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Cerdelga (*eliglustat*)

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

What is Cerdelga and what is it used for?

Cerdelga is a medicine used for the long-term treatment of type 1 Gaucher disease in adults and in children from 6 years of age, weighing at least 15 kg, whose disease is well controlled by enzyme replacement therapy (ERT).

Gaucher disease is a genetic condition, in which a fat called glucosylceramide (or glucocerebroside) builds up in the body, typically in the liver, spleen and bone. This causes symptoms such as anaemia (low levels of red blood cells), tiredness, easy bruising, an enlarged spleen and liver, and bone pain and fractures. The disease is caused by the lack of an enzyme responsible for breaking down fat.

Gaucher disease is rare, and Cerdelga was designated an 'orphan medicine' (a medicine used in rare diseases) on 4 December 2007. Further information on the orphan designation can be found on the EMA website.

Cerdelga contains the active substance eliglustat.

How is Cerdelga used?

Cerdelga can only be obtained with a prescription and treatment should be started and supervised by a doctor who has experience in the management of Gaucher disease.

Cerdelga is available as capsules to be taken by mouth. It should not be taken together with grapefruit or its juice. To be treated with Cerdelga, children must be able to swallow the capsules. Before starting treatment, the patient is tested to see how rapidly their bodies break down the medicine. Adults who break down the medicine at normal speed take 1 capsule twice a day, while those who break down the medicine slowly take 1 capsule once a day. Children who break down the medicine at a normal speed take 2 capsules twice a day, while those who break down the medicine slowly take 1 capsule once a day.

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Patients whose bodies break down this medicine very rapidly ('ultrarapid metabolisers') should not take Cerdelga. Patients who have not been tested or for whom results of the test are not clear should also not take this medicine.

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

How does Cerdelga work?

The active substance in Cerdelga, eliglustat, works by blocking the action of an enzyme involved in the production of the glucosylceramide fat. Because the build-up of this fat in organs like the spleen, liver and bones is responsible for the symptoms of type 1 Gaucher disease, reducing its production helps prevent its build-up, and thereby helps the affected organs to function better.

What benefits of Cerdelga have been shown in studies?

Studies show that Cerdelga is effective at treating type 1 Gaucher disease, including reducing the size of enlarged spleens and livers.

In a study involving 40 previously untreated patients with type 1 Gaucher disease, patients taking Cerdelga had a 28% reduction in spleen size, compared with a 2% increase in those taking placebo (a dummy treatment) after 9 months of treatment. Patients who took Cerdelga also showed an improvement in other signs of the disease such as a reduction in liver size and increased levels of haemoglobin (the protein in red blood cells that carries oxygen).

Another study looked at Cerdelga in 159 patients whose disease was well controlled by enzyme replacement therapy. In this study, the disease remained stable in 85% of patients who switched to Cerdelga for a year and in 94% of patients who continued with enzyme replacement therapy.

A further study in 51 children aged 2 to 17 years with type 1 and 3 Gaucher disease showed that Cerdelga is expected to work similarly in children as it does in adults.

What are the risks associated with Cerdelga?

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

The most common side effect with Cerdelga is dyspepsia (heartburn), which may affect up to 1 in 10 adults and more than 1 in 10 children.

Cerdelga must not be taken by some patients with liver problems or those taking certain medicines that can interfere with the ability of the body to break down the medicine.

Why is Cerdelga authorised in the EU?

Studies show that Cerdelga is effective in improving the symptoms of type 1 Gaucher disease in a majority of adults who had not previously been treated, and in keeping the disease stable in most adults previously treated with enzyme replacement therapy (ERT). However, a minority of patients (about 15%) switching from ERT to Cerdelga did not respond optimally after treatment for one year. For these patients, other treatment options should be considered. All patients switching from ERT to Cerdelga should be regularly monitored for disease progression.

Regarding safety, side effects were mostly mild and short-lived, but the company that markets Cerdelga must further investigate the long-term safety of the medicine.

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

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

The company that markets Cerdelga will provide doctors expected to prescribe the medicine with a prescriber guide and patients who are prescribed the medicine with a patient alert card. These educational materials will help ensure that only patients with type 1 Gaucher disease are treated with Cerdelga and that Cerdelga is not used in some patients with liver problems and together with medicines which can significantly alter Cerdelga's blood levels. The company will also keep record of patients treated with Cerdelga to look at the long-term safety of the medicine.

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

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

Other information about Cerdelga

Cerdelga received a marketing authorisation valid throughout the EU on 19 January 2015.

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

This overview was last updated in 11-2024.