24 September 2015
EMA/COMP/471205/2015
Committee for Orphan Medicinal Products

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

Strensiq (asfotase alfa) for the treatment of hypophosphatasia

During its meeting of 14 to 16 July 2015, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/08/594 for Strensiq (asfotase alfa[1]) as an orphan medicinal product for the treatment of hypophosphatasia. 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. 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 Strensiq for:

'long-term enzyme replacement therapy in patients with paediatric-onset hypophospatasia to treat the bone manifestations of the disease'.

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

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2008. Hypophosphatasia remains a condition that is debilitating in the long term and life threatening due incomplete development of the bones and respiratory problems.

Prevalence of the condition

The sponsor performed a search of the scientific literature and concluded that no publications are available which suggest a change in prevalence of hypophosphatasia.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of hypophosphatasia 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

[1] Previously known as 'recombinant human tissue non-specific alkaline phosphatase - Fc - deca-aspartate fusion protein'.
[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 a comparable therapeutic indication cannot be placed on the market.

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still estimated to be less than 0.01 people in 10,000. This is equivalent to a total of fewer than 500 people in the EU.

Existence of other methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no treatments were authorised in the EU for patients affected by this condition. Treatment aimed at relieving the symptoms of the disease such as plaster casts for broken bones, calcium supplements for maintaining the levels of calcium in the blood and painkillers.

Conclusions

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