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EMA/COMP/430552/2010
Committee for Orphan Medicinal Products

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

Arzerra (ofatumumab) for the treatment of chronic lymphocytic leukaemia

During its meeting of 2-3 February 2010, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/08/581 for Arzerra (ofatumumab) as an orphan medicinal product for the treatment of chronic lymphocytic leukaemia (CLL). The COMP reviewed 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 Arzerra for:

'the treatment of chronic lymphocytic leukaemia (CLL) in patients who are refractory to fludarabine and alemtuzumab'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2008. CLL remains a condition that is chronically debilitating and life threatening, in particular due to poor long-term survival in high-risk patients.

Prevalence of the condition

The sponsor informed the COMP that no changes to the prevalence of the condition had been reported since the orphan designation of Arzerra in 2008.

[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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 threshold 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 3.5 people in 10,000. This is equivalent to a total of around 177,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, other treatments such as alemtuzumab and fludarabine were authorised in the EU for the treatment of CLL.

Significant benefit over existing treatments

Overall, the COMP concluded that the significant benefit of Arzerra in CLL is justified on the basis of a clinically relevant advantage, particularly in patients who are refractory to fludarabine and alemtuzumab. This is supported by data from clinical studies, which showed that the overall response rate was 58% in CLL patients refractory to fludarabine and alemtuzumab, and 47% in CLL patients refractory to fludarabine and with bulky lymphadenopathy.

In conclusion, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Arzerra is of significant benefit for patients with CLL.

Conclusions

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

Further information on the current regulatory status of Arzerra 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.