18 May 2016
EMA/COMP/261865/2016
Committee for Orphan Medicinal Products

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

Empliciti (elotuzumab) for the treatment of multiple myeloma

On 8 April 2016, the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/12/1037 of Empliciti (elotuzumab) as an orphan medicinal product for the treatment of multiple myeloma. 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 multiple myeloma. The COMP concluded that one of the criteria for orphan designation was no longer met and therefore recommended that the orphan designation of the product should not be maintained[1].

Life-threatening or chronically debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Empliciti with the following indication:

'Empliciti is indicated in combination with lenalidomide and dexamethasone for the treatment of multiple myeloma in adult patients who have received at least one prior therapy.'

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2012. Multiple myeloma remains a condition that is debilitating in the long term and life threatening, particularly because it disrupts the normal functioning of the bone marrow, leads to bone destruction and causes kidney failure.

[1] The removal of the orphan designation at time of marketing authorisation means that the product cannot benefit from 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 can be placed on the market.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

Prevalence of the condition

The sponsor provided updated information on the prevalence of multiple myeloma based on data from GLOBOCAN 2012, an international web portal for cancer research.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of multiple myeloma 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 less than 4 people in 10,000. This is equivalent to a total of fewer than 205,000 people in the EU.

Existence of other methods of treatments

At the time of the review of the orphan designation, bortezomib, carfilzomib, doxorubicin and lenalidomide were authorised in the EU for the treatment of patients with multiple myeloma who had received at least one previous treatment ('second-line therapy'). Carfilzomib, an orphan medicine, was the most recent to have received marketing authorisation.

Significant benefit of Empliciti

The COMP concluded that the claim of a significant benefit of Empliciti in multiple myeloma is no longer justified. In indirect comparisons between Empliciti and carfilzomib, 2-year survival was similar with both treatments (at around 73%) and overall survival did not appear to be greater with Empliciti. In addition, analyses of outcomes in different subgroups of patients did not conclusively show a better efficacy or safety with Empliciti.

Therefore, the COMP concluded that Empliciti did not meet the criteria for significant benefit assumed at the time of the original orphan designation.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP concluded that Empliciti no longer meets one of the criteria for designation as an orphan medicinal product. Therefore, the COMP recommended that the product should be removed from the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Empliciti 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.