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Committee for Orphan Medicinal Products

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

Tepadina (thiotepa) for the conditioning treatment prior to haematopoietic progenitor cell transplantation

During its meeting of 5-6 January 2010, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/06/424 for Tepadina (thiotepa) as an orphan medicinal product for the conditioning treatment prior to haematopoietic progenitor cell transplantation. 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 satisfactory 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 Tepadina for use:

'in combination with other chemotherapy medicinal products:

1) with or without total body irradiation (TBI), as conditioning treatment prior to allogeneic or autologous haematopoietic progenitor cell transplantation (HPCT) in haematological diseases in adult and paediatric patients;

2) when high dose chemotherapy with HPCT support is appropriate for the treatment of solid tumours in adult and paediatric patients'.

This falls within the scope of the product's designated orphan indication, which is: 'conditioning treatment prior to haematopoietic progenitor cell transplantation'.

[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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The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2007. Haematopoietic progenitor cell transplantation, a treatment procedure which is performed in diseases such as acute lymphoblastic leukaemia, myelogenous leukaemia, myelodysplatic syndromes, malignant lymphomas, multiple myelomas and aplastic anaemia, is life threatening due to the underlying primary diseases and the complications that can occur during transplantation.

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 number of patients who undergo conditioning treatment prior to haematopoietic progenitor cell transplantation remains below the threshold 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 0.6 people in 10,000. This is equivalent to a total of around 30,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the conditioning treatment prior to haematopoietic progenitor cell transplantation. This included the orphan medicine Busilvex.

Significant benefit over existing treatments

Overall, the COMP concluded that the claim of a significant benefit of Tepadina as a conditioning treatment prior to haematopoietic progenitor cell transplantation is justified on the basis of a clinically relevant advantage. This is supported by the fact that Tepadina has a different safety profile to currently authorised treatments. Products for conditioning treatment are known to have serious adverse effects, and the Committee therefore considered that an alternative treatment with a different safety profile would be of significant benefit to patients.

The Committee also considered that the product might be used in combination with existing treatments, to improve the overall outcome of patients with this condition.

In conclusion, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Tepadina is of significant benefit for patients undergoing conditioning treatment prior to haematopoietic progenitor cell transplantation.

Conclusions

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

Further information on the current regulatory status of Tepadina 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.