7 January 2015
EMA/COMP/621067/2014
Committee for Orphan Medicinal Products

Recommendation for maintenance of orphan designation at the time of addition of a new indication to the marketing authorisation

Signifor (pasireotide) for the treatment of acromegaly

During its meeting of 7 to 9 October 2014, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/09/670 for Signifor (pasireotide) as an orphan medicinal product for the treatment of acromegaly. The COMP assessed whether, at the time of addition of a new indication to the 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 acromegaly. 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 extending the approved therapeutic indication for Signifor to include the following indication:

'treatment of adult patients with acromegaly for whom surgery is not an option or has not been curative and who are inadequately controlled on treatment with another somatostatin analogue'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2009. Acromegaly remains a condition that is debilitating in the long term and life threatening, particularly because of its cardiovascular (heart and blood vessels) and respiratory (lungs and airways) complications and the increased risk of developing cancer.

[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

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of acromegaly 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 2 people in 10,000. This is equivalent to a total of around 102,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, other treatments were authorised in the EU for the treatment of acromegaly. These included 'somatostatin analogues' (medicines that block the release of growth hormone) such as octreotide and lanreotide, and pegvisomant (a medicine that blocks the effects of growth hormone). Other treatments included surgery and, in rare cases, radiotherapy (treatment with radiation).

Significant benefit of Signifor

The COMP concluded that the claim of a significant benefit of Signifor in acromegaly is justified on the basis of relevant data from a study which shows improved reduction of growth hormone levels in patients inadequately controlled with the currently authorized somatostatin analogues.

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

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.