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Press Office

Press release

First treatment recommended for rare bone disease

Enzyme replacement therapy Strensiq to benefit patients with hypophosphatasia developed in childhood

The European Medicines Agency (EMA) has recommended granting a marketing authorisation under exceptional circumstances for Strensiq (asfotase alfa), for the long-term treatment of hypophosphatasia, a rare inherited metabolic disorder affecting the bones, in patients who have developed the disease in childhood.

Hypophosphatasia is caused by defects in the gene responsible for producing '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 have symptoms such as early loss of teeth, malformed bones and frequent bone fractures. This disease can be life-threatening when it affects unborn babies or infants, because of the incomplete development of their bones and additional complications such as respiratory problems. When it develops later in life, the disease is generally not fatal but can be highly debilitating.

There is currently no approved treatment for this condition; patients usually receive supportive treatment such as plaster casts for broken bones, calcium supplements for maintaining the levels of calcium in the blood and painkillers. Strensiq, the first therapy for this disease, could contribute to respond to this unmet medical need as it is expected to help improve the composition of bones and make them stronger. Asfotase alfa, its active substance, is a modified copy of the human ALP enzyme and serves as a replacement for the defective enzyme.

The Committee for Medicinal Products for Human Use (CHMP) considered that Strensiq should be recommended for marketing authorisation under exceptional circumstances. This type of authorisation can be granted for medicines that offer new or improved treatment options for patients with no or only limited alternatives, in cases where the applicant is not able to provide comprehensive data. In the case of Strensiq, data on the efficacy and safety are limited due to the extreme rarity of the disease. However, CHMP required the applicant to collect further data on its clinical efficacy and safety and submit these data regularly for review by the Committee after the granting of a marketing authorisation.

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Strensiq has benefitted from a range of EMA tools to support innovation. The company received scientific advice from the CHMP. This is one of the Agency's main tools to facilitate and stimulate research and development within the European Union (EU).

Because hypophosphatasia is rare, Strensiq was also designated as an orphan medicine by the Committee for Orphan Medicinal Products (COMP) in 2008. Orphan designation gives medicine developers access to incentives such as fee reductions for scientific advice, and is the key instrument available in the EU to encourage the development of medicines for patients with rare diseases.

Strensiq will be given to young patients during their childhood and adolescence, and can be used later on during their adult life. The medicine will be available in various strengths and volumes so that doses can be adjusted to the patient's size and weight. To ensure that the medicine is given correctly, EMA has advised on a colour code system to better distinguish between the different forms of the medicine. Booklets with detailed information on the correct use of the medicine will be provided to patients and carers.

The opinion adopted by the CHMP at its June 2015 meeting is an intermediary step on Strensiq's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, each Member State will take a decision on price and reimbursement based on the potential role/use of this medicine in the context of its national health system.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The marketing-authorisation applicant for Strensiq is Alexion Europe SAS.
3. Following this positive CHMP opinion, the COMP will assess whether the orphan designation should be maintained.
4. More information on EMA's recommendation for the safe use of Strensiq is available here.
5. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu.

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