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EMA/COMP/4640/2014
Committee for Orphan Medicinal Products

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

Cometriq (cabozantinib) for the treatment of medullary thyroid carcinoma

During its meeting of 7-9 January 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/08/610 for Cometriq (cabozantinib)[1] as an orphan medicinal product for the treatment of medullary thyroid carcinoma. 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 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[2].

Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Cometriq for:

'treatment of adult patients with progressive, unresectable locally advanced or metastatic medullary thyroid carcinoma'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Medullary thyroid carcinoma remains debilitating due to the major surgery needed, and is a life-threatening condition in patients whose disease cannot be treated by surgery or has spread throughout the body.

[1] Previously known as cyclopropane-1,1-dicarboxylic acid [4-(6,7-dimethoxy-quinolin-4-yloxy)-phenyl]-amide (4-fluoro-phenyl)-amide, (L)-malate salt).
[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 informed the COMP that no literature suggestive of a change in the EU prevalence of medullary thyroid carcinoma has been published since the orphan designation was granted in 2009.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of medullary thyroid carcinoma 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 0.7 people in 10,000. This is equivalent to a total of fewer than 36,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, medullary thyroid carcinoma was primarily treated by surgical removal of the thyroid gland. Caprelsa (vandetanib) and, in Sweden, doxorubicin were authorised in the EU for the treatment of medullary thyroid carcinoma.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Cometriq in medullary thyroid carcinoma is justified on the basis of a different safety profile to existing treatments, which means that Cometriq can be used in some patients who are unsuitable for other treatments such as Caprelsa due to risks of heart problems. Additionally, Cometriq may offer a treatment option for patients for whom other treatments do not work or have stopped working: the sponsor has shown that out of 25 patients with progressive disease who had previously been given Caprelsa, 7 had a partial response when treated with Cometriq.

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

Conclusions

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

Further information on the current regulatory status of Cometriq 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.