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

Zejula (niraparib) for the treatment of ovarian cancer

During its meeting of 3 to 5 October 2017, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/10/760 for Zejula (niraparib[1]) as an orphan medicinal product for the treatment of ovarian cancer. 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 ovarian cancer.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 Zejula 'as monotherapy for the maintenance treatment of adult patients with platinum-sensitive relapsed high grade serous epithelial ovarian, fallopian tube, or primary peritoneal cancer who are in response (complete or partial) to platinum-based chemotherapy'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Ovarian cancer remains a debilitating and life-threatening condition that is associated with shortened life expectancy.

Prevalence of the condition

The sponsor provided updated information on the prevalence of ovarian cancer based on data from the database EUCAN (2012).

[1] Previously known as (3S)-3-{4-[7-(aminocarbonyl)-2H-indazol-2-yl] phenyl} piperidine tosylate monohydrate 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 the same therapeutic indication cannot be placed on the market.

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On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of ovarian cancer 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 estimated to be approximately 4.3 people in 10,000. This is equivalent to a total of around 222,000 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 ovarian cancer were surgery and/or medicines to treat cancer, which might include platinum–based therapy. In addition, Lynparza (olaparib), like Zejula, was authorised for use after platinum-based therapy for the maintenance treatment of relapsed high grade serous epithelial cancer of the ovary. However, Lynparza was only for use in patients who have mutations in either of two genes known as *BRCA1* and *BRCA2*.

Significant benefit of Zejula

The COMP concluded that the claim of a significant benefit of Zejula in ovarian cancer is justified because the medicine can be given to maintain the response to treatment with platinum–based medicines. Compared with Lynparza, Zejula is authorised for use in patients with or without *BRCA* mutations.

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

Conclusions

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

More information on the COMP assessment can be found in the October 2017 COMP minutes.

Further information on Zejula 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.