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EMA/COMP/399837/2015
Committee for Orphan Medicinal Products

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

Unituxin (dinutuximab) for the treatment of neuroblastoma

During its meeting of 16 to 18 June 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/11/879 for Unituxin (dinutuximab) for the treatment of neuroblastoma. 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 neuroblastoma. 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 Unituxin for:

'the treatment of high-risk neuroblastoma in patients aged 12 months to 17 years, who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and autologous stem cell transplantation (ASCT). It is administered in combination with granulocyte-macrophage colony-stimulating factor (GM-CSF), interleukin-2 (IL-2), and isotretinoin.'

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2011. Neuroblastoma remains a condition that is debilitating in the long term and associated with poor overall survival.

[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

The sponsor performed a search of the scientific literature and concluded that no publications are available which suggest a change in prevalence of neuroblastoma.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of neuroblastoma 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 1.1 people in 10,000. This is equivalent to a total of around 56,000 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 neuroblastoma. Treatments included surgery, radiotherapy (treatment with radiation), chemotherapy (medicines to treat cancer) and blood stem-cell transplantation.

Significant benefit of Unituxin

The claim of a significant benefit of Unituxin is based on data showing that the use of Unituxin in maintenance treatment improves survival in previously treated patients with high-risk neuroblastoma. Therefore, although other methods for the treatment of neuroblastoma have been authorised in the EU, the COMP concluded that Unituxin is of significant benefit to patients affected by this condition.

Conclusions

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

Further information on the current regulatory status of Unituxin 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.