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

Lutathera (lutetium (^{177}Lu) oxodotreotide) for the treatment of gastro-entero-pancreatic neuroendocrine tumours

On 25 July 2017, the Committee for Orphan Medicinal Products (COMP) completed a review of the designation EU/3/07/523 for Lutathera (lutetium (^{177}Lu) oxodotreotide) as an orphan medicinal product for the treatment of gastro-entero-pancreatic neuroendocrine tumours. 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 gastro-entero-pancreatic neuroendocrine tumours. 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 Lutathera for:

'treatment of unresectable or metastatic, progressive, well differentiated (G1 and G2), somatostatin receptor positive gastroenteropancreatic neuroendocrine tumours (GEP-NETs) in adults'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of gastro-entero-pancreatic neuroendocrine tumours'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2008. Gastro-entero-pancreatic neuroendocrine tumours remain chronically debilitating as they may cause severe symptoms, and are life-threatening if they spread to other organs in 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 gastro-entero-pancreatic neuroendocrine tumours based on data from the published literature and from the RARECARE database.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of gastro-entero-pancreatic neuroendocrine tumours 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 3.8 people in 10,000. This is equivalent to a total of around 196,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, several products were authorised in the EU for the treatment of gastro-entero-pancreatic neuroendocrine tumours. Commonly, surgery and treatment with so called somatostatin analogues were used. These products are active against the symptoms, but are not active against the growth of the tumours. The cancer medicines Afinitor (everolimus) and Sutent (sunitinib), which target the growth of the tumours, were used for the treatment of pancreatic neuroendocrine tumours.

Significant benefit of Lutathera

The COMP concluded that the claim of a significant benefit of Lutathera in the treatment of gastro-entero-pancreatic neuroendocrine tumours is justified on the basis of the longer time patients lived without their disease getting worse when treated with Lutathera compared with other existing treatments. These included somatostatin analogues, everolimus and sunitinib.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Lutathera is of significant benefit to patients affected by gastro-entero-pancreatic neuroendocrine tumours.

Conclusions

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

Further information on Lutathera 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.