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Committee for Orphan Medicinal Products

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

Plenadren (hydrocortisone) for the treatment of adrenal insufficiency

During its meeting of 6-8 September 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/06/372 for Plenadren (hydrocortisone) as an orphan medicinal product for the treatment of adrenal insufficiency. 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 Plenadren for:

'treatment of adrenal insufficiency in adults'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2006. Adrenal insufficiency remains a condition that is debilitating in the long term because of tiredness and weakness that affect quality of life. If left untreated the condition can be life threatening, as it can progress rapidly to adrenal crisis leading to shock and death.

Prevalence of the condition

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of adrenal insufficiency 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 less than 4.5 people in 10,000. This is equivalent to a total of fewer than 228,000 people in the EU.

[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.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

Existence of other satisfactory methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the treatment of adrenal insufficiency, including hydrocortisone oral tablets administered in two or three daily doses, and synthetic glucocorticoids (steroid hormones).

Significant benefit over existing treatments

The COMP concluded that the claim of a significant benefit of Plenadren in adrenal insufficiency is justified on the basis that the once-daily modified release formulation produces levels of active substance in the body which more closely mimic the natural release pattern of the hormone in healthy people compared with existing treatments. This offers a major contribution to patient care.

Therefore, although other satisfactory methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Plenadren is of significant benefit for adult patients affected by adrenal insufficiency.

Conclusions

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

Further information on the current regulatory status of Plenadren 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.