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Committee for Orphan Medicinal Products

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

Blincyto (blinatumomab) for the treatment of acute lymphoblastic leukaemia

During its meeting of 6 to 8 October 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/09/650 for Blincyto (blinatumomab) as an orphan medicinal product for the treatment of acute lymphoblastic leukaemia. 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 acute lymphoblastic leukaemia. 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 Blincyto for:

'adults with Philadelphia chromosome negative relapsed or refractory B-precursor acute lymphoblastic leukaemia'.

This falls within the scope of the product's designated orphan indication, which is: 'acute lymphoblastic leukaemia'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Acute lymphoblastic leukaemia remains a condition that is life threatening, particularly due to a poor long-term prognosis if the cancer comes back after treatment.

Prevalence of the condition

The sponsor provided updated information on the prevalence of acute lymphoblastic leukaemia based on data from cancer registries (EUCAN 2012, GLOBOCAN 2012 and NORDCAN 2012).

[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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On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of acute lymphoblastic leukaemia 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 estimated to be 1.8 people in 10,000. This is equivalent to a total of 92,000 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 acute lymphoblastic leukaemia, including clofarabine, dasatinib, imatininb, ponatinib and xaluprine mercaptopurine.

Significant benefit of Blincyto

The COMP concluded that the claim of a significant benefit of Blincyto in acute lymphoblastic leukaemia is justified because Blincyto has been shown to improve the outcome of a subset of patients with the disease for whom standard treatments do not usually work. The patients in this subset are Philadelphia chromosome negative (Ph-), which means that they have acute lymphoblastic leukaemia but do not have an abnormal rearrangement of their genes called the Philadelphia chromosome that is seen in some other patients with the condition.

This is based on a main study in 189 patients with Ph- B-cell acute lymphoblastic leukaemia whose disease had come back after, or had not responded to, previous treatment, and which showed that 42.9% (81 out of 189) of patients given Blincyto responded to treatment.

Therefore, although other methods for the treatment of acute lymphoblastic leukaemia have been authorised in the EU, the COMP concluded that Blincyto is of significant benefit to patients with this condition.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Blincyto 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 Blincyto 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.