14 September 2015
EMA/COMP/470322/2015
Committee for Orphan Medicinal Products

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

Farydak (panobinostat) for the treatment of multiple myeloma

During its meeting of 14 to 16 July 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/12/1063 for Farydak (panobinostat) as an orphan medicinal product for the treatment of multiple myeloma (also known as plasma cell 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 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 Farydak for:

'Farydak, in combination with bortezomib and dexamethasone, is indicated for the treatment of adult patients with relapsed and/or refractory multiple myeloma who have received at least two prior regimens including bortezomib and an immunomodulatory agent'.

This falls within the scope of the product's designated orphan indication, which is: '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 debilitating and life-threatening disease because it disrupts the normal functioning of the bone marrow, leads to bone destruction and causes kidney failure.

[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 multiple myeloma based on data from the scientific literature and the EUCAN and NORDCAN databases.

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 170,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, several medicines were authorised for multiple myeloma in the EU, including various medicines for cancer such as bortezomib, and immunomodulatory agents (thalidomide, lenalidomide and pomalidomide). They were usually combined with steroids to reduce the activity of the immune system, the body's natural defences. Where these medicines did not work, some patients received an allogeneic stem-cell transplant (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

Significant benefit of Farydak

The COMP concluded that the claim of a significant benefit of Farydak in multiple myeloma is justified because Farydak has been shown to improve progression-free survival (how long the patients lived without their disease getting worse) of patients whose multiple myeloma had come back or got worse after previous treatments including bortezomib and immunomodulatory agents. These patients have limited treatment options and, therefore, a high unmet medical need.

The COMP conclusions are supported by data from a main study in multiple myeloma patients which showed that in those patients who had previously received at least two previous treatments, including bortezomib and an immunomodulatory medicine (such as thalidomide or lenalidomide), progression-free survival was 12.5 months with Farydak, versus about 5 months with placebo.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Farydak 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 Farydak still meets the criteria for designation as an orphan medicinal product and that Farydak should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Farydak 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.