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EPAR summary for the public

Elaprase

idursulfase

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

What is Elaprase?

Elaprase is a medicine that contains the active substance idursulfase. It is available as a concentrate that is made up into a solution for infusion (drip) into a vein.

What is Elaprase used for?

Elaprase is used to treat patients with Hunter syndrome. It is designed for long-term use.

Hunter syndrome, which is also known as mucopolysaccharidosis II, is a rare, inherited disease that primarily affects male patients. Patients with Hunter syndrome do not produce an enzyme called iduronate-2-sulfatase. This enzyme is needed to break down substances in the body called glycosaminoglycans (GAGs). Since patients with Hunter syndrome cannot break these substances down, the GAGs gradually build up in most of the organs in the body and damage them. This causes a wide range of symptoms, particularly difficulty breathing and difficulty walking. Without treatment, these symptoms become more severe over time.

Because the number of patients with Hunter syndrome is low, the disease is considered 'rare', and Elaprase was designated an 'orphan medicine' (a medicine used in rare diseases) on 11 December 2001.

The medicine can only be obtained with a prescription.

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How is Elaprase used?

Elaprase treatment should be supervised by a doctor or other healthcare professional who has experience in the management of patients with Hunter syndrome or other inherited diseases affecting the metabolism.

Elaprase is given every week, as an infusion into a vein, at a dose of 0.5 mg per kilogram body weight. The infusion should last three hours. However, as long as the patient does not develop infusion reactions (rash, itching, fever, headache, high blood pressure or flushing), the duration of the infusion can be gradually reduced to one hour.

Patients who tolerate the infusions well for several months in a clinic may be able to start having them at home. Home infusions should be supervised by a doctor or nurse.

How does Elaprase work?

The active substance in Elaprase, idursulfase, is a copy of the human enzyme iduronate-2-sulfatase. It replaces the enzyme that is missing or defective in patients with Hunter syndrome. Supplying the enzyme will help to break down GAGs and stop them building up in body tissue, thereby helping to improve the symptoms of the disease.

How has Elaprase been studied?

The main study of Elaprase involved 96 male patients aged between 5 and 31 years, and compared it with placebo (a dummy treatment). The main measures of effectiveness were lung function ('forced vital capacity', the maximum amount of air the patient could breathe out), and the distance the patients could walk in six minutes, which measures the combined effects of the illness on the heart, lungs, joints and other organs. These measurements were taken at the start of the study and after a year of treatment.

What benefit has Elaprase shown during the studies?

Elaprase improved lung function and the walking ability of the patients. At the start of the study, the patients could walk an average of around 395 metres in six minutes. After a year, the patients receiving Elaprase could walk a further 43 metres on average, and the patients receiving placebo could walk a further 8 metres. The medicine also produced an improvement in lung function, while the patients on placebo showed a slight worsening.

What is the risk associated with Elaprase?

The most common side effects with Elaprase are related to the infusion, including skin reactions (rash or itching), fever, headache, high blood pressure and flushing. Other side effects seen in more than 1 patient in 10 are wheezing, dyspnoea (difficulty breathing), abdominal pain (stomach ache), nausea (feeling sick), dyspepsia (heartburn), diarrhoea, vomiting, swelling at the site of infusion and chest pain. Severe allergic reactions have occurred in some patients taking Elaprase. For the full list of all side effects reported with Elaprase, see the package leaflet.

Elaprase must not be used in people who have had a severe or life-threatening allergic (anaphylactic) reaction to idursulfase or any of the other ingredients if their allergy is not controllable. If given to patients who have had severe allergic reactions in the past, Elaprase should be used with caution, and trained staff and equipment for emergency resuscitation should be available during infusion.

Why has Elaprase been approved?

The CHMP concluded that the improvements shown in the study, even if limited, represent a clinical benefit in the treatment of Hunter syndrome. The Committee decided that Elaprase's benefits are greater than its risks and recommended that it be given marketing authorisation.

Elaprase has been authorised under 'exceptional circumstances'. This means that because Hunter syndrome is rare, it has not been possible to obtain complete information about Elaprase. Every year, the European Medicines Agency will review any new information that may become available and this summary will be updated as necessary.

What information is still awaited for Elaprase?

The company that markets Elaprase will investigate the long-term effects of the medicine, particularly on the lungs, the heart and the blood vessels, and whether the body produces antibodies that could affect the medicine's safety and effectiveness.

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

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

Other information about Elaprase

The European Commission granted a marketing authorisation valid throughout the European Union for Elaprase on 8 January 2007.

The full EPAR for Elaprase 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 Elaprase, 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 Elaprase can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 09-2016.