SPECIALTY GUIDELINE MANAGEMENT

STRENSIQ (asfotase alfa)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Strensiq is indicated for the treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP)

All other indications are considered experimental/investigational and are not a covered benefit.

II. CRITERIA FOR INITIAL APPROVAL

Authorization of 12 months may be granted for treatment of HPP when all of the following criteria are met:
A. The member has clinical signs and/or symptoms of hypophosphatasia (See Appendix A)
B. The onset of the disease was perinatal/infantile or juvenile
C. The diagnosis was confirmed by the presence of mutation(s) in the ALPL gene as detected by ALPL molecular genetic testing OR the diagnosis is supported by ALL of the following:
    1. Radiographic imaging demonstrating skeletal abnormalities (See Appendix B)
    2. A serum alkaline phosphatase level below the gender- and age-specific reference range of the laboratory performing the test
    3. Elevated TNSALP substrate level (ie, serum PLP level, serum or urine PEA level, urinary PPi level)

III. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

IV. APPENDIX

Appendix A. Examples of Signs and Symptoms of HPP
A. Perinatal/infantile-onset HPP:
- Generalized hypomineralization with rachitic features, chest deformities and rib fractures
- Skeletal abnormalities (eg, short limbs, abnormally shaped chest, soft skull bone)
- Respiratory problems (eg, pneumonia)
- Hypercalcemia
- Failure to thrive
- Severe muscular hypotonia and weakness
- Nephrocalcinosis secondary to hypercalciuria
- Swallowing problems
- Seizures

B. Juvenile-onset HPP:
- Premature loss of deciduous teeth
- Failure to thrive with anorexia, nausea, and gastrointestinal problems
- Short stature with bowed legs or knock knees
- Skeletal deformities (eg, enlarged wrist and ankle joints, abnormal skull shape)
- Bone and joint pain
- Rickets
- Fractures
- Delayed walking
- Waddling gait

Appendix B. Examples of Radiographic Findings that Support HPP Diagnosis
- Infantile rickets
- Alveolar bone loss
- Focal bony defects of the metaphyses
- Metatarsal stress fractures
- Osteomalacia with lateral pseudofractures
- Osteopenia, osteoporosis, or low bone mineral content for age (as detected by dual-energy x-ray absorptiometry [DEXA])

V. REFERENCES

1. Strensiq [package insert]. Cheshire, CT: Alexion Pharmaceuticals, Inc.; October 2016.
2. Bianchi ML. Hypophosphatasia: an overview of the disease and its treatment. *Osteoporos Int.* 2015;26(12):2743-57.
3. Mornet E, Nunes ME. Hypophosphatasia. GeneReviews [Internet]. Available at http://www.ncbi.nlm.nih.gov/books/NBK1150/. Updated February 4, 2016. Accessed October 18, 2017.