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Committee for Orphan Medicinal Products

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

Vpriv (velaglucerase alfa) for the treatment of Gaucher disease

During its meeting of 7-8 July 2010, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/10/752 for Vpriv (velaglucerase alfa) 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 satisfactory methods of treatment. As other satisfactory methods of treatment for patients with this condition are authorised in the European Union (EU), the COMP also looked at the significant benefit of the product over existing treatments. The COMP recommended that the orphan designation of the medicine be maintained[1].

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

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Vpriv for: 'long-term enzyme replacement therapy (ERT) in patients with type 1 Gaucher disease'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in June 2010. Gaucher disease remains a condition that is debilitating in the long term and life threatening because it is associated with a reduced life expectancy.

Prevalence of the condition

The sponsor informed the COMP that no changes to the prevalence of the condition had been reported since the orphan designation of Vpriv.

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 threshold for orphan designation,

[1] 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.

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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 satisfactory methods of treatment

At the time of the review of the orphan designation, two medicines, Cerezyme (imiglucerase) and Zavesca (miglustat), were authorised for the treatment of Gaucher disease in the EU. Cerezyme is an enzyme replacement therapy that works by substituting the missing enzyme. Zavesca is an orphan medicine that blocks the production of glucocerebroside and is used as a second-line treatment in patients who cannot receive enzyme replacement therapy.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Vpriv over Cerezyme is justified on the basis of its potential major contribution to patient care in light of the current unsatisfactory supply situation for Cerezyme. For patients diagnosed with type 1 Gaucher disease, Vpriv can represent an alternative treatment to Cerezyme, should the supply problems that are occurring with this medicine continue or happen again in the future.

The current and persistent shortage of Cerezyme has obliged the CHMP to recommend temporary changes to the treatment recommendations for Gaucher's disease that are not in line with the prescribing information of this medicine. An alternative enzyme replacement therapy such as Vpriv that has demonstrated non-inferiority in clinical trials in terms of efficacy can bring a significant benefit to those patients who under the current situation have had to receive suboptimal treatment as a result of the Cerezyme shortage.

Comparing Vpriv with Zavesca, the COMP noted that Zavesca has a different mechanism of action and can only be used in patients who cannot receive enzyme replacement therapy.

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

Conclusions

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

Further information on the current regulatory status of Vpriv 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.