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EMA/COMP/345188/2014
Committee for Orphan Medicinal Products

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

Gazyvaro (obinutuzumab) for the treatment of chronic lymphocytic leukaemia

During its meeting of 10 to 12 June 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/12/1054 for Gazyvaro (obinutuzumab) as an orphan medicinal product for the treatment of chronic lymphocytic leukaemia (CLL). 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 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 CLL. 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 Gazyvaro for:

'treatment, in combination with chlorambucil, of adult patients with previously untreated chronic lymphocytic leukaemia and with co morbidities making them unable to tolerate a full-dose fludarabine based therapy'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2012. CLL remains a condition that is debilitating in the long term and life threatening, because patients may develop severe infections.

[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 provided updated information on the prevalence of CLL based on the most recent leukaemia IARC data (GLOBOCAN 2008) and data from the UK Office of National Statistics.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of CLL 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 approximately 3 people in 10,000. This is equivalent to a total of around 153,000 people in the EU.

Existence of other methods of treatment

The COMP noted that, at the time of the review of the orphan designation, the main treatment for CLL was chemotherapy (medicines to treat cancer), which might include chlorambucil or fludarabine-based therapy.

Significant benefit of Gazyvaro

The COMP concluded that the claim of a significant benefit of Gazyvaro in CLL is justified on the basis of it being more effective than existing treatments at improving the progression-free survival (the time patients lived without their disease getting worse) of previously untreated CLL patients.

This is based on a main study that showed that the average progression-free survival in patients treated with Gazyvaro plus chlorambucil (26.7 months) was significantly longer than the average progression-free survival with chlorambucil alone (11.1 months) or with chlorambucil plus rituximab (15.2 months).

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

Conclusions

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

Further information on the current regulatory status of Gazyvaro 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.