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Galafold (*migalastat*)

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

What is Galafold and what is it used for?

Galafold is a medicine used to treat patients aged 12 years and above who have 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. This enzyme normally breaks down a fatty substance called globotriaosylceramide (GL-3). As a result of the mutations, the enzyme does not work properly and cannot break down GL-3. This leads to a build up of GL-3 in various cells in the body, including in the heart and kidneys.

Fabry disease is rare, and Galafold was designated an 'orphan medicine' (a medicine used in rare diseases) on 22 May 2006. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu306368.

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. The recommended dose of Galafold is one capsule every other day. Patients should not consume any food at least 2 hours before and 2 hours after taking Galafold to allow full absorption.

Galafold is only for use in patients with certain mutations in the alpha-galactosidase A gene. For more information about using Galafold, see the package leaflet or contact your doctor or pharmacist.

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.

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What benefits of Galafold have been shown in studies?

Galafold has been investigated in two main studies involving a total of 127 patients [AI1]aged over 16 yewith 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.

The company also provided results of a study that showed that Galafold produces the same levels of the active substance in the body and has the same effect in adolescents aged 12 up to and including 15 years old as in adults and young people aged 16 and above.

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 authorised in the EU?

The European Medicines Agency decided that Galafold's benefits are greater than its risks and it can be authorised for use in the EU. The Agency noted that Galafold was studied in a limited number of patients, however the available evidence is considered sufficient for such a rare disease. The Agency 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?

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

Other information about Galafold

Galafold received a marketing authorisation valid throughout the EU on 26 May 2016.

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

This overview was last updated in 07-2021.