20 February 2014
EMA/92845/2014
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Vimizim

Elosulfase alfa

On 20 February 2014, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Vimizim, 1 mg/ml concentrate for solution for infusion intended for treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA). Vimizim was designated as an orphan medicinal product on 24 July 2009. The applicant for this medicinal product is BioMarin Europe Ltd. They may request a re-examination of any CHMP opinion, provided they notify the European Medicines Agency in writing of their intention within 15 days of receipt of the opinion.

The active substance of Vimizim is elosulfase alfa, a recombinant human N-acetylgalactosamine-6-sulfatase, Other alimentary tract and metabolism products, enzymes; ATC code: A16AB12. Elosulfase alfa is intended to supplement the deficit enzyme N-acetylgalactosamine-6-sulfatase. After intravenous administration the enzyme will be taken up into the lysosomes and will increase the catabolism of the glycosaminoglycans (GAGs).

The benefits with Vimizim are its ability to improve patient's 6 minute walking test, and to improve other symptoms of Morquio disease, such as respiratory or anthropometric functions. The most common side effects are infusion reactions, including anaphylaxis, hypersensitivity and vomiting.

A pharmacovigilance plan for Vimizim will be implemented as part of the marketing authorisation.

The approved indication is: "Vimizim is indicated for the treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of all ages". It is proposed that Vimizim treatment should be supervised by a physician experienced in the management of patients with MPS IVA or other inherited metabolic diseases. Administration of Vimizim should be carried out by an appropriately trained healthcare professional with the ability to manage medical emergencies.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.

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made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

The CHMP, on the basis of quality, safety and efficacy data submitted, considers there to be a favourable benefit-to-risk balance for Vimizm and therefore recommends the granting of the marketing authorisation.