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EMA/COMP/536533/2013
Committee for Orphan Medicinal Products

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

Defitelio (defibrotide) for the treatment of hepatic veno-occlusive disease

During its meeting of 3 to 4 September 2013, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/04/212 for Defitelio (defibrotide) as an orphan medicinal product for the treatment of hepatic veno-occlusive 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. 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 Defitelio for:

'the treatment of severe hepatic veno-occlusive disease (VOD) also known as sinusoidal obstructive syndrome (SOS) in haematopoietic stem-cell transplantation (HSCT) therapy'.

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

'treatment of hepatic veno-occlusive disease'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2004. Hepatic veno-occlusive disease remains a condition that is debilitating in the long term or life threatening, particularly as it is associated with high mortality and can lead to multi-organ 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 new data that were not available at the time of the orphan designation in 2004. These included data from a recently published study on the proportion of patients undergoing haematopoietic stem-cell transplantation who develop hepatic veno-occlusive disease.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of hepatic veno-occlusive 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 estimated to be approximately 0.07 people in 10,000. This is equivalent to a total of around 3,600 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 Defitelio still meets the criteria for designation as an orphan medicinal product and that Defitelio should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Defitelio 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.