Generic Name:
Asfotase alfa
Trade Name:
Strensiq
Company:
Alexion Pharmaceuticals
Notes:

FDA approved asfotase alfa as the first approved treatment for perinatal, infantile-onset, and juvenile-onset hypophosphatasia (HPP).

HPP is a rare, genetic, progressive, metabolic disease in which patients experience devastating effects on multiple systems of the body, leading to severe disability and life-threatening complications. It is characterized by defective bone mineralization that can lead to rickets and softening of the bones that result in skeletal abnormalities. It can also cause complications such as profound muscle weakness with loss of mobility, seizures, pain, respiratory failure, and premature death. Severe forms of HPP affect an estimated 1 in 100,000 newborns, but milder cases, such as those that appear in childhood or adulthood, may occur more frequently.

Asfotase alfa received a breakthrough therapy designation because it is the first and only treatment for perinatal, infantile-onset, and juvenile-onset HPP. FDA also granted asfotase alfa orphan drug designation because it treats a disease affecting fewer than 200,000 patients in the United States, as well as priority review and a rare pediatric disease priority review voucher.

Development of this drug was also in part supported by the FDA Orphan Products Grants Program, which provides grants for clinical studies on safety and/or effectiveness of products for use in rare diseases or conditions.

Asfotase alfa is administered via injection three or six times per week. The medication works by replacing the enzyme (known as tissue-nonspecific alkaline phosphatase) responsible for formation of an essential mineral in normal bone, which has been shown to improve patient outcomes.

Safety and efficacy of the drug were established in 99 patients with perinatal, infantile, or juvenile-onset HPP who received treatment for up to 6.5 years during four prospective, open-label studies. Study results showed that patients with perinatal- and infantile-onset HPP treated with asfotase alfa had improved overall survival and survival without the need for a ventilator.

Ninety-seven percent of treated patients were alive at 1 year of age, compared with 42% of control patients selected from a natural history study group. Similarly, the ventilator-free survival rate at 1 year of age was 85% for treated patients, compared with less than 50% for the natural history control patients.

Patients with juvenile-onset HPP treated with asfotase alfa showed improvements in growth

and bone health compared with control patients selected from a natural history database. All treated patients had improvement in low weight or short stature or maintained normal height and weight. In comparison, approximately 20% of control patients had growth delays over time, with shifts in height or weight from the normal range for children their age to heights and weights well below normal for age.

Juvenile-onset patients also showed improvements in bone mineralization, as measured on a scale that evaluates the severity of rickets and other HPP-related skeletal abnormalities based on x-ray images. All treated patients demonstrated substantial healing of rickets on x-rays, while some natural history control patients showed increasing signs of rickets over time.

The most common adverse effects in patients treated with asfotase alfa include injection site reactions, hypersensitivity reactions (such as difficulty breathing, nausea, dizziness and fever), lipodystrophy at the injection site, and ectopic calcifications of the eyes and kidney.

Medication Monitor Categories:
New Drug Approvals
