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EMA/COMP/259512/2016
Committee for Orphan Medicinal Products

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

Alprolix (eftrenonacog alfa) for the treatment of haemophilia B

During its meeting of 21 to 23 March 2016, the Committee for Orphan Medicinal Products (COMP) reviewed the designation EU/3/07/453 for Alprolix (eftrenonacog alfa[1]) as an orphan medicinal product for the treatment of haemophilia B. 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 haemophilia B. 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 Alprolix for 'treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency)'.

This falls within the scope of the product's designated orphan indication, which is 'treatment of haemophilia B'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2007. Haemophilia B remains a condition that is debilitating in the long term and life threatening because it can lead to bleeding in the brain and spinal cord and from the throat and the gut.

[1] Previously known as recombinant fusion protein consisting of human coagulation factor IX attached to the Fc domain of human IgG1.
[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 haemophilia B based on data from the 2013 World Federation of Hemophilia Global Survey.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of haemophilia B 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 still estimated to be approximately 0.2 people in 10,000. This is equivalent to a total of around 10,000 people in the EU.

Existence of other methods of treatment

At the time of the review of the orphan designation, injectable medicines containing factor IX were authorised in the EU to treat haemophilia B. Medicines containing factor VII were authorised for use in patients who developed inhibitors (antibodies) that stopped factor IX medicines from working.

Significant benefit of Alprolix

The COMP concluded that the claim of a significant benefit of Alprolix in haemophilia B is justified because this medicine is given less frequently (once every 1 to 2 weeks) than other currently authorised treatments (which are given twice a week). This results in improved quality of life for patients and helps patients adhere to their treatment.

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

Conclusions

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