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Lamzede (*velmanase alfa*)

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

What is Lamzede and what is it used for?

Lamzede is a medicine used for patients with mild to moderate alpha-mannosidosis. It is used for treating effects of the disease that do not involve the brain (non-neurological effects).

Alpha-mannosidosis is an inherited disease with features that include learning disability, difficulty controlling movement, deafness, speaking difficulty, frequent infections, enlarged liver and spleen, bone abnormalities, and muscle pain and weakness.

Lamzede contains the active substance velmanase alfa.

Alpha-mannosidosis is rare, and Lamzede was designated an 'orphan medicine' (a medicine used in rare diseases) on 26 January 2005. Further information on the orphan designation can be found here: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

How is Lamzede used?

Lamzede can only be obtained with a prescription. Treatment should be supervised by a doctor experienced in treating alpha-mannosidosis or in giving enzyme replacement treatments for similar conditions.

Lamzede is given by infusion (drip) into a vein at a dose of 1 mg per kg of body weight once a week. The infusion is given over at least 50 minutes, using a pump to control the speed of infusion.

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

How does Lamzede work?

Patients with alpha-mannosidosis lack an enzyme called alpha-mannosidase which is important for breaking down certain glycosides (substances that contain proteins and sugars). As a result, certain oligosaccharides (types of sugar) build up in the body and cause damage. The active substance in Lamzede, velmanase alfa, acts in the same way as alpha-mannosidase. In this way, Lamzede prevents

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worsening of some body functions (such as breathing and movement) caused by the build-up of oligosaccharides.

What benefits of Lamzede have been shown in studies?

One main study involving 25 patients with alpha-mannosidosis found Lamzede more effective than placebo (a dummy treatment) at treating non-neurological effects of alpha-mannosidosis. Two main measures of effectiveness were used: a change in the level of oligosaccharides in patients' blood after a year of treatment and physical endurance measured as a change in the number of steps the patient could climb on a staircase. The levels of oligosaccharides decreased, on average, three times as much in patients receiving Lamzede compared with those receiving placebo. After treatment for one year, patients receiving Lamzede could climb about half a step more in 1 minute than they could previously, while patients receiving placebo could climb two steps less.

What are the risks associated with Lamzede?

The most common side effects with Lamzede (which may affect up to 1 in 10 people) are weight gain, infusion-related reactions (such as allergic reactions, nausea, vomiting, fever, chills, feeling unwell and itchiness), diarrhoea, headache, joint pain, pain in the arms and legs and increased appetite.

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

Why is Lamzede authorised in the EU?

The European Medicines Agency decided that Lamzede's benefits are greater than its risks and it can be authorised for use in the EU. Lamzede lowers the levels of oligosaccharides in the blood and it is better than placebo at slowing down the worsening of many disease effects. The Agency noted that the medicine does not appear to reach the brain and it does not improve effects such as loss of hearing or loss of control over movement. Lamzede's side effects are manageable.

Lamzede has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Lamzede 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.

What information is still awaited for Lamzede?

Since Lamzede has been authorised under exceptional circumstances, the company that markets the medicine will set up a registry of patients treated with Lamzede and provide additional information on the long-term effectiveness and safety of the medicine as well as the medicine's effects on variants of the condition. The company will also provide results of a 2-year study looking at the safety and effectiveness of Lamzede in children aged up to 6 years.

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

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

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

Other information about Lamzede

Lamzede received a marketing authorisation valid throughout the EU on 23 March 2018.

Further information on Lamzede can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports.

This overview was last updated in 03-2018.