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EPAR summary for the public

Galafold

migalastat

This is a summary of the European public assessment report (EPAR) for Galafold. 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 Galafold.

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

What is Galafold and what is it used for?

Galafold is a medicine used to treat patients aged 16 years or over with Fabry disease. This is a rare inherited disorder where patients have various mutations (changes) in the gene responsible for the production of an enzyme called alpha-galactosidase A, which normally breaks down a fatty substance called globotriaosylceramide (GL-3). In patients with Fabry disease, this enzyme does not work properly. As a result, GL-3 cannot be broken down and it builds up in various cells in the body, including in the heart and kidneys.

Because the number of patients with Fabry disease is low, the disease is considered 'rare' and Galafold was designated an 'orphan medicine' (a medicine used in rare diseases) on 22 May 2006.

Galafold contains the active substance migalastat.

How is Galafold used?

Galafold can only be obtained with a prescription and treatment should only be started and supervised by a doctor who is experienced in the diagnosis and treatment of Fabry disease.

Galafold is available as capsules (123 mg). The recommended dose of Galafold is one capsule every other day, taken by mouth at least 2 hours before or after food.

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Galafold is only for use in patients with certain mutations in the alpha-galactosidase A gene. For more information, see the summary of product characteristics (also part of the EPAR).

How does Galafold work?

The active substance in Galafold, migalastat, attaches to certain unstable forms of alpha-galactosidase A, stabilising the enzyme. This allows the enzyme to be transported into areas of the cell where it can break down GL-3.

What benefits of Galafold have been shown in studies?

Galafold has been investigated in two main studies involving a total of 127 patients with Fabry disease.

The first study, which compared Galafold with placebo (a dummy treatment) in 67 patients, looked at the proportion of patients who responded to treatment (defined as a reduction of at least 50% in GL-3 deposits in the kidneys). Overall, Galafold was not found to be more effective than placebo at reducing GL-3 deposits; however, additional analyses including only patients with those genetic mutations that can be treated with Galafold showed that patients responded better to Galafold than to placebo after 6 months of treatment.

The second study, in 60 patients, compared Galafold with the medicines agalsidase alfa or agalsidase beta, two treatments that replace the missing enzyme. The main measure of effectiveness was the change in patients' kidney function after 18 months of treatment. In this study, Galafold was found to be as effective as enzyme replacement in stabilising patients' kidney function.

What are the risks associated with Galafold?

The most common side effect with Galafold (which may affect around 1 in 10 people) is headache.

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

Why is Galafold approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Galafold's benefits are greater than its risks and recommended that it be approved for use in the EU. The Committee noted that Galafold was studied in a limited number of patients, however the available evidence is considered sufficient for such a rare disease. The CHMP also noted that Galafold is taken by mouth and this could be an advantage compared with other authorised treatments such as enzyme replacement which are given by infusion (drip) into a vein. Regarding safety, Galafold was well-tolerated.

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

A risk management plan has been developed to ensure that Galafold is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Galafold, including the appropriate precautions to be followed by healthcare professionals and patients.

Other information about Galafold

The European Commission granted a marketing authorisation valid throughout the European Union for Galafold on 26 May 2016.

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

The summary of the opinion of the Committee for Orphan Medicinal Products for Galafold can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 05-2016.