EMA/58201/2014
EMEA/H/C/000636

EPAR summary for the public

Myozyme

alglucosidase alfa

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

What is Myozyme?

Myozyme is a powder that is made up into a solution for infusion (drip into a vein). It contains the active substance alglucosidase alfa.

What is Myozyme used for?

Myozyme is used to treat patients who have Pompe disease, a rare inherited disorder. Patients with Pompe disease do not have enough of an enzyme called alpha-glucosidase. This enzyme normally breaks down sugar stored as glycogen into glucose that can be used for energy by the body's cells. If the enzyme is not present, glycogen builds up in certain tissues, particularly the muscles, including the heart and diaphragm (the main breathing muscle under the lungs). The progressive build-up of glycogen causes a wide range of symptoms, including an enlarged heart, breathing difficulties and muscle weakness. The disease can appear at birth (the 'infantile-onset' form) but also later in life (the 'late-onset' form).

Because the number of patients with Pompe disease is low, the disease is considered 'rare', and Myozyme was designated as an 'orphan medicine' (a medicine used in rare diseases) on 14 February 2001.

The medicine can only be obtained with a prescription.

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How is Myozyme used?

Myozyme treatment should be supervised by a doctor who has experience in the management of patients with Pompe disease or other inherited diseases of the same type.

Myozyme is given as an infusion of 20 mg per kilogram body weight given once every two weeks. The infusion should start slowly and then be gradually sped up as long as there are no signs of side effects caused by the infusion.

How does Myozyme work?

Myozyme is an enzyme replacement therapy. Enzyme replacement therapy provides patients with the enzyme they are lacking; in this case, alpha-glucosidase. The active substance in Myozyme, alglucosidase alfa, is a copy of human alpha-glucosidase, which is produced by a method known as 'recombinant DNA technology': the enzyme is made by a cell that has received a gene (DNA) that makes it able to produce the enzyme. The replacement enzyme helps to break down glycogen and stops it building up abnormally in the cells.

How has Myozyme been studied?

Myozyme has been studied in two main studies involving a total of 39 babies and children up to the age of three and a half years with infantile-onset Pompe disease. These patients were compared with a 'historical comparison group' of babies and young children with Pompe disease who had not received treatment and did not take part in the studies. The main measures of effectiveness were the number of patients who survived and the number who did not need a ventilator to help them breathe.

Myozyme has also been compared with placebo (a dummy treatment) in one main study involving 90 patients with late-onset disease. The main measures of effectiveness were the improvement in the distance the patients could walk in six minutes and in their 'forced vital capacity' (a measure of how well their lungs were working). The study lasted for up to 18 months.

What benefit has Myozyme shown during the studies?

In the first main study, which involved babies less than six months old, all 18 patients treated with Myozyme were alive at 18 months of age, and 15 of these did not need a ventilator to help them breathe. In contrast, only one of the 42 patients in the historical comparison group was alive at 18 months of age. The results were confirmed in the other study, which involved children aged between six months and three and a half years.

In late-onset disease, Myozyme was more effective than placebo at improving both the distance the patients could walk and their lung function over the course of the study.

What is the risk associated with Myozyme?

During the studies in patients with infantile-onset Pompe disease, the most common side effects with Myozyme (seen in more than 1 patient in 10) were tachycardia (rapid heartbeat), flushing (redness), cough, tachypnoea (rapid breathing), vomiting, urticaria (itchy rash), rash, pyrexia (fever) and decreased oxygen saturation (low oxygen levels in the blood). In the study of late-onset disease, patients had many of the same side effects, but they were seen less often than in studies of infantile-onset disease. Almost all of the side effects seen with Myozyme occurred during or just after the infusion and were mild or moderate. For the full list of all side effects reported with Myozyme, see the package leaflet.

Patients who receive Myozyme may develop antibodies (proteins that are produced in response to Myozyme). The effect of these antibodies on the safety and effectiveness of Myozyme is not yet clear.

Myozyme must not be used in people who have had a life-threatening anaphylactic (severe allergic) reaction to alglucosidase alfa or any of the other ingredients, which could not be managed by giving the medicine at a slower infusion rate and reduced dose.

Why has Myozyme been approved?

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

What measures are being taken to ensure the safe use of Myozyme?

The company that makes Myozyme is putting a plan in place to ensure that Myozyme is used safely, mainly by monitoring how patients who receive Myozyme develop antibodies, by setting up a register open to all patients with Pompe disease, and by ensuring that doctors know about the reactions that patients may have to the infusion.

Other information about Myozyme

The European Commission granted a marketing authorisation valid throughout the European Union for Myozyme to Genzyme Europe B.V. on 29 March 2006. The marketing authorisation is valid for an unlimited period.

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

This summary was last updated in 01-2014.