Strensiq

**Description**

Strensiq (asfotase alfa)

**Background**

Strensiq is used to treat hypophosphatasia (HPP), a rare genetic disorder characterized by the abnormal development of bones and teeth. These abnormalities occur due to defective mineralization, the process by which bones and teeth take up minerals such as calcium and phosphorus. These minerals are required for proper hardness and strength. Hypophosphatasia is caused by mutations in the tissue nonspecific alkaline phosphatase gene. Such mutations lead to low levels of the tissue nonspecific alkaline phosphatase (TNSALP) enzyme. This enzyme is needed for the proper development and health of bones and teeth (1). Strensiq is administered via injection three or six times per week (2).

**Regulatory Status**

FDA-approved indication: Strensiq is a tissue nonspecific alkaline phosphatase indicated for the treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (2).

Patients with HPP are at increased risk for developing ectopic calcifications of the eye and kidneys. Ophthalmology (eye) examinations and renal ultrasounds are recommended at baseline and periodically during treatment to monitor for signs and symptoms of ophthalmic and renal ectopic calcifications and for changes in vision or renal function (2).

During clinical trials, anti-drug antibodies have been detected in patients receiving treatment with Strensiq using an electrochemiluminescent (ECL) immunoassay. Antibody positive samples
were tested to determine the presence of neutralizing antibodies based on in vitro inhibition of the catalytic activity of Strensiq. Formation of anti-drug antibody resulted in a reduced systemic exposure Strensiq (2).

The safety and effectiveness of Strensiq have been established in pediatric patients. The majority of patients in the clinical trials were pediatric patients 1 day to 16 years of age (2).

Related policies

Policy
This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Strensiq may be considered medically necessary in patients for the treatment of perinatal/infantile- and juvenile-onset hypophosphatasia and if the conditions indicated below are met.

Strensiq is considered investigational for patients with all other indications.

Prior-Approval Requirements

Diagnoses

Patient must have ONE of the following:
1. Perinatal/infantile-onset hypophosphatasia
2. Juvenile-onset hypophosphatasia

AND ALL of the following:
   a. Ophthalmology examination at baseline and periodically throughout treatment
   b. Renal ultrasound at baseline and periodically throughout treatment
   c. Physician agrees to assess patient’s improvement in growth and radiographical findings after one year of therapy and discontinue if NO improvement is seen

Prior – Approval Renewal Requirements

Diagnoses
Patient must have **ONE** of the following:
1. Perinatal/infantile onset hypophosphatasia
2. Juvenile-onset hypophosphatasia

**AND ALL** of the following:
- a. Ophthalmology examinations are done periodically throughout treatment
- b. Renal ultrasound are done periodically throughout treatment
- c. Documented improvement is seen in growth and radiographical findings

### Policy Guidelines

#### Pre - PA Allowance

None

#### Prior – Approval Limit

**Duration** 12 months

#### Prior – Approval **Renewal** Limits

**Duration** 2 years

### Rationale

**Summary**
Strensiq is a tissue nonspecific alkaline phosphatase indicated for the treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia. Ophthalmology (eye) examinations and renal ultrasounds are recommended at baseline and periodically during treatment to monitor for signs and symptoms of ophthalmic and renal ectopic calcifications and for changes in vision or renal function. The safety and effectiveness of Strensiq have been established in pediatric patients (2).

Prior authorization is required to ensure the safe, clinically appropriate and cost-effective use of Strensiq while maintaining optimal therapeutic outcomes.

#### References

1. Hypophosphatasia. Rare disease information. National Organization for Rare Disorders (NORD) website.
Section: Prescription Drugs  Effective Date: January 1, 2020
Subsection: Endocrine and Metabolic Drugs  Original Policy Date: December 4, 2015
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<td>Addition to PA</td>
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<td>Addition of the requirement of the physician agrees to assess patient’s improvement in growth and radiographical findings after one year of therapy and discontinue if no improvement is seen</td>
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<td>Change of duration from lifetime to 12 months for initiation</td>
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<td>Addition of renewal section for lifetime duration per SME</td>
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<td>Policy number change from 5.08.15 to 5.30.15</td>
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<td>June 2016</td>
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Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.