9 April 2013
EMA/COMP/71582/2013
Committee for Orphan Medicinal Products

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

Bosulif (bosutinib) for the treatment chronic myeloid leukaemia

On 12 February 2013, the Committee for Orphan Medicinal Products (COMP) finalised the review of the designation EU/3/10/762 for Bosulif (bosutinib) as an orphan medicinal product for the treatment chronic myeloid leukaemia (CML). 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 Bosulif for:

'treatment of adult patients with chronic phase, accelerated phase, and blast phase Philadelphia chromosome positive CML previously treated with one or more tyrosine kinase inhibitors and for whom imatinib, nilotinib and dasatinib are not considered appropriate treatment options'.

This falls within the scope of the product's designated orphan indications, which is CML.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Although CML usually develops very slowly, which is why it is called 'chronic', when it progresses, it is a severe and life-threatening disease that is associated with poor overall survival.

[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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

Prevalence of the condition

The sponsor provided updated information on the prevalence of CML based on data from the Globocan 2008 database (which contains prevalence data for all types of leukaemia combined).

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of CML 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 approximately 1 person in 10,000. This is equivalent to a total of around 50,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, several medicines were authorised in the EU for the treatment of CML, including imatinib, nilotinib and dasatinib, which are orphan medicines known as 'tyrosine kinase inhibitors' (TKIs). In addition, haematopoietic stem cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow) was used in some patients.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Bosulif in CML is justified because this medicine represents a valuable alternative treatment option in a sub-group of patients identified as having an unmet medical need. This is based on the results of a study in 52 patients in the advanced stages of CML, for whom treatment with other TKIs was not considered suitable (due to concomitant medical conditions, disease resistance or the risk of severe side effects). The results showed that Bosulif was effective as a last treatment option for controlling CML in these patients.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Bosulif is of significant benefit for patients affected by CML.

Conclusions

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

Further information on the current regulatory status of Bosulif 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.