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EPAR summary for the public

Strensiq

asfotase alfa

This is a summary of the European public assessment report (EPAR) for Strensiq. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Strensiq.

For practical information about using Strensiq, patients should read the package leaflet or contact their doctor or pharmacist.

What is Strensiq and what is it used for?

Strensiq is a medicine used long-term to treat patients with hypophosphatasia that started in childhood. Hypophosphatasia is a rare inherited disease of the bones which can lead to early loss of teeth, malformed bones, frequent bone fractures, and difficulty breathing.

Strensiq contains the active substance asfotase alfa. Because the number of patients with hypophosphatasia is low, the disease is considered 'rare', and Strensiq was designated an 'orphan medicine' (a medicine used in rare diseases) on 3 December 2008.

How is Strensiq used?

Strensiq can only be obtained with a prescription and treatment should be started by a doctor who is experienced in managing metabolic or bone disorders.

The medicine is available as a solution for injection in vials of different strengths and volumes. It is given by injection under the skin either six times a week (in a dose of 1 mg per kilogram bodyweight) or three times a week (in a dose of 2 mg/kg). As the amount given depends on the individual's bodyweight, the doctor will need to adjust the dose as patient's weight changes, particularly in growing children. For further information, see the package leaflet.

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How does Strensiq work?

Hypophosphatasia is caused by defects in the gene responsible for producing 'tissue non-specific alkaline phosphatase' (ALP), an enzyme that plays a key role in creating and maintaining healthy bones, and managing calcium and phosphate in the body. Patients with hypophosphatasia do not have enough normally functioning ALP, which leads to weak bones. Asfotase alfa, the active substance in Strensiq, is a modified copy of the human ALP enzyme and serves as a replacement for the defective enzyme, thereby increasing levels of functioning ALP.

What benefits of Strensiq have been shown in studies?

Strensiq has been studied in one main study in 13 children between 6 and 12 years of age. Patients were given either 2 mg/kg or 3 mg/kg Strensiq three times a week for 24 weeks. The main measure to indicate effectiveness of the medicine was the improvement in x-ray appearance of the wrists and knee joints of patients before and after treatment with Strensiq. X-rays of children given Strensiq were also compared with similar x-rays available from 16 children who had not received Strensiq ('historical controls'). The study also looked at other measures of effectiveness such as growth in height. This study showed that children given Strensiq had an improvement in their joint structure as demonstrated by x-rays and most of them seemed to gain in height. In the historical controls, most children did not experience similar improvements in their joints or gain in height over a comparable period of time.

The effectiveness of Strensiq was also generally supported by several additional small studies. Some of the studies also looked at the dose of 1 mg/kg Strensiq given six times a week.

What are the risks associated with Strensiq?

The most common side effects with Strensiq (which may affect more than 1 in 10 people) are headache, erythema (reddening of the skin), pain in arms and legs, fever, irritability, injection site reactions (such as pain, rash and itching) and contusion (bruising). For the full list of all side effects and restrictions reported with Strensiq, see the package leaflet.

Why is Strensiq approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Strensiq's benefits are greater than its risks and recommended that it be approved for use in the EU. The Committee noted that hypophosphatasia is a serious and life-threatening disease for which no treatment is authorised. Although the main study was small and did not directly compare Strensiq with another treatment or with untreated patients, the CHMP considered that the improvement seen in the bones and the apparent growth was relevant. Given that hypophosphatasia is an extremely rare disease, data in this population will likely remain limited. Regarding safety, injection site reactions and other side effects were considered manageable with the recommendations in place.

Strensiq has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Strensiq due to the rarity of the disease. Every year, the European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

What information is still awaited for Strensiq?

Since Strensiq has been approved under exceptional circumstances, the company that markets Strensiq will set up a registry of patients with hypophosphatasia to collect information on the disease and on the long-term safety and effectiveness of Strensiq.

What measures are being taken to ensure the safe and effective use of Strensiq?

A risk management plan has been developed to ensure that Strensiq is used as safely as possible. Based on this plan, safety information has been included in the summary of product characteristics and the package leaflet for Strensiq, including the appropriate precautions to be followed by healthcare professionals and patients.

In addition, the company will provide patients and carers with educational materials to ensure that Strensiq is used correctly and to minimise the risk of medication errors. This material will include a self-injection guide for patients, as well as an injection guide for parents or caregivers of children with the condition.

The company will also carry out a study to explore how adult patients respond to the dose of Strensiq given to children. It will also continue ongoing studies to further analyse the benefit of Strensiq in children from 13 to 18 years of age.

Further information can be found in the summary of the risk management plan.

Other information about Strensiq

The European Commission granted a marketing authorisation valid throughout the European Union for Strensiq on < date of issue of the Marketing Authorisation>.

The full EPAR and risk management plan summary for Strensiq can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Strensiq, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Strensiq can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 08-2015.