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EMA/COMP/3833/2014
Committee for Orphan Medicinal Products

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

Sirturo (bedaquiline) for the treatment of tuberculosis

During its meeting of 7 to 9 January 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/05/314 for Sirturo (bedaquiline)[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 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[2].

Life-threatening or long-term debilitating nature of the condition

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

'use as part of an appropriate combination regimen for pulmonary multidrug 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 2005. Tuberculosis remains a condition that is debilitating in the long term and life threatening, particularly due to irreversible lung damage and the presence of drug resistant tuberculosis bacteria.

[1] Previously known as (1R,2S)6-bromo-alpha-[2-(dimethylamino)ethyl]-2-methoxy-alpha-(1-naphthyl)-beta-phenyl-3-quinolineethanol.
[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 from tuberculosis surveillance in Europe from the European Centre for Disease Control (ECDC) and the World Health Organization (WHO).

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 was still estimated to be approximately 2 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 concluded that the claim of a significant benefit of Sirturo in tuberculosis is justified on the basis of clinical study data on the effect of adding Sirturo to standard tuberculosis medicines in adults with multidrug resistant tuberculosis. The data showed that adding Sirturo to standard treatment shortened the time needed for the tuberculosis bacteria to clear from the patients' sputum (83 days versus 125 days in patients not given Sirturo). In addition, more patients who were given Sirturo in addition to standard treatments had negative sputum results after 24 weeks compared with those who were not given Sirturo (79% versus 58%) and the negative sputum results were sustained.

The COMP considered that the shorter time for the bacteria to clear from the sputum indicates that adding Sirturo to other treatments helps cure patients faster while also lowering the risk of the bacteria being transmitted to other people.

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

Conclusions

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

Further information on the current regulatory status of Sirturo 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.