1 April 2016
EMA/COMP/180349/2016
Committee for Orphan Medicinal Products

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

Coagadex (human coagulation factor X) for the treatment of hereditary factor X deficiency

During its meeting of 16 to 18 February 2016, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/471 for Coagadex (human coagulation factor X) as an orphan medicinal product for the treatment of hereditary factor X deficiency. 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 hereditary factor X deficiency. 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 Coagadex for the 'treatment and prophylaxis of bleeding episodes and for perioperative management in patients with hereditary factor X deficiency'.

This falls within the scope of the product's designated orphan indication, which is: 'treatment of hereditary factor X deficiency'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2007. Hereditary factor X deficiency remains a condition that is debilitating in the long term and life threatening due to the risk of major bleeding, which can also happen in the brain.

[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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Prevalence of the condition

The sponsor provided updated information on the prevalence of hereditary factor X deficiency based on data from the published scientific literature and the 2013 annual report of the World Federation of Haemophilia, as well as other registries and databases.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of hereditary factor X deficiency 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 people in 10,000. This is equivalent to a total of around 51,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, other treatments containing a mixture of human coagulation factors were authorised in several member states of the EU for the treatment and prevention of bleeding caused by deficiency of prothrombin complex coagulation factors (which include factor X deficiency).

Significant benefit of Coagadex

The COMP concluded that the claim of a significant benefit of Coagadex in hereditary factor X deficiency is justified because Coagadex is considered to be safer than currently authorised treatments. This is because, unlike other treatments, Coagadex contains only factor X and does not contain other coagulation factors that could become 'activated' during manufacturing and lead to the formation of blood clots.

Therefore, although other methods for the treatment of this condition have been authorised in the EU, the COMP concluded that Coagadex is of significant benefit to patients with hereditary factor X deficiency.

Conclusions

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

Further information on the current regulatory status of Coagadex 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.