Brand Name: Strensiq
Generic: asfotase alfa
Type: glycoprotein
Year Accepted/Phase: 2015

Mechanism:

Asfotase alfa is a recombinant form of human alkaline phosphatase that replaces the deficient enzyme activity in patients with HPP. Asfotase alfa helps to hydrolyze pyrophosphate, a natural substrate of alkaline phosphatase, thereby reducing pyrophosphate levels and allowing for proper bone mineralization.

Chemical Structure: N/A

Indication:

Strensiq is indicated for the treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP) to improve bone mineralization and physical function.

Clinical trials:

ENB-002-08/ENB-003-08 Trial (Phase II)
Purpose: Assess the safety and efficacy of asfotase alfa in pediatric patients with hypophosphatasia.
Dates: Conducted from 2008 to 2014.
Results: These studies demonstrated that asfotase alfa significantly improved bone mineralization, growth, and respiratory function in pediatric patients with severe perinatal and infantile HPP. Radiographic evidence showed marked improvement in skeletal abnormalities, and there were notable gains in weight and height.
Impact: The results from these trials provided substantial evidence for the use of asfotase alfa in treating pediatric patients with severe HPP, leading to its regulatory approval.

ENB-010-10 Trial (Phase III)
Purpose: Evaluate the long-term efficacy and safety of asfotase alfa in infants and young children with HPP.
Dates: Conducted from 2010 to 2015.
Results: The trial showed that long-term treatment with asfotase alfa improved survival, skeletal mineralization, and motor development in infants and young children with life-threatening HPP. Patients exhibited continued improvements in skeletal health and physical function over the course of the study.
Impact: This study reinforced the benefits of early and sustained treatment with asfotase alfa in infants and young children, supporting its use as a long-term therapy for HPP.

ENB-006-09/ENB-008-10 Trials (Phase II/III)
Purpose: Assess the efficacy and safety of asfotase alfa in adolescent and adult patients with HPP.
Dates: Conducted from 2009 to 2014.
Results: These trials demonstrated significant improvements in bone mineral density, physical function, and pain reduction in adolescent and adult patients treated with asfotase alfa. Improvements in radiographic measures and patient-reported outcomes were also observed.
Impact: The findings from these trials provided important evidence for the effectiveness of asfotase alfa in older patients with HPP, expanding its indicated patient population.