EMA/331501/2024
EMEA/H/C/004774

Durveqtix (*fidanacogene elaparvovec*)

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

What is Durveqtix and what is it used for?

Durveqtix is a medicine used to treat adults with severe or 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 and who do not have antibodies against the virus contained in the medicine (see section on how the medicine works).

Durveqtix contains the active substance fidanacogene elaparvovec 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 Durveqtix used?

Durveqtix can only be obtained with a prescription. Treatment must be given by a doctor experienced in the treatment of haemophilia and in a qualified facility that is equipped to promptly treat possible infusion-related reactions.

Durveqtix is given once, as a single infusion (drip) into a vein lasting about an hour. The dose depends on the patient's weight.

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

How does Durveqtix work?

Patients with haemophilia B have mutations (changes) in a gene that the body needs to make the clotting protein factor IX, resulting in either a reduction or absence of factor IX activity. Without enough factor IX, the blood cannot clot properly to control bleeding.

The active substance in Durveqtix, fidanacogene elaparvovec, is based on a virus that has been modified to contain a copy 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.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

The type of virus used in this medicine (adeno-associated virus serotype Rh74) does not cause disease in humans.

What benefits of Durveqtix have been shown in studies?

A main study in 45 men with severe or moderately severe haemophilia B found that Durveqtix is more effective than regular preventive factor IX replacement therapy at reducing the number of bleedings.

The study compared the number of bleeding episodes patients had with regular preventive factor IX replacement therapy during a 6-month period before receiving Durveqtix with the number they experienced over a 1-year period starting three months after receiving Durveqtix. Data showed that Durveqtix was effective at increasing factor IX levels and reduced the yearly bleeding rate from 4.50 to 1.44 bleedings per year. About 60% of patients (27 out of 45) did not experience any bleeding for at least 2 years after treatment with Durveqtix.

What are the risks associated with Durveqtix?

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

The most common side effects with Durveqtix (which may affect more than 1 in 10 people) include increased levels of certain liver enzymes (transaminases).

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

Why is Durveqtix authorised in the EU?

At the time of approval, most treatment options for patients with severe haemophilia B involved lifelong frequent treatment with factor IX replacement therapy. Durveqtix, given as a single infusion, was shown to be effective at preventing bleeding over a period of at least 2 years, thus enabling most patients to stop treatment with preventive factor IX replacement therapy. It was noted however that some patients in the main study had to resume preventive factor IX therapy because Durveqtix did not work well enough for them. In addition, there are some uncertainties about how long the benefits of Durveqtix last. The safety profile of Durveqtix was considered acceptable.

The European Medicines Agency therefore decided that Durveqtix’s benefits are greater than its risks and that it can be authorised for use in the EU.

Durveqtix has been given conditional authorisation. This means that it has been authorised on the basis of less comprehensive data than are normally required because it fulfils an unmet medical need. The Agency considers that the benefit of having the medicine available earlier outweighs any risks associated with using it while awaiting further evidence.

The company must provide further data on Durveqtix. It must submit long-term data on the effectiveness and safety of the medicine from ongoing studies. Every year, the Agency will review any new information that becomes available.

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

In addition to the data requested as part of the conditional marketing authorisation, the company that markets Durveqtix will also provide data from a registry-based study with patients treated with the medicine to study its long-term safety and effectiveness as well as data from a long-term study.

The company 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 Durveqtix.

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

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

Other information about Durveqtix

Durveqtix received a conditional marketing authorisation valid throughout the EU on 24 July 2024.

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

This overview was last updated in 07-2024.