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EPAR summary for the public

Vimizim

elosulfase alfa

This is a summary of the European public assessment report (EPAR) for Vimizim. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Vimizim.

For practical information about using Vimizim, patients should read the package leaflet or contact their doctor or pharmacist.

What is Vimizim and what is it used for?

Vimizim is a medicine that contains the active substance elosulfase alfa. It is used to treat patients with mucopolysaccharidosis type IVA (MPS IVA, also known as Morquio A syndrome). This disease is caused by the lack of an enzyme called N-acetylgalactosamine-6-sulfatase, which is needed to break down substances in the body called glycosaminoglycans (GAGs). If the enzyme is not present, or only present in very small quantities, GAGs cannot be broken down and they build up in bones and organs. This causes the signs of the disease, the most noticeable being short bones, difficulty moving and breathing, clouding of the eyes and hearing loss.

Because the number of patients with MPS IVA is low, the disease is considered ‘rare’, and Vimizim was designated an ‘orphan medicine’ (a medicine used in rare diseases) on 24 July 2009.

How is Vimizim used?

Treatment with Vimizim should be supervised by a doctor experienced in MPS IVA or similar diseases. The medicine can only be obtained with a prescription and should be given by an appropriately trained healthcare professional.

Vimizim is available as a concentrate to be made into a solution for infusion (drip) into a vein. The recommended dose is 2 mg per kilogram body weight, given once a week. The infusion should last

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around 4 hours. Before receiving Vimizim, the patient should be given a medicine to prevent an allergic reaction to Vimizim. Patients may also be given a medicine to prevent fever.

How does Vimizim work?

Vimizim is an enzyme replacement therapy. Enzyme replacement therapy provides patients with the enzyme they are lacking. The active substance in Vimizim, elosulfase alfa, is a copy of the human enzyme N-acetylgalactosamine-6-sulfatase. The replacement enzyme helps to break down GAGs and stop them building up in cells, thereby improving the symptoms of MPS IVA.

Elosulfase alfa is produced by a method known as 'recombinant DNA technology': it is made by cells into which a gene (DNA) has been introduced, which makes them able to produce the enzyme.

What benefits of Vimizim have been shown in studies?

Vimizim has been investigated in one main study involving 176 patients with MPS IVA, which compared Vimizim with placebo (a dummy treatment). The main measure of effectiveness was the change in the distance patients could walk in six minutes after 6 months of treatment.

Before treatment, the patients could walk on average just over 200 metres in six minutes. After 6 months, patients treated with the recommended dose of Vimizim could walk an extra 37 metres on average in six minutes, compared with an increase of 14 metres in patients receiving placebo. Study results also suggested that the medicine could improve how well patients breathe or climb stairs, and in children, how well they grew.

What are the risks associated with Vimizim?

The most common side effects with Vimizim (which may affect more than 1 in 10 people) are infusion-related reactions, including headache, nausea (feeling sick), vomiting, fever, chills and abdominal pain (stomach ache). These are usually mild or moderate and more frequent in the first 12 weeks of treatment. Serious but uncommon infusion-related reactions include anaphylaxis (severe allergic reaction). For the full list of all side effects reported with Vimizim, see the package leaflet.

Vimizim must not be used in patients who have experienced life-threatening allergic reactions to elosulfase alfa or any of the other ingredients in Vimizim.

Why is Vimizim approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Vimizim's benefits are greater than its risks and recommended that it be approved for use in the EU. The CHMP concluded that, following 6 months of treatment, Vimizim was shown to be effective at improving the distance patients could walk in six minutes, and this was accompanied by other beneficial effects, including improved ability to carry out daily activities. The Committee also considered that the safety profile of Vimizim appears manageable and serious side effects are uncommon, but further long-term safety data are to be collected.

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

A risk management plan has been developed to ensure that Vimizim is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and

the package leaflet for Vimizim, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company that makes Vimizim must ensure that all healthcare professionals expected to prescribe or use the medicine are provided with educational material, informing them of how the medicine should be used and the risk of severe allergic reactions. The company will also set up a registry to assess the long-term benefits and risks of Vimizim.

Further information can be found in the summary of the risk management plan.

Other information about Vimizim

The European Commission granted a marketing authorisation valid throughout the European Union for Vimizim on 28 April 2014.

The full EPAR and risk management plan summary for Vimizim 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 Vimizim, 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 Vimizim can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 04-2014.