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Loargys (*pegzilarginase*)

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

What is Loargys and what is it used for?

Loargys is a medicine used to treat people from 2 years of age with hyperargininaemia.

Patients with hyperargininaemia are unable to break down the amino acid arginine because they lack the liver enzyme arginase 1. As a result, arginine accumulates in the body. This can cause problems with the nervous system, including seizures and stiffness in the legs.

Hyperargininaemia is rare, and Loargys was designated an 'orphan medicine' (a medicine used in rare diseases) on 14 July 2016. Further information on the orphan designation can be found on the EMA website: ema.europa.eu/en/medicines/human/orphan-designations/eu-3-16-1701.

Loargys contains the active substance pegzilarginase.

How is Loargys used?

The medicine can only be obtained with a prescription and treatment should be started and supervised by a doctor experienced in treating patients with inherited metabolic diseases. The first doses should be given in a location with appropriate medical support to manage allergic reactions.

Loargys is given once a week, as an infusion (drip) into a vein or as an injection under the skin. The dose depends on the patient's weight; it may be adjusted based on the levels of arginine in the patient's blood. Regular blood tests are needed to monitor arginine and adjust the dose if necessary.

Patients or their carers may be able to inject Loargys themselves after appropriate training. Before patients can inject Loargys, they must have received treatment for at least 8 weeks and be on a stable maintenance dose. Additionally, their risk of allergic reactions to Loargys must be assessed as low.

Loargys should be used together with other measures to manage the disease, including a low-protein diet, amino acid supplements and any other medicines needed to manage the disease.

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

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How does Loargys work?

The active substance in Loargys, pegzilarginase, works in a similar way as arginase 1, the enzyme lacking in people with hyperargininaemia. Pegzilarginase breaks down excess arginine from the blood. This prevents damage to the brain and other organs.

What benefits of Loargys have been shown in studies?

Loargys was compared with placebo (a dummy treatment) in a study involving 32 adults and children with hyperargininaemia. The main measure of effectiveness was the change in the blood level of arginine after 24 weeks of treatment. The study showed that the arginine levels were reduced by 77% in patients treated with Loargys, while there was no reduction in arginine levels in patients given placebo.

Although the study also suggested that Loargys may improve motor function compared with placebo, the difference was not statistically significant (i.e. it may be due to chance). However, preliminary longer-term data collected beyond the 24-week treatment period indicated that motor function (walking and standing) may stabilise or gradually improve with long-term use of the medicine.

What are the risks associated with Loargys?

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

The most common side effects with Loargys include allergic reactions (which may affect more than 1 in 10 people).

Why is Loargys authorised in the EU?

Loargys was shown to reduce arginine levels in patients with hyperargininaemia. Data also indicated that long-term treatment with Loargys gradually improves or stabilises patients' motor skills. Although safety data are limited, side effects are generally considered manageable. The European Medicines Agency therefore decided that Loargys' benefits are greater than its risks and it can be authorised for use in the EU.

Loargys has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Loargys due to the rarity of the disease. Every year, the Agency will review any new information that becomes available and this overview will be updated as necessary.

Since Loargys was authorised under exceptional circumstances, at the time of authorisation the company marketing Loargys was required to provide annual updates on the collection of data from two registry-based studies on the long-term effectiveness and safety of pegzilarginase in patients with hyperargininaemia. The company will also provide the final results from two studies on the long-term safety, tolerability and effectiveness of pegzilarginase in adults, adolescents and children with hyperargininaemia. They will also provide an annual update on any new information on the effectiveness and safety of the medicine in the intended population.

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

The company that markets Loargys will provide patients and carers with educational material on how to handle and inject the medicine, to inform them about the risks of severe allergic reactions and the need to contact their doctor if they experience symptoms.

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

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

Other information about Loargys

Loargys received a marketing authorisation under exceptional circumstances valid throughout the EU on 15 December 2023.

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

This overview was last updated in 12-2023.