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EMA/COMP/10667/2011
Committee for Orphan Medicinal Products

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

Orphacol (cholic acid) for the treatment of inborn errors in primary bile acid synthesis

During its meeting of 11-12 January 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/02/127 for Orphacol (cholic acid) as an orphan medicinal product for the treatment of inborn errors in primary bile acid synthesis. 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. 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 Orphacol for:

'the treatment of inborn errors in primary bile acid synthesis due to 3β-hydroxy-Δ^5-C27-steroid oxidoreductase deficiency or Δ^4-3-oxosteroid-5β-reductase deficiency in infants, children and adolescents aged 1 month to 18 years and adults'.

This falls within the scope of the product's designated orphan condition, which is: 'treatment of inborn errors in primary bile acid synthesis'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2002. Inborn errors in primary bile acid synthesis remain a condition that is debilitating in the long term and life threatening, particularly due to the development of liver cirrhosis and liver failure.

[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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Prevalence of the condition

The sponsor provided recent data on the prevalence of the orphan condition, inborn errors in primary bile acid synthesis, from scientific literature published since the orphan designation of Orphacol in 2002.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of this condition 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 of inborn errors in primary bile acid synthesis was estimated to be between 0.06 and 0.07 people in 10,000. This is equivalent to a total of between 3,000 and 3,500 people in the EU.

Existence of other satisfactory methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no satisfactory treatments were authorised in the EU for patients affected by this condition.

Conclusions

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

Further information on the current regulatory status of Orphacol 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.