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Committee for Orphan Medicinal Products

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

Natpar (parathyroid hormone) for the treatment of hypoparathyroidism

On 6 March 2017, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/13/1210 for Natpar (parathyroid hormone) as an orphan medicinal product for the treatment of hypoparathyroidism. 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 treatment of hypoparathyroidism. 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 Natpar for the 'adjuvant treatment of adult patients with chronic hypoparathyroidism who cannot be adequately controlled with standard therapy alone'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2013. Hypoparathyroidism remains a debilitating and life-threatening condition because low levels of calcium can lead to problems with the bones, muscles, heart, kidneys and 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 the same therapeutic indication cannot be placed on the market.

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Prevalence of the condition

The sponsor provided updated information on the prevalence of hypoparathyroidism based on data from the published literature, the General Practice Registry of the UK and the US Health Analytics MarketScan Research Databases.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of hypoparathyroidism 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 3.5 people in 10,000. This is equivalent to a total of around 180,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, calcium and vitamin D supplements were authorised in the EU for the treatment of hypoparathyroidism. Some patients were also treated with thiazide diuretics to lower the amount of calcium that is eliminated in the urine.

Significant benefit of Natpar

The COMP concluded that the claim of a significant benefit of Natpar in hypoparathyroidism is justified on the basis of clinical data showing that Natpar helps control blood calcium levels, reducing the need for calcium and vitamin D supplements, which may lead to complications when used at high doses.

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

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Natpar 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 March 2017 COMP minutes.

Further information on Natpar 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.