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EMA/COMP/748368/2014
Committee for Orphan Medicinal Products

Recommendation for maintenance of orphan designation at the time of marketing authorisation

Cerdelga (eliglustat) for the treatment of Gaucher disease

During its meeting of 9 to 11 December 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/514 for Cerdelga (eliglustat)[1] as an orphan medicinal product for the treatment of Gaucher disease. The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. As other methods of treatment are authorised in the European Union (EU), the COMP also considered whether the medicine is of significant benefit to patients with Gaucher disease. The COMP recommended that the orphan designation of the medicine be maintained[2].

Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Cerdelga for:

'Long-term treatment of adult patients with Gaucher disease type 1 (GD1), who are CYP2D6 poor metabolisers (PM), intermediate metabolisers (IMs) or extensive metabolisers (EMs)'.

This falls within the scope of the product's designated orphan indication, which is: 'Gaucher disease'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2007. Gaucher disease remains a long-term debilitating and life-threatening condition that is associated with a reduced life expectancy if left untreated.

[1] Previously known as (1R,2R)-octanoic acid[2-(2',3'-dihydro-benzo[1,4] dioxin-6'-yl)-2-hydroxy-1-pyrrolidin-1-ylmethyl-ethyl]-amide-L-tartaric acid salt.
[2] The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with a comparable therapeutic indication cannot be placed on the market.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Prevalence of the condition

The sponsor provided updated information on the prevalence of Gaucher disease based on data from Orphanet (2013), the ICGG Gaucher registry and from the scientific literature. On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of Gaucher disease remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was still estimated to be approximately 0.3 people in 10,000. This is equivalent to a total of around 15,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, three medicines, Cerezyme (imiglucerase), Vpriv (velaglucerase alfa) and Zavesca (miglustat), were authorised for the treatment of Gaucher disease in the EU. Cerezyme and Vpriv are 'enzyme replacement therapies' that work by replacing the missing enzyme. Zavesca is used as a second-line treatment in Gaucher disease patients who cannot receive enzyme replacement therapy.

Significant benefit of Cerdelga

The COMP concluded that the claim of a significant benefit of Cerdelga over enzyme replacement therapies is justified on the basis of its major contribution to patients' care. This is because Cerdelga is to be taken by mouth, which is more convenient for patients with long-term disease than enzyme replacement therapies, which are given as a drip into a vein.

Comparing Cerdelga with Zavesca, the COMP noted that Zavesca can only be used in patients who cannot receive enzyme replacement therapy.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Cerdelga is of significant benefit to patients affected by Gaucher disease.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Cerdelga still meets the criteria for designation as an orphan medicinal product and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Cerdelga can be found in the European public assessment report (EPAR) on the Agency's website ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports.