7 December 2016
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Committee for Orphan Medicinal Products

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

Ninlaro (ixazomib) for the treatment of multiple myeloma

During its meeting of 4 to 6 October 2016, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/11/899 for Ninlaro (ixazomib[1]) 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 recommended that the orphan designation of the medicine be maintained[2].

Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Ninlaro in combination with lenalidomide and dexamethasone for the treatment 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 2011. Multiple myeloma remains a debilitating and life-threatening disease because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

[1] Previously known as 2,2'-{2-[(1R)-1-({[(2,5-dichlorobenzoyl)amino]acetyl}amino)-3-methylbutyl]-5-oxo-1,3,2-dioxaborolane-4,4-diyl}diacetic acid.
[2] 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 the same therapeutic indication cannot 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 the scientific literature and the Globocan 2012 database.

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 treatment

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

Significant benefit of Ninlaro

The COMP concluded that the claim of a significant benefit of Ninlaro in multiple myeloma is justified because it is taken by mouth together with two other oral medicines, lenalidomide and dexamethasone, meaning that patients who received a previous treatment and require triple therapy can conveniently take their medicines at home. In addition, Ninlaro is well tolerated by patients, and does not seem to significantly increase the frequency of serious side effects when added to lenalidomide and dexamethasone.

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

More information on the COMP assessment can be found in the October 2016 COMP minutes.

Further information on Ninlaro 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.