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EMA/COMP/684822/2013
Committee for Orphan Medicinal Products

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

Opsumit (macitentan) for the treatment of pulmonary arterial hypertension

During its meeting of 5-6 November 2013, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/11/909 for Opsumit (macitentan) as an orphan medicinal product for the treatment of pulmonary arterial hypertension. 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 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 Opsumit, as monotherapy or in combination, for the long-term treatment of pulmonary arterial hypertension (PAH) in adult patients of WHO Functional Class (FC) II to III.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of pulmonary arterial hypertension (PAH)'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2011. PAH remains a long-term debilitating and life-threatening condition that shortens patients' life expectancy because it leads to heart failure and difficulty in breathing that worsens over time.

[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

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of PAH 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 1.8 people in 10,000. This is equivalent to a total of less than 92,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, several medicines were authorised for the treatment of pulmonary arterial hypertension in the EU. They included ambrisentan, bosentan, epoprostenol, iloprost, sildenafil, tadalafil, and treprostinil.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Opsumit in the treatment of PAH is justified on the basis of data from a main clinical study in 742 patients. The study showed a reduction of PAH-related illness (including serious heart and lung problems or worsening of PAH symptoms) and of death in patients already treated with currently authorised treatments.

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

Conclusions

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

Further information on the current regulatory status of Opsumit 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.