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EPAR summary for the public

Yargesa

miglustat

This is a summary of the European public assessment report (EPAR) for Yargesa. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Yargesa.

For practical information about using Yargesa, patients should read the package leaflet or contact their doctor or pharmacist.

What is Yargesa and what is it used for?

Yargesa is a medicine used to treat adults with mild to moderate type-1 Gaucher disease.

Patients with this disease lack an enzyme that breaks down a type of fat called glucosylceramide. As a result, glucosylceramide builds up in different parts of the body, such as the spleen, liver and bones. Yargesa is used in patients who cannot receive enzyme-replacement therapy.

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

How is Yargesa used?

Yargesa is available as 100 mg capsules to be taken by mouth. The recommended starting dose is one capsule three times a day. A lower dose should be used in patients with reduced kidney function and those who develop diarrhoea.

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, see the package leaflet.

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How does Yargesa work?

The active substance in Yargesa, miglustat, prevents an enzyme called glucosylceramide synthase from working. 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 Yargesa been studied?

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

As for every medicine, the company provided studies on the quality of Yargesa. 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 Yargesa?

Because Yargesa 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 Yargesa approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) concluded that, in accordance with EU requirements, Yargesa has been shown to have comparable quality and to be bioequivalent to Zavesca. Therefore, the CHMP's view was that, as for Zavesca, the benefit outweighs the identified risk. The Committee recommended that Yargesa be approved for use in the EU.

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

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

Other information about Yargesa

The European Commission granted a marketing authorisation valid throughout the European Union for Yargesa on 22 March 2017.

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

The full EPAR for the reference medicine can also be found on the Agency's website.

This summary was last updated in 03-2017.