25 June 2015
EMA/CHMP/383497/2015
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Strensiq

asfotase alfa

On 25 June 2015, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation under exceptional circumstances[2] for the medicinal product Strensiq, intended for the treatment of paediatric-onset hypophosphatasia. Strensiq was designated as an orphan medicinal product on 3 December 2008. The applicant for this medicinal product is Alexion Europe SAS.

Strensiq will be available as 40 mg/ml and 100 mg/ml solutions for injection. The active substance of Strensiq is asfotase alfa, a human recombinant tissue-nonspecific alkaline phosphatase-Fc-deca-aspartate fusion protein. Asfotase alfa is an enzyme replacement therapy intended to supplement tissue-nonspecific alkaline phosphatase activity. It is thought to exert its beneficial effects by promoting mineralisation of the skeleton in patients with paediatric-onset hypophosphatasia.

The benefit of exposure to Strensiq is an improvement in skeletal structure, as demonstrated by x-ray appearance of joints, by histological appearance of bone biopsy material and by apparent catch-up height-gain seen in some patients. The most common side effects are injection site reactions and injection-associated adverse reactions of mostly non-serious character and mild to moderate intensity.

The full indication is: "Strensiq is indicated for long-term enzyme replacement therapy in patients with paediatric-onset hypophosphatasia to treat the bone manifestations of the disease (see section 5.1)." It is proposed that initiation of treatment with Strensiq should be by physicians experienced in the management of patients with metabolic or bone disorders.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

[2] In exceptional circumstances, an authorisation may be granted subject to certain specific obligations, to be reviewed annually. This happens when the applicant can show that they are unable to provide comprehensive data on the efficacy and safety of the medicinal product, due to the rarity of the condition it is intended for, limited scientific knowledge in the area concerned, or ethical considerations involved in the collection of such data.

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