8 September 2015
EMA/COMP/396537/2015
Committee for Orphan Medicinal Products

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

Imbruvica (ibrutinib) for the treatment of lymphoplasmacytic lymphoma

During its meeting of 16 to 18 June 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/14/1264 for Imbruvica (ibrutinib) as an orphan medicinal product for the treatment of lymphoplasmacytic lymphoma (also known as Waldenström's macroglobulinaemia). 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 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 lymphoplasmacytic lymphoma. 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 Imbruvica to include the following indication:

'treatment of adult patients with Waldenström's macroglobulinaemia who have received at least one prior therapy, or in first line treatment for patients unsuitable for chemo-immunotherapy'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2014. Lymphoplasmacytic lymphoma remains a long-term debilitating and life-threatening condition due to damage to the bone marrow and other organs.

[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 lymphoplasmacytic lymphoma based on data from the Globocan 2012 database.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of lymphoplasmacytic lymphoma 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 less than 0.1 people in 10,000. This is equivalent to a total of fewer than 5,000 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 treatments for lymphoplasmacytic lymphoma included immunotherapy (medicines that stimulate the body's own immune system to kill the cancer cells), and combinations of immunotherapy (such as rituximab) with chemotherapy (cancer medicines intended to kill the cancer cells).

Significant benefit of Imbruvica

The COMP concluded that the claim of a significant benefit of Imbruvica for patients with lymphoplasmacytic lymphoma is justified. Imbruvica was shown to improve outcomes in patients whose disease had come back after, or had not responded to, previous treatments. This is based on a main study of previously treated patients which showed that around 87% (55 out of 63) of patients responded to treatment with Imbruvica.

Furthermore, Imbruvica could also be of benefit to previously untreated patients who cannot receive chemo-immunotherapy. No satisfactory treatments are currently available to these patients.

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 for patients affected by lymphoplasmacytic lymphoma.

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.