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Committee for Orphan Medicinal Products

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

Nexavar (sorafenib) for the treatment of follicular and papillary thyroid cancers

During its meeting of 13 to 14 May 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designations EU/3/13/1199 and EU/3/13/1200 for Nexavar (sorafenib) as an orphan medicinal product for the treatment of follicular and papillary thyroid cancers. 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 and prevalence of the conditions, 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 follicular and papillary thyroid cancer. The COMP recommended that the orphan designations 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 Nexavar to include the following indication:

'treatment of patients with progressive, locally advanced or metastatic, differentiated (papillary/follicular/Hürthle cell) thyroid carcinoma, refractory to radioactive iodine'.

This falls within the scope of the product's designated orphan conditions, which are: follicular and papillary thyroid cancers.

The COMP concluded that there had been no change in the seriousness of the conditions since the orphan designation in November 2013. Follicular and papillary thyroid cancers remain conditions that are debilitating in the long term and life threatening, particularly when the cancer does not respond to treatment and spreads to other parts of the body.

[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 information on the prevalence of follicular and papillary thyroid cancers based on data from the 2008 Globocan database.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of follicular and papillary thyroid cancers remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designations, the prevalence of follicular thyroid cancer was still estimated to be between 0.2 and 0.9 people in 10,000, and the prevalence for papillary thyroid cancer was still between 1 and 3 people in 10,000. This is equivalent to a total of between 10,000 and 46,000 people in the EU for follicular thyroid cancer, and between 51,000 and 153,000 people in the EU for papillary thyroid cancer.

Existence of other methods of treatment

At the time of the review of the orphan designation, the main treatment for follicular thyroid and papillary cancers in the EU was surgery to remove the thyroid. Therapy using radioactive iodine (^{131}I) to destroy thyroid cells was also used. Hormonal therapy was used as an additional treatment for preventing recurrence of the disease. In addition, the anticancer medicine doxorubicin was authorised for the treatment of follicular and papillary thyroid cancers in one EU Member State.

Significant benefit of Nexavar

The COMP concluded that the claim of a significant benefit of Nexavar in follicular and papillary thyroid cancers is justified because of its demonstrated benefit in patients whose cancer has progressed or spread locally or to other parts of the body and does not respond to radioactive iodine. These patients have no appropriate treatment options.

The COMP conclusions are based on data from a main study involving patients with differentiated (papillary/follicular/Hürthle cell) thyroid cancer that had progressed or spread locally or to other parts of the body and did not respond to radioactive iodine. The study showed that Nexavar increased the time that patients lived without their disease getting worse by an average of about 5 months more than placebo (a dummy treatment).

Therefore, although other methods for the treatment of these conditions have been authorised in the EU, the COMP concluded that Nexavar is of significant benefit to patients affected by follicular and papillary thyroid cancer.

Conclusions

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