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Committee for Orphan Medicinal Products

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

Deltyba (delamanid) for the treatment of tuberculosis

During its meeting of 11 to 12 March 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/524 for Deltyba (delamanid)[1] as an orphan medicinal product for the treatment of tuberculosis. 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 for patients with tuberculosis 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 Deltyba be maintained[2].

Life-threatening or chronically debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Deltyba for:

'use as part of an appropriate combination regimen for pulmonary multi-drug resistant tuberculosis in adult patients when an effective treatment regimen cannot otherwise be composed for reasons of resistance or tolerability.'

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2008. Tuberculosis remains a condition that is debilitating in the long term or life threatening, particularly due to irreversible lung damage and the presence of drug resistant tuberculosis bacteria.

[1] Previously known as (R)-2-Methyl-6-nitro-2-{4-[4-(4-trifluoromethoxyphenoxy)piperidin-1-yl]phenoxymethyl}-2,3-dihydroimidazo[2,1-b]oxazole

[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 a comparable 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 tuberculosis based on data from the World Health Organization (WHO) and the United Nations.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of tuberculosis in the EU 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 in the EU was still estimated to be approximately 2 people in 10,000. This is equivalent to a total of around 102,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, several other products were authorised in the EU for the treatment of tuberculosis either as combinations (e.g. ethambutol/isoniazid, isoniazide/rifampin and isoniazide/rifampin/pyrazinamide) or as products containing only one active substance (e.g. ethambutol, isoniazide, rifampin, pyrazinamide and streptomycin).

Significant benefit over existing treatments

The COMP was of the opinion that the data provided sufficiently demonstrate that Deltyba is of significant benefit to patients affected by multi-drug resistant tuberculosis when added to a standard background treatment. The main study showed that adding Deltyba to standard treatment shortened the time needed for the tuberculosis bacteria to clear from the patients' sputum after two months of treatment. The COMP considered that these effects are likely to be sustained if Deltyba is used for the full treatment duration of six months therefore resulting in better survival and lower rates of relapse. This was supported by additional data from multi-drug resistant tuberculosis patients who continued to receive Deltyba for up to six months and who showed sustained negative sputum results after 24 months and better survival rates compared with patients who did not receive six months treatment with Deltyba. Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Deltyba is of significant benefit for patients affected by multi-drug resistant tuberculosis.

Conclusions

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

Further information on the current regulatory status of Deltyba 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.