20 December 2011
EMA/COMP/729178/2011
Committee for Orphan Medicinal Products

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

Vyndaqel (N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichloro-phenyl)-benzoxazole-6-carboxylate) for treatment of familial amyloid polyneuropathy

During its meeting of 6–8 September 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/06/401 for Vyndaqel (N-methyl D-(2,3,4,5,6-pentahydroxy-hexyl)-ammonium; 2-(3,5-dichloro-phenyl)-benzoxazole-6-carboxylate) as an orphan medicinal product for the treatment of familial amyloid polyneuropathy. 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 exist 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 Vyndaqel for:

'the treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment'.

This falls within the scope of the product's designated orphan indication(s), which is: 'treatment of familial amyloid polyneuropathy'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2006. Familial amyloid polyneuropathy remains a condition that is debilitating in

[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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the long term and life threatening, particularly due to the progressive loss of the functions of nervous system causing symptoms such as loss of sensation and muscle weakness.

Prevalence of the condition

The sponsor provided updated prevalence data for familial amyloid polyneuropathy. On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of familial amyloid polyneuropathy 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 less than 0.1 people in 10,000. This is equivalent to a total of less than 5,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, the only available treatment in the EU for the condition was liver transplantation.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Vyndaqel in familial amyloid polyneuropathy is justified by the fact that it is the first medicine for this condition authorised in the EU which targets the disease thereby providing an additional treatment option.

Therefore, although other satisfactory methods for the treatment of this condition exist in the EU, the COMP concluded that Vyndaqel is of significant benefit for patients affected by familial amyloid polyneuropathy.

Conclusions

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

Further information on the current regulatory status of Vyndaqel 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.