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EPAR summary for the public

Aldurazyme

laronidase

This is a summary of the European public assessment report (EPAR) for Aldurazyme. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Aldurazyme.

What is Aldurazyme?

Aldurazyme is a solution for infusion (drip) into a vein that contains the active substance laronidase.

What is Aldurazyme used for?

Aldurazyme is used in patients with a confirmed diagnosis of mucopolysaccharidosis I (MPS I; α L iduronidase deficiency) to treat the non-neurological symptoms of the disease (symptoms that are not connected with the brain or nerves). MPS I is a rare, inherited disease, in which the level of α L iduronidase enzyme activity is much lower than normal. This means that substances called glycosaminoglycans (GAGs) are not broken down, so they build up in most of the organs in the body and damage them. The non-neurological symptoms of MPS I can be an enlarged liver, stiff joints that make moving more difficult, reduced lung volume, heart disease and eye disease.

The medicine can only be obtained with a prescription.

How is Aldurazyme used?

Aldurazyme treatment should be supervised by a doctor who has experience in the management of patients with MPS I or other inherited metabolic diseases. Aldurazyme should be given in a hospital or clinic where resuscitation equipment is available, and patients may need to receive some medicines before the infusion to prevent an allergic reaction. Aldurazyme is given once a week as an infusion into a vein. It is intended for long-term use.

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How does Aldurazyme work?

The active substance in Aldurazyme, laronidase, is a copy of the human enzyme α L iduronidase. It is produced by a method known as 'recombinant DNA technology': the enzyme is made by cells into which a gene (DNA) has been introduced that makes them able to produce laronidase. Laronidase is used as 'enzyme replacement therapy', which means that it replaces the enzyme that is missing in patients with MPS I. This controls the symptoms of MPS I, improving the patient's quality of life.

How has Aldurazyme been studied?

Aldurazyme has been compared with placebo (a dummy treatment) in 45 patients aged six years and over with a confirmed diagnosis of MPS I. The main measure of effectiveness was the forced vital capacity (FVC, a measure of how well the lungs are working) and the distance the patients could walk over six minutes. These were measured before and after 26 weeks of treatment. After this, the study continued for up to four years and all of the patients were treated with Aldurazyme.

Aldurazyme has also been studied in 20 children below the age of five years who received Aldurazyme for a year. The study was looking mainly at the safety of the medicine, but it also measured its ability to reduce the levels of GAGs in the urine and the size of the liver.

What benefit has Aldurazyme shown during the studies?

The study showed that Aldurazyme had improved both the FVC and the walking ability of patients at 26 weeks. This effect was maintained for up to four years.

In children under five years of age, Aldurazyme reduced the levels of GAGs in the urine by about 60%. Half of the children treated had a normal size liver at the end of the study.

What is the risk associated with Aldurazyme?

Most of the side effects seen with Aldurazyme are reactions caused by the infusion procedure rather than the medicine itself. Some of these are severe, but the number of side effects tends to decrease with time. The most common side effects in patients over the age of five years (seen in more than 1 patient in 10) are headache, nausea (feeling sick), abdominal pain (stomach ache), rash, arthropathy (damage to the joints), arthralgia (joint pain), back pain, pain in the extremities (hands and feet), flushing, pyrexia (fever) and reactions at the site of the infusion. In patients under five years of age, the most common side effects (seen in more than 1 patient in 10) are increased blood pressure, decreased oxygen saturation (a measure of how well the lungs are working), tachycardia (rapid heart rate), pyrexia and chills. For the full list of all side effects reported with Aldurazyme, see the package leaflet.

Almost all patients who receive Aldurazyme develop antibodies (proteins that are produced in response to Aldurazyme). The effect of these on the safety and effectiveness of the medicine is not fully known.

Aldurazyme must not be used in people who are severely allergic to laronidase or any of the other ingredients.

Why has Aldurazyme been approved?

The CHMP decided that Aldurazyme gives effective control of the symptoms of MPS I. The Committee decided that Aldurazyme's benefits are greater than its risks and recommended that Aldurazyme be given marketing authorisation.

Aldurazyme was originally authorised under 'exceptional circumstances', because, as the disease is rare, limited information was available at the time of approval. As the company had supplied the additional information requested, the 'exceptional circumstances' ended on 16 December 2015.

Other information about Aldurazyme

The European Commission granted a marketing authorisation valid throughout the European Union for Aldurazyme on 10 June 2003.

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

This summary was last updated in 11-2015.