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Miglustat Dipharma (*miglustat*)

An overview of Miglustat Dipharma and why it is authorised in the EU

What is Miglustat Dipharma and what is it used for?

Miglustat Dipharma is used to treat two inherited diseases that affect the way the body handles fats. Both diseases cause a build-up of fatty substances called glycosphingolipids in the body. Miglustat Dipharma is used to treat the following patients:

- adults (aged 18 years and above) with mild to moderate type-1 Gaucher disease. Patients with this disease lack an enzyme called glucocerebrosidase, which results in a glycosphingolipid called glucosylceramide building up in different parts of the body, such as the spleen, liver and bones. Miglustat Dipharma is used in patients who cannot receive the standard treatment of enzyme-replacement therapy (ERT);
- patients of all ages with Niemann-Pick type-C disease, a potentially fatal disease in which glycosphingolipids build up within cells in the brain and elsewhere in the body. Miglustat Dipharma is used to treat the neurological symptoms of the disease (symptoms affecting the brain and nerves). These include loss of coordination, problems with 'saccadic' (rapid) eye movements that can lead to impaired vision, delayed development, difficulty swallowing, decreased muscle tone, fits and learning difficulties.

Miglustat Dipharma is a 'generic medicine'. This means that Miglustat Dipharma contains the same active substance and works in the same way as a 'reference medicine' already authorised in the EU called Zavesca. For more information on generic medicines, see the question-and-answer document here.

How is Miglustat Dipharma used?

Miglustat Dipharma is available as 100 mg capsules to be taken by mouth. The recommended starting dose for type-1 Gaucher disease is one capsule three times a day. For Niemann-Pick type-C disease, it is two capsules three times a day for patients aged 12 years and over; in younger patients, the dose depends on their weight and height. Miglustat Dipharma is intended for long-term use.

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The medicine can only be obtained with a prescription and treatment should be supervised by doctors who are experienced in the management of Gaucher disease.

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

How does Miglustat Dipharma work?

The active substance in Miglustat Dipharma, miglustat, blocks an enzyme called glucosylceramide synthase. This enzyme is involved in the first step of the production of glucosylceramide. By preventing the enzyme from working, miglustat can reduce the production of glucosylceramide in cells, thereby reducing the symptoms of type-1 Gaucher disease.

How has Miglustat Dipharma been studied?

Studies on the benefits and risks of the active substance in Gaucher disease have already been carried out with the reference medicine, Zavesca, and do not need to be repeated for Miglustat Dipharma.

As for every medicine, the company provided studies on the quality of Miglustat Dipharma. The company also carried out a study that showed that it is 'bioequivalent' to the reference medicine. Two medicines are bioequivalent when they produce the same levels of the active substance in the body and are therefore expected to have the same effect.

What are the benefits and risks of Miglustat Dipharma?

Because Miglustat Dipharma is a generic medicine and is bioequivalent to the reference medicine, its benefits and risks are taken as being the same as the reference medicine's.

Why is Miglustat Dipharma authorised in the EU?

The European Medicines Agency concluded that, in accordance with EU requirements, Miglustat Dipharma has been shown to have comparable quality and to be bioequivalent to Zavesca. Therefore, the Agency's view was that, as for Zavesca, the benefit of Miglustat Dipharma outweighs the identified risk and it can be authorised for use in the EU.

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

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

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

Other information about Miglustat Dipharma

Miglustat Dipharma received a marketing authorisation valid throughout the EU on 18 February 2019.

Further information on Miglustat Dipharma can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/miglustat-dipharma. Information on the reference medicine can also be found on the Agency's website.

This overview was last updated in 06-2019