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EPAR summary for the public

Naglazyme

galsulfase

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

What is Naglazyme?

Naglazyme is a solution for infusion (drip into a vein) that contains the active substance galsulfase (1 mg/ml).

What is Naglazyme used for?

Naglazyme is used to treat patients who have mucopolysaccharidosis VI (MPS VI or Maroteaux-Lamy syndrome). This disease is caused by the lack of an enzyme called N-acetylgalactosamine 4-sulfatase, which is needed to break down substances in the body called glycosaminoglycans (GAGs). If the enzyme is not present, GAGs cannot be broken down and they build up in the cells. This causes the signs of the disease, the most noticeable being a short body, a large head and difficulty moving about. The disease is usually diagnosed in infants between one and five years of age.

Because the number of patients with MPS VI is low, the disease is considered ‘rare’, and Naglazyme was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 14 February 2001.

The medicine can only be obtained with a prescription.

How is Naglazyme used?

Naglazyme treatment should be supervised by a doctor who has experience in the management of patients with MPS VI or similar diseases. It should be given where resuscitation equipment is available in case of a medical emergency.

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Naglazyme is given as a four-hour infusion once a week. The recommended dose is 1 mg per kilogram bodyweight. Before each infusion, patients should be given an antihistamine to reduce the risk of an allergic reaction. Patients may also be given a medicine to prevent fever.

How does Naglazyme work?

Naglazyme is an enzyme replacement therapy. Enzyme replacement therapy provides patients with the enzyme they are lacking. The active substance in Naglazyme, galsulfase, is a copy of the human enzyme N-acetylgalactosamine 4-sulfatase. Naglazyme helps to break down the GAGs and stop them building up in the cells. This can improve the symptoms of MPS VI, including the ability of patients to move about.

Galsulfase is produced by a method known as 'recombinant DNA technology': it is made by a cell that has received a gene (DNA), which makes it able to produce the enzyme.

How has Naglazyme been studied?

Naglazyme has been compared with placebo (a dummy treatment) in one main study involving 39 patients with MPS VI aged between five and 29 years. The main measure of effectiveness was how far the patients could walk after 24 weeks of treatment.

What benefit has Naglazyme shown during the studies?

Naglazyme was more effective than placebo. After 24 weeks, the average distance walked over 12 minutes increased by 109 metres in the patients treated with Naglazyme and by 18 metres in those receiving placebo.

What is the risk associated with Naglazyme?

In studies, the most common side effects with Naglazyme (seen in more than 1 patient in 10) were ear pain, dyspnoea (difficulty breathing), abdominal pain and general pain. Patients can also have reactions to the infusion (such as fever, chills, rash and hives). For the full list of all side effects reported with Naglazyme, see the package leaflet.

Naglazyme should not be used in people who may be hypersensitive (allergic) to galsulfase or any of the other ingredients.

Why has Naglazyme been approved?

The CHMP decided that Naglazyme's benefits are greater than its risks and recommended that it be given marketing authorisation.

The Committee noted that, although patients under five years of age were not included in the main study of Naglazyme, it is important that they are treated if they have a severe form of MPS VI.

Naglazyme has been authorised under 'exceptional circumstances'. This means that because the disease is rare, it has not been possible to obtain complete information about Naglazyme. 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 Naglazyme?

The company that makes Naglazyme is carrying out studies looking at the long-term safety and effectiveness of Naglazyme in pregnant and breast-feeding women and in children under the age of five years, to see if they develop antibodies (proteins that are produced in the body in response to Naglazyme that could affect the response to treatment) and to look at the medicine's side effects. The company is also carrying out studies to determine the best dose to give to patients on a regular long-term basis.

Other information about Naglazyme

The European Commission granted a marketing authorisation valid throughout the European Union for Naglazyme to BioMarin Europe Limited on 24 January 2006. The marketing authorisation is valid for an unlimited period.

The summary of the opinion of the Committee for Orphan Medicinal Products for Nagalzyme can be found on the Agency's website ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

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

This summary was last updated in 12-2010.