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Committee for Orphan Medicinal Products

COMP assesses whether Plenadren still meets orphan designation criteria

Recommendation to maintain the period of market exclusivity at 10 years

During its meeting of 21 to 23 March 2016, the Committee for Orphan Medicinal Products (COMP) assessed whether Plenadren (hydrocortisone) still met the criteria for orphan designation as there appeared to be an increase in the prevalence of the condition.[1] Plenadren has been authorised in the European Union for the treatment of adrenal insufficiency since 3 November 2011. At the time, because Plenadren met the criteria for orphan designation, it was granted 10 years of market exclusivity in the EU.[2]

A Member State can ask that this period of market exclusivity be reduced to 6 years if at the end of 5 years the criteria for orphan designation no longer apply and the medicine is sufficiently profitable.

At the request of the United Kingdom, the COMP therefore reviewed the criteria for orphan designation for Plenadren. 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 adrenal insufficiency. As these criteria continue to be met, the COMP recommended that the 10-year period of market exclusivity granted to Plenadren in 2011 for the treatment of adrenal insufficiency should not be reduced.

For more information about the original review at the time of initial marketing authorisation see:

> Recommendation for maintenance of orphan designation at the time of marketing authorisation: Plenadren (hydrocortisone) for the treatment of adrenal insufficiency

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

Plenadren is authorised in the EU for the treatment of adrenal insufficiency in adults. The product's designated orphan indication is 'treatment of adrenal insufficiency'.

The COMP concluded that there had been no change in the seriousness of the condition since the review of the designation at the time of marketing authorisation of Plenadren. Adrenal insufficiency

[1] In accordance with Article 8(2) of Regulation (EC) No 141/2000.
[2] This means that in the 10 years after authorisation similar products for the same therapeutic indication cannot be placed on the market.

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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 to adrenal crisis leading to shock and death.

Prevalence of the condition

The sponsor provided updated information on the prevalence of adrenal insufficiency based on data from the scientific literature.

On the basis of the information provided by the sponsor and the discussion within the COMP, the COMP concluded that the prevalence of adrenal insufficiency has increased to 4.85 people in 10,000 since its authorisation.[3] This still remains below the ceiling for orphan designation, which is 5 people in 10,000, and is equivalent to a total of around 249,000 people in the EU.

Existence of other methods of treatment

At the time of this review, 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 Plenadren remains of significant benefits for patients with adrenal insufficiency because based on clinical data its once-daily modified release formulation produces benefits in terms of body fat, control of blood sugar, and aspects of patients' quality of life compared with existing treatments. This was considered a major contribution to patient care.

Conclusions

Based on the available data 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 the period of market exclusivity for Plenadren should not be reduced.

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.

[3] At the time of authorisation, the estimated prevalence was 4.5 people in 10,000.