Strensiq® (asfotase alfa)
Approved January 2020

Background:

Strensiq is a tissue nonspecific alkaline phosphatase indicated for the treatment of patients with perinatal/infantile and juvenile onset hypophosphatasia.

Hypophosphatasia (HPP) is a rare inherited disease caused by mutations of the ALPL gene, which encodes the tissue nonspecific alkaline phosphatase (TNSALP) isoenzyme.

Criteria for approval:

1. Patient has a diagnosis of perinatal/infantile or juvenile onset hypophosphatasia (HPP) evidenced by the following:
   a. Patient has clinical symptoms consistent with hypophosphatasia at the age of onset [for example, vitamin B6-dependent seizures, skeletal abnormalities such as flawed and frayed metaphysis; AND
   b. Molecular genetic test has confirmed mutations in the ALPL gene that encodes the tissue nonspecific isoenzyme of ALP (TNSALP); AND
   c. There is reduced activity of unfractionated serum alkaline phosphatase (ALP) [below the age and gender-adjusted normal range]; AND
   d. Patient has one of the following:
      i. Elevated urine concentration of phosphoethanolamine (PEA)
      ii. Elevated serum concentration of pyridoxal 5'-phosphate (PLP) in the absence of vitamin supplements within a week prior to assaying.
      iii. Elevated urine inorganic pyrophosphate (PPi)
2. Prescribed by or in consultation with a physician who specializes in the treatment of inherited metabolic disorders
3. Baseline ophthalmologic examination and renal ultrasound completed
4. Weight must be received for drugs that have weight-based dosing. Height and weight must be received for drugs that have dosing based on body surface area.
5. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, or national guidelines.

Initial Approval Duration: 6 months

Continuation of therapy:

1. Patient has responded to treatment as demonstrated by an improvement and/or stabilization (for example, radiographic findings, growth, mobility, respiratory status)
2. For dose increases, height and weight must be received for drugs that have dosing based on body surface area.
3. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, or national guidelines.

Renewal Approval Duration: 12 months

References:

1. Strensiq [prescribing information]. Cheshire, CT; Alexion Pharmaceuticals, Inc; October 2015
2. Rush ET. Childhood hypophosphatasia: to treat or not to treat. Orphanet Journal of Rare Diseases (2018)13:116.

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3. Mornet E, Nunes ME. Hypophosphatasia. U.S. National Library of Medicine (2007). Updated February 2016.
4. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically
5. National Institute for Health and Care Excellence (NICE) Guidance. Asfotase alfa for treating paediatric-onset hypophosphatasia. 2017. https://www.nice.org.uk/guidance/hst6. Accessed October 25, 2019.