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

Dinutuximab beta Apeiron (dinutuximab beta) for the treatment of neuroblastoma

On 18 April 2017, the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/12/1062 for Dinutuximab beta Apeiron (dinutuximab beta, previously known as chimeric monoclonal antibody against GD2) as an orphan medicinal product 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 Dinutuximab beta Apeiron for 'treatment of high-risk neuroblastoma in patients aged 12 months and above, who have previously received induction chemotherapy and achieved at least a partial response, followed by myeloablative therapy and stem cell transplantation, as well as patients with history of relapsed or refractory neuroblastoma, with or without residual disease'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2012. Neuroblastoma remains a debilitating condition and is life-threatening when the cancer spreads to other parts of the body.

[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 the same 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 57,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, several chemotherapy medicines were authorised in the EU for the treatment of neuroblastoma, including cyclophosphamide, doxorubicin, melphalan and vincristine. Treatments for neuroblastoma also included surgery, radiotherapy (treatment with radiation), and haematopoietic stem cell transplantation (a transplant of blood-producing cells).

Significant benefit of Dinutuximab beta Apeiron

The COMP concluded that the claim of a significant benefit of Dinutuximab beta Apeiron in neuroblastoma is justified because in studies the medicine improved survival in neuroblastoma patients whose cancer did not improve or came back after other cancer treatments.

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

Conclusions

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

More information on the COMP assessment can be found in the March 2017 COMP minutes.

Further information on Dinutuximab beta Apeiron 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.