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Committee for Orphan Medicinal Products

Recommendation for maintenance of orphan designation at the time of addition of a new indication to the marketing authorisation

Revlimid (lenalidomide) for the treatment of myelodysplastic syndromes

During its meeting of 14-15 May 2013, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EMA/OD/083/03 for Revlimid (lenalidomide, previously known as 3-(4'aminoisoindoline-1'-one)-1-piperidine-2,6-dione) as an orphan medicinal product for the treatment of myelodysplastic syndromes. The COMP assessed whether, at the time of addition of a new indication to the marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the conditions, and the existence of other satisfactory methods of treatment. As other methods of treatment for patients with myelodysplastic syndromes are authorised in the European Union (EU), the COMP also looked at the significant benefit of the product over existing treatments. 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 extending the approved therapeutic indication for Revlimid to include the following indication:

'treatment of patients with transfusion-dependent anaemia due to low- or intermediate-1-risk myelodysplastic syndromes associated with an isolated deletion 5q cytogenetic abnormality when other therapeutic options are insufficient or inadequate'.

This falls within the scope of the product's designated orphan condition, which is: 'treatment of myelodysplastic syndromes'.

The COMP concluded that there had been no change in the seriousness of the conditions since the orphan designation in 2004. Myelodysplastic syndromes remain long-term debilitating and life-threatening diseases because of the need of frequent blood transfusions and the risk of infections and bleeding.

[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 a review of recent scientific literature on the prevalence of myelodysplastic syndromes. On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of myelodysplastic syndromes 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 3 people in 10,000. This is equivalent to a total of fewer than 153,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, Vidaza (azacitidine) and Glivec (imatinib) were authorised in the EU for the treatment of myelodysplastic syndromes. However, Vidaza was only authorised for use in patients with higher risk myelodysplastic syndromes, and Glivec was for use in patients with myelodysplastic/myeloproliferative syndromes associated with platelet-derived growth factor (PDGFR) gene re-arrangements, which represents a small minority of the myelodysplastic syndromes patient population.

Haematopoietic (blood) stem cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow) was the only potentially curative treatment for myelodysplastic syndromes, but because of its risks and the lack of suitable donors it was only considered in younger patients with high-risk disease.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Revlimid in myelodysplastic syndromes is justified because of its effectiveness in a sub-group of patients with lower risk myelodysplastic syndromes, for whom no authorised treatments are available. This is based on the results of a main study in 205 patients with lower risk myelodysplastic syndromes, which showed that 55% of patients treated with Revlimid did not need blood transfusions for at least 6 months, compared with 6% of patients who received placebo (a dummy treatment). In addition, patients treated with Revlimid had a clinically relevant increase in haemoglobin (the protein found in red blood cells that carries oxygen around the body).

Therefore, although other methods for treating some patients with myelodysplastic syndromes have been authorised in the EU, the COMP concluded that Revlimid is of significant benefit in myelodysplastic syndrome therapy.

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Revlimid still meets the criteria for designation as an orphan medicinal product for the treatment of myelodysplastic syndromes and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Revlimid 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.