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EMA/COMP/558969/2012
Committee for Orphan Medicinal Products

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

Dacogen (decitabine) for the treatment of acute myeloid leukaemia

During its meeting of 4-5 September 2012 the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/06/370 for Dacogen (decitabine) as an orphan medicinal product for the treatment of acute myeloid leukaemia. 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 satisfactory methods of treatment. As other satisfactory methods of treatment for patients with this condition 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 the authorisation of Dacogen for:

'treatment of adult patients aged 65 years and above with newly diagnosed de novo and secondary acute myeloid leukaemia (AML), according to the World Health Organisation (WHO) classification, who are not candidates for standard induction chemotherapy'.

This falls within the scope of the product's designated orphan indication, which is:' acute myeloid leukaemia'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in June 2006. Acute myeloid leukaemia remains a condition that is a long-term debilitating and life-threatening disease because it reduces the patient's ability to fight infections and causes bleeding in the gut and brain.

[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 mainly derived from the database Globocan 2008. On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of acute myeloid leukaemia 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 2 people in 10,000. This is equivalent to a total of fewer than 101,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, other methods of treatment were authorised in the EU for the treatment of acute myeloid leukaemia. The main treatment was chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Dacogen in acute myeloid leukaemia is justified on the basis of improved effectiveness in a sub-group of patients aged 65 years and above with acute myeloid leukaemia who are not eligible for standard induction chemotherapy. This is based on the results from a clinical trial comparing Dacogen with either supportive care (any medicine or technique to help patients, excluding anticancer medicines or surgery) or low-dose cytarabine (another anticancer medicine) which showed an improvement in median survival of 2.7 months for Dacogen-treated patients.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Dacogen is of significant benefit for patients affected by acute myeloid leukaemia.

Conclusions

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