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

Verkazia (ciclosporin) for the treatment of vernal keratoconjunctivitis

On 31 May 2018 the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/06/360 for Verkazia (ciclosporin) as an orphan medicinal product in the treatment of vernal keratoconjunctivitis. 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 vernal keratoconjunctivitis. The COMP recommended that the orphan designation of the medicine be maintained[1].

Life-threatening or chronically debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Verkazia for: 'treatment of severe vernal keratoconjunctivitis (VKC) in children from 4 years of age and adolescents.'

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2006. Vernal keratoconjunctivitis remains a condition that is debilitating in the long term, particularly due to possible corneal ulcers and sight loss.

Prevalence of the condition

The sponsor provided updated information on the prevalence of vernal keratoconjunctivitis based on a survey conducted among ophthalmologists and on further epidemiological data.

[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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of vernal keratoconjunctivitis 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 3.2 people in 10,000. This is equivalent to a total of around 165,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, antihistamines and corticosteroids were authorised in the EU for the treatment of vernal keratoconjunctivitis. Although preparations of ciclosporin made in pharmacies for individual patients were available in some EU countries, these were not considered satisfactory methods of treatment.

Significant benefit of Verkazia

The COMP concluded that Verkazia is of significant benefit over corticosteroids because these medicines cannot be used long term due to their side effects. Regarding antihistamines, the COMP considered that the combined use of Verkazia with antihistamines may improve the outcome of patients with severe vernal keratoconjunctivitis.

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

Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP concluded that Verkazia still meets the criteria for designation as an orphan medicinal product and that the product should remain in the Community Register of Orphan Medicinal Products.

Further information on Verkazia 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.