



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/716209/2018
EMA/H/C/003794

Strensiq (*asfotase alfa*)

An overview of Strensiq and why it is authorised in the EU

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.

Hypophosphatasia is rare, and Strensiq was designated an 'orphan medicine' (a medicine used in rare diseases) on 3 December 2008. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu308594.

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. 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 patient's bodyweight, the doctor will need to adjust the dose as the patient's weight changes, particularly in growing children.

For more information about using Strensiq, see the package leaflet or contact your doctor or pharmacist.

How does Strensiq work?

The enzyme 'tissue non-specific alkaline phosphatase' (ALP) plays a key role in creating and maintaining healthy bones, and managing calcium and phosphate in the body. Patients with hypophosphatasia cannot make enough working ALP, which leads to weak bones. Asfotase alfa, the active substance in Strensiq, is a version of the human ALP enzyme and serves as a replacement, thereby increasing levels of working 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 of effectiveness of the medicine was the improvement in x-ray appearance of the wrists and knee joints 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. Additional data from an extension of this study in children aged 13 to 18 years confirmed the initial results.

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 side effects and restrictions with Strensiq, see the package leaflet.

Why is Strensiq authorised in the EU?

The European Medicines Agency decided that Strensiq's benefits are greater than its risks and it is authorised for use in the EU.

The Agency 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 Agency 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 Agency will review any new information that becomes available and this overview will be updated as necessary.

What information is still awaited for Strensiq?

Since Strensiq has been authorised 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?

The company that markets Strensiq will provide patients and carers with educational materials to ensure that the medicine 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.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Strensiq have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Strensiq is continuously monitored. Side effects reported with Strensiq are carefully evaluated and any necessary action taken to protect patients.

Other information about Strensiq

Strensiq received a marketing authorisation valid throughout the EU on 28 August 2015.

Further information on Strensiq can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/Strensiq

This overview was last updated in 10-2018.