovec*)

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

What is Hemgenix and what is it used for?

Hemgenix is a medicine used to treat adults with severe and moderately severe haemophilia B, an inherited bleeding disorder caused by the lack of factor IX (a protein needed to produce blood clots to stop bleeding). It is used in adults who have not developed inhibitors (proteins made by the body's natural defenses) against factor IX.

Haemophilia B is rare, and Hemgenix was designated an 'orphan medicine' (a medicine used in rare diseases) on 21 March 2018. Further information on the orphan designation can be found here: https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu-3-18-1999.

Hemgenix contains the active substance etranacogene dezaparvovec and is a type of advanced therapy medicine called a 'gene therapy product'. This is a type of medicine that works by delivering genes into the body.

How is Hemgenix used?

The medicine can only be obtained with a prescription. Treatment must be started under the supervision of a doctor experienced in the treatment of haemophilia and/or bleeding disorders, in a facility equipped to promptly treat infusion-related reactions.

Hemgenix is given once, as a single infusion (drip) into a vein lasting one to two hours. The dose depends on the patient's body weight.

Before receiving the infusion, the patient will have a number of tests, including tests to check their liver health and if they have factor IX inhibitors. As it will be several weeks before Hemgenix shows any effects, patients will be monitored closely for at least 3 months after the infusion to decide if they need additional treatment with factor IX replacement therapy.

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

How does Hemgenix work?

Patients with haemophilia B have mutations (changes) in a gene which the body needs to make the clotting protein Factor IX, resulting in either a partial or complete lack of its activity.

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The active substance in Hemgenix, etranacogene dezaparvovec, is based on a virus that contains copies of the gene responsible for producing factor IX. When given to the patient, the virus will carry the factor IX gene to the liver cells, enabling them to produce the missing factor IX and thereby limit bleeding episodes.

The type of virus used in this medicine (adeno-associated virus) does not cause disease in humans.

What benefits of Hemgenix have been shown in studies?

A study in 54 adult male patients with severe or moderately severe haemophilia B found that Hemgenix was more effective at reducing bleeding events than factor IX replacement therapy. The study compared the number of bleeding episodes patients had with factor IX replacement therapy during a 6-month period before receiving Hemgenix with the number experienced over a 1-year period after achieving stable factor IX levels with Hemgenix. Data from the study showed that Hemgenix reduced the yearly bleeding rate from 4.2 to 1.5 bleeds per year. The study also found that Hemgenix was effective at increasing factor IX levels, with 96% of patients (52 out of 54) no longer needing factor IX replacement therapy for up to 2 years after the infusion.

What are the risks associated with Hemgenix?

The most common side effects with Hemgenix (which may affect more than 1 in 10 people) are headache, increased levels of certain liver enzymes and flu-like symptoms.

For the full list of side effects of Hemgenix, see the package leaflet.

Hemgenix must not be given to people who are hypersensitive (allergic) to any of its ingredients, who have an active or chronic (long-term) infection that is not controlled by medicines, or who have advanced liver fibrosis or liver cirrhosis (scarring of the liver).

Why is Hemgenix authorised in the EU?

At the time of approval, patients with severe haemophilia B required lifelong treatment with factor IX replacement therapy. Hemgenix, given as a single infusion, was effective at preventing bleeding over a period of at least 2 years, thus enabling patients to discontinue treatment with factor IX replacement therapy, which reduces the burden caused by treating the disease. There are some uncertainties about how long the benefits of Hemgenix last, given that the main study evaluated the response in a small number of patients for up to 2 years. Although the long-term safety data were limited, the safety profile was considered acceptable.

Hemgenix has been given ‘conditional authorisation’. This means that the European Medicines Agency decided that the benefits of Hemgenix are greater than its risks, but the company will have to provide additional evidence after authorisation.

Conditional authorisation is granted on the basis of less comprehensive data than are normally required. It is granted for medicines that fulfil an unmet medical need to treat serious diseases and when the benefits of having them available earlier outweigh any risks associated with using the medicines while waiting for further evidence. Every year, the European Medicines Agency will review any new information that becomes available until data become comprehensive and this overview will be updated as necessary.

What information is still awaited for Hemgenix?

Since Hemgenix has been given conditional authorisation, the company that markets this medicine will provide additional data from ongoing studies on the long-term safety and effectiveness of the medicine, including how long the response lasts, in patients with severe to moderately severe haemophilia B. The company will also provide data from a registry of patients treated with Hemgenix to study its long-term safety and effectiveness.

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

The company that markets Hemgenix will provide educational materials to patients or their carers and to healthcare professionals, with information on the benefits, risks and uncertainties about the long-term effects and safety of the medicine. Patients must also be given a patient card to inform healthcare professionals that they have been treated with Hemgenix.

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

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

Other information about Hemgenix

Hemgenix received a conditional marketing authorisation valid throughout the EU on 20 February 2023.

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

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