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

Xermelo (telotristat) for the treatment of carcinoid syndrome

On 25 July 2017, the Committee for Orphan Medicinal Products (COMP) completed a review of the designation EU/3/09/661 for Xermelo (telotristat[1]) as an orphan medicinal product for the treatment of carcinoid syndrome. 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 carcinoid syndrome. The COMP recommended that the orphan designation of the medicine be maintained[2].

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

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Xermelo for:

'Xermelo is indicated as an adjunct to somatostatin analogue (SSA) therapy for the treatment of carcinoid syndrome to improve symptom control in adult patients with metastatic neuroendocrine tumours'.

This falls within the scope of the product's designated orphan indication, which was originally 'treatment of carcinoid tumours' and was changed to 'carcinoid syndrome' in 2016.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2016. Carcinoid syndrome remains a condition that is debilitating in the long term, particularly due to flushing and diarrhoea, and can be life-threatening due to heart failure and difficulty in breathing.

[1] Previously known as (S)-ethyl 2-amino-3-(4-(2-amino-6(-1-(4-chloro-2-(3-methyl-1H-pyrazol-1-yl)phenyl)-2,2,2-trifluoroethoxy)pyrimidin-4-yl)phenyl)propanoate.
[2] 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 carcinoid syndrome based on data from national and European cancer registries and articles from the scientific literature.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of carcinoid syndrome 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 1 person in 10,000. This is equivalent to a total of around 52,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, somatostatin analogues, such as octreotide and lanreotide, were authorised in the EU to treat symptoms like diarrhoea caused by carcinoid tumours. Other medicines used to manage aspects of the condition included everolimus, sunitinib, and interferon alfa.

Significant benefit of Xermelo

The COMP concluded that the claim of a significant benefit of Xermelo in carcinoid syndrome is justified on the basis of a clinically relevant decrease in number of bowel movements suffered by patients with carcinoid syndrome not fully controlled with somatostatin analogues.

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

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Xermelo 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 Xermelo 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.