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EMA/COMP/656221/2015
Committee for Orphan Medicinal Products

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

Kyprolis (carfilzomib) for the treatment of multiple myeloma

During its meeting of 6 to 8 October 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/08/548 for Kyprolis (carfilzomib) 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 meyloma. 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 Kyprolis for:

treatment, in combination with lenalidomide and dexamethasone, of adult patients with multiple myeloma who have received at least one prior therapy.

This 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 2008. Multiple myeloma remains a debilitating and life-threatening condition because it disrupts the normal functioning of the bone marrow, leads to bone destruction and causes kidney failure.

Prevalence of the condition

The sponsor provided updated information on the prevalence of multiple myeloma based on data from the GLOBOCAN 2012 database.

[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 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 approximately 3.3 people in 10,000. This is equivalent to a total of around 169,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, bortezomib, doxorubicin and lenalidomide were authorised in the EU for the treatment of multiple myeloma in patients who had received a previous treatment ('second-line therapy'), as is the case for Kyprolis.

Significant benefit of Kyprolis

The COMP concluded that the claim of a significant benefit of Kyprolis in multiple myeloma is justified because data show that, in patients who received at least one prior therapy, Kyprolis is more effective at improving the progression-free survival (how long the patients lived without their disease getting worse) than bortezomib, doxorubicin or lenalidomide. This is based on a study directly comparing Kyprolis with bortezomib, and on indirect comparisons versus doxorubicin or lenalidomide.

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

Conclusions

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