EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 November 2014
EMA/COMP/535574/2014
Committee for Orphan Medicinal Products

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

Imbruvica (ibrutinib) for the treatment of chronic lymphocytic leukaemia

During its meeting of 2 to 4 September 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/12/984 for Imbruvica (ibrutinib[1]) as an orphan medicinal product for the treatment of chronic lymphocytic leukaemia (CLL). 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 CLL. 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 Imbruvica for:

'treatment of adult patients with chronic lymphocytic leukaemia (CLL) who have received at least one prior therapy, or in first line in the presence of 17p deletion or TP53 mutation in patients unsuitable for chemo-immunotherapy'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2012. CLL remains a condition that is debilitating in the long term and life threatening because patients may develop disease-related complications including severe infections.

[1] Previously known as 1-[(3R)-3-[4-amino-3-(4-phenoxyphenyl)-1H- pyrazolo [3,4-d]pyrimidin-1-yl]-1-piperidinyl]-2-propen-1-one.
[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 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 of CLL based on data from RARECARE and the GLOBOCAN 2008 database.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of CLL 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 still estimated to be approximately 3 people in 10,000. This is equivalent to a total of around 153,000 people in the EU.

Existence of other methods of treatment

The COMP noted that, at the time of the review of the orphan designation, the main treatment for CLL was chemotherapy (medicines to treat cancer), which might include chlorambucil or fludarabine-based therapy. In patients with CLL that did not respond to or had come back after previous treatment, subsequent treatments included ofatumumab and the combination of rituximab with chemotherapy medicines.

Significant benefit of Imbruvica

The COMP concluded that the claim of a significant benefit of Imbruvica in CLL is justified by data showing that treatment with Imbruvica improved patient's overall survival (how long they lived) and progression-free survival (how long they lived without their disease getting worse) compared with existing treatments. Imbruvica was also shown to be effective in patients with those mutations (17p deletion or TP53 mutation) that confer a poor prognosis. In addition, the COMP noted that Imbruvica has the advantage of being available as capsules that can be taken by mouth at home unlike current treatments that are given by injection in hospital.

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

Conclusions

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