20 November 2014
EMA/CHMP/687220/2014
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Cerdelga

eliglustat

On 20 November 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 Cerdelga, 84 mg hard capsules intended for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs).

Cerdelga was designated as an orphan medicinal product on 4 December 2007. The applicant for this medicinal product is Genzyme Europe BV. 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 Cerdelga is eliglustat, a product belonging to various alimentary tract and metabolism products (ATC code: A16AX10).

Eliglustat is a potent and specific inhibitor of glucosylceramide synthase, and acts as a substrate reduction therapy (SRT) for GD1. SRT aims to reduce the rate of synthesis of the major substrate glucosylceramide (GL-1) to match its impaired rate of catabolism in patients with GD1, thereby preventing glucosylceramide accumulation and alleviating clinical manifestations.

The benefits with Cerdelga are its ability to decrease the plasma GL-1 levels that result in improvements in organ volume (spleen and liver volumes), haematological parameters (haemoglobin levels and platelet count) and skeletal parameters.

The most common side effects were headache, nausea, diarrhoea, abdominal pain, flatulence, arthralgia, fatigue.

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

[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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The approved indication is: "*Cerdelga is indicated for the long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PMs), intermediate metabolisers (IMs) or extensive metabolisers (EMs)*".

It is proposed that Cerdelga should be initiated and supervised by a physician knowledgeable in the management of Gaucher disease.

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 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 Cerdelga and therefore recommends the granting of the marketing authorisation.