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EMA/COMP/518763/2011
Committee for Orphan Medicinal Products

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

Votubia (everolimus) for the treatment of tuberous sclerosis

During its meeting of 8-9 June 2011, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/10/764 for Votubia (everolimus) as an orphan medicinal product for the treatment of tuberous sclerosis. 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 satisfactory methods of treatment. 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 Votubia for:

'the treatment of patients aged 3 years and older with subependymal giant cell astrocytoma (SEGA) associated with tuberous sclerosis complex (TSC) who require therapeutic intervention but are not amenable to surgery.'

This falls within the scope of the product's designated orphan indication(s), which is: 'treatment of tuberous sclerosis'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2010. Tuberous sclerosis remains a condition that is debilitating in the long term and life threatening, particularly due to the formation of multiple tumours and severe neurodevelopmental symptoms.

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 tuberous sclerosis remains below the ceiling for orphan designation,

[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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which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was still estimated to be approximately 1 person in 10,000. This is equivalent to a total of around 51,000 people in the EU.

Existence of other satisfactory methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no satisfactory treatments were authorised in the EU for patients affected by this condition.

Conclusions

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

Further information on the current regulatory status of Votubia 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.