8 June 2016
EMA/COMP/332269/2016
Committee for Orphan Medicinal Products

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

Darzalex (daratumumab) for the treatment of plasma cell myeloma

During its meeting of 19 to 21 April 2016, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/13/1153 for Darzalex (daratumumab) as an orphan medicinal product for the treatment of plasma cell myeloma (also known as 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 plasma cell 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 Darzalex be given conditional marketing authorisation for:

'treatment of adult patients with relapsed and refractory multiple myeloma, whose prior therapy included a proteasome inhibitor and an immunomodulatory agent and who have demonstrated disease progression on the last therapy'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2013. Plasma cell myeloma remains a debilitating and life-threatening condition because it disrupts the normal functioning of the bone marrow, leads to bone lesions 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.

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 plasma cell myeloma based on data from the GLOBOCAN and NORDCAN 2012 databases, as well as data from the haematological malignancy research network.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of plasma cell 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, several medicines were authorised in the EU for treating plasma cell myeloma as first- and second-line therapies (in patients who had receive no or one previous treatment). The immunomodulatory agent pomalidomide and the medicine Farydak (panobinostat) were the only medicines authorised as third-line therapy, with similar indications to Darzalex.

Significant benefit of Darzalex

The COMP concluded that the claim of a significant benefit of Darzalex in plasma cell myeloma is justified based on study data in patients whose disease came back after, or did not respond to, at least two previous treatments including a proteasome inhibitor and an immunomodulatory agent. Results showed that around 31% of patients responded to treatment with Darzalex and the estimated overall survival was 20 months. In addition, indirect comparisons with Farydak, also authorised as third-line therapy, showed that Darzalex is more effective and has a better safety profile than Farydak.

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

Conclusions

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