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Committee for Orphan Medicinal Products

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

Ocaliva (obeticholic acid) for the treatment of primary biliary cirrhosis

On 14 October 2016, the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/10/753 for Ocaliva (obeticholic acid[1]) as an orphan medicinal product for the treatment of primary biliary cirrhosis. 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 primary biliary cirrhosis. 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 Ocaliva for:

'treatment of primary biliary cholangitis (also known as primary biliary cirrhosis) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA or as monotherapy in adults unable to tolerate UDCA'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of primary biliary cirrhosis'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Primary biliary cirrhosis remains a condition that is debilitating in the long-term and life-threatening because, when the disease progresses, it may lead to scarring and liver failure, and may increase the risk of liver cancer.

[1] Previously known as 6alpha-ethyl-chenodeoxycholic acid.
[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 the same therapeutic indication cannot be placed on the market.

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Prevalence of the condition

The sponsor provided updated information on the prevalence of primary biliary cirrhosis based on data from the published literature.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of primary biliary cirrhosis 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 concluded to be 3.9 people in 10,000. This is equivalent to a total of around 200,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, ursodeoxycholic acid (UDCA) was authorised in the EU for the treatment of primary biliary cirrhosis.

Significant benefit of Ocaliva

The COMP concluded that the claim of a significant benefit of Ocaliva in primary biliary cirrhosis is justified on the basis of the results of a clinical study in patients with the condition, which showed that Ocaliva can reduce the blood levels of bilirubin and ALP (markers of liver damage) in patients who do not respond adequately or cannot take UDCA. This is considered an indication of improvement of liver function.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Ocaliva is of significant benefit to patients affected by primary biliary cirrhosis.

Conclusions

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

Further information on the current regulatory status of Ocaliva 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.