5 November 2015
EMA/582963/2015
Committee for Orphan Medicinal Products

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

Cresemba (isavuconazole) for the treatment of mucormycosis

During its meeting of 1 to 3 September 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/14/1276 for Cresemba (isavuconazole) as an orphan medicinal product for the treatment of mucormycosis. 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 mucormycosis. 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 Cresemba for the treatment of: 'mucormycosis in patients for whom amphotericin B is inappropriate.'

This falls within the scope of one of the product's designated orphan indications, which is: 'treatment of mucormycosis'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2014. Mucormycosis, a fungal infection, remains a condition that is debilitating in the long term or life threatening, because the fungi can invade the surrounding blood vessels causing blood clots and cause death of the infected tissue in affected organs.

Prevalence of the condition

The sponsor provided updated information on the prevalence of mucormycosis based on data from the published literature.

[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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On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of mucormycosis 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 approximately 0.06 people in 10,000. This is equivalent to a total of around 3,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the treatment of mucormycosis, including amphotericin B.

Significant benefit of name of product

The COMP concluded that the claim of a significant benefit of Cresemba in mucormycosis is justified on the basis of data supporting the use of the medicine in patients who are not eligible for treatment with amphotericin B.

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

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Cresemba 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 Cresemba 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.