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EMA/COMP/93349/2012
Committee for Orphan Medicinal Products

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

Signifor (pasireotide) for the treatment of Cushing's disease

During its meeting of 7-8 February 2012, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/09/671 for Signifor (pasireotide) as an orphan medicinal product for the treatment of Cushing's disease. 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 satisfactory methods of treatment. As other satisfactory 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[1].

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

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Signifor for the treatment of adult patients with Cushing's disease for whom surgery is not an option or for whom surgery has failed.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Cushing's disease remains a condition that is debilitating in the long term and life threatening, particularly due to its complications resulting from the high levels of cortisol, which include cardiovascular disease, diabetes, clotting disorders, muscular weakness, osteoporosis and psychiatric conditions.

[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 recent scientific literature on the prevalence of Cushing's disease. On the basis of this information and the knowledge of the COMP, the COMP concluded that the prevalence of Cushing's disease 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 0.46 people in 10,000. This is equivalent to a total of around 23,000 people in the EU.

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, the main treatment for Cushing's disease involved surgery to remove the tumour responsible for causing high cortisol levels, sometimes followed by radiotherapy (treatment with radiation). In addition, medicines were used in the EU to reduce the production of cortisol or prevent it from working.

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Signifor over surgical treatment is justified on the basis of a clinically relevant advantage in the sub-group of patients with Cushing's disease for whom surgery has failed or is not an option.

When comparing Signifor with other medicines used in Cushing's disease, the COMP noted that Signifor has a different mechanism of action that directly targets the cause of the disease (i.e. the overproduction of adrenocorticotropic hormone (ACTH) by the pituitary gland). This results in a reduction of the risk of developing secondary pituitary tumours, which justifies the claim of significant benefit over currently authorised medicines.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Signifor is of significant benefit for patients affected by Cushing's disease.

Conclusions

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