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Elfabrio (*pegunigalsidase alfa*)

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

What is Elfabrio and what is it used for?

Elfabrio is a medicine used in adults to treat Fabry disease, a rare inherited disorder. Patients with Fabry disease do not have enough of an enzyme, called alpha-galactosidase A, that normally breaks down a fatty substance called globotriaosylceramide (Gb3). If the enzyme is not present, Gb3 cannot be broken down and it builds up in the organs, such as the kidney and heart, causing kidney failure and heart problems.

Elfabrio contains the active substance pegunigalsidase alfa.

How is Elfabrio used?

Elfabrio can only be obtained with a prescription and treatment should be supervised by a doctor with experience in treating Fabry disease.

Elfabrio is available as an infusion (drip) into a vein, which is given once every two weeks. It is intended for long-term use. All patients are monitored for any reactions during the infusion and for at least one to two hours afterwards. To reduce the risk of infusion-related reactions, patients may be given other medicines before or during treatment with Elfabrio or the infusion time may be slowed down. The infusions are given in hospital but may be given at home if the patient is tolerating the infusions well.

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

How does Elfabrio work?

Elfabrio is an enzyme replacement therapy. This type of therapy provides patients with the enzyme they are lacking. Elfabrio is designed to replace the human enzyme alpha-galactosidase A, which is lacking in patients with Fabry disease. The active substance in Elfabrio, pegunigalsidase alfa, is a copy of the human enzyme, produced by a method known as ‘recombinant DNA technology’: it is made by cells that have received a gene (DNA), which makes them able to produce the enzyme. The replacement enzyme helps to break down Gb-3 and stops it building up in the patient’s cells.

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What benefits of Elfabrio have been shown in studies?

The benefits of Elfabrio were evaluated in one main study, involving 78 patients with Fabry disease. The study compared Elfabrio with Fabrazyme, another enzyme replacement therapy used to treat patients with Fabry disease. The main measure of effectiveness was based on glomerular filtration rate (GFR; a measure of how well the kidneys are working), which decreases as a patient's kidney function gets worse. The average GFR reduced by 2.5 mL/min/1.73m^2 per year after both 12 and 24 months of treatment with Elfabrio compared with 1.7 mL/min/1.73m^2 and 2.2 mL/min/1.73m^2 in those who received Fabrazyme. Supportive data showed a significant decrease in the levels of Gb3 in the kidneys and blood of patients treated with Elfabrio.

What are the risks associated with Elfabrio?

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

The most common side effects with Elfabrio (which may affect up to 1 in 10 people) include hypersensitivity (allergic reactions), asthenia (weakness) and infusion-associated reactions.

Allergic reactions, including excessive and prolonged contraction of the airway muscles causing breathing difficulty (bronchospasm), can occur in people receiving Elfabrio.

Why is Elfabrio authorised in the EU?

The Agency considered that the efficacy of Elfabrio was supported by nature of the active substance, specifically that it is a pegylated form of the natural occurring enzyme, and also by the well-established mechanism of action of enzyme replacement therapy in the treatment of Fabry disease. Results from the main study showed a significant decrease in the levels of Gb3 in both the kidneys and blood of patients treated with Elfabrio. Due to limitations with the study design, including the small number of patients involved, results of the main study did not conclusively show that Elfabrio was at least as effective as Fabrazyme.

Furthermore, the side effects of Elfabrio, which are mainly related to the infusion, are considered manageable. The European Medicines Agency therefore decided that Elfabrio'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 Elfabrio?

The company that markets Elfabrio will provide educational materials to patients or their carers and to healthcare professionals, with information on how to give the product correctly within the home setting.

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

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

Other information about Elfabrio

Elfabrio received a marketing authorisation valid throughout the EU on 4 May 2023.

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

This overview was last updated in 05-2023.