Statement of Medical Necessity

Treatment of Perinatal/Infantile- or Juvenile-Onset Hypophosphatasia (HPP)

Phone: **1-844-787-6747 (ext 8006)** Fax: **1-844-787-2527**

Fax this form to PANTHERx RARE to document medical necessity for initiating treatment.
Note: Additional information may be required based on insurance plan.

Please see Important Safety Information on the following page.

Patient Demographics

Name _____ Date of Birth ____/____/____ Age _____

Address _____ City, State _____ ZIP _____

Gender ☐ Male ☐ Female

Phone No. (home) _____ Phone No. (cell) _____

Parent/Guardian (if applicable) _____ Relation to patient _____ Phone _____

Please Provide the Following HPP Labs & Diagnostic Work-up/Testing

Baseline ALP _____	MM/DD/YYYY ___/___/___	Serum Calcium _____	MM/DD/YYYY ___/___/___
Latest ALP _____	MM/DD/YYYY ___/___/___	Creatinine _____	MM/DD/YYYY ___/___/___
Urinary PEA _____	MM/DD/YYYY ___/___/___	PTH _____	MM/DD/YYYY ___/___/___
Plasma PLP (B_6) _____	MM/DD/YYYY ___/___/___	Magnesium _____	MM/DD/YYYY ___/___/___
Genetic Mutation _____	MM/DD/YYYY ___/___/___	Vitamin D _____	MM/DD/YYYY ___/___/___

Diagnosis

Hypophosphatasia Dx:
MM/DD/YYYY _____/_____/_____

Onset type:
☐ Perinatal/Infantile-Onset ☐ Juvenile-Onset ☐ Other

The Following Symptoms and Complications Can Be Involved With HPP (check all that patient has experienced or is currently experiencing)

	History of/ currently has:	Onset <age 18
Skeletal		
Hypomineralization	☐	☐
Skeletal deformities	☐	☐
Fractures/pseudofractures	☐	☐
Craniosynostosis	☐	☐
Rachitic chest	☐	☐
Rickets	☐	☐
Bowing	☐	☐
Bone pain	☐	☐
Osteomalacia	☐	☐
Respiratory		
Respiratory insufficiency	☐	☐
Respiratory failure	☐	☐
Respiratory support	☐	☐
Dental		
Premature tooth loss/nontraumatic tooth loss (with root intact)	☐	☐
Poor/abnormal dentition	☐	☐

	History of/ currently has:	Onset <age 18
Muscular/Rheumatologic		
Muscle weakness	☐	☐
Hypotonia	☐	☐
Muscle/joint pain	☐	☐
Waddling gait	☐	☐
Difficulty walking	☐	☐
Neurologic		
Vitamin B_6–responsive seizures	☐	☐
Increased intracranial pressure	☐	☐
Growth/Development		
Failure to thrive	☐	☐
Delayed/missed motor milestones	☐	☐
Short stature	☐	☐
Functional Disabilities		
Wheelchair	☐	☐
Uses walking device	☐	☐

Healthcare Provider

I verify that the patient prescriber information contained in this enrollment form is complete and accurate to the best of my knowledge and based on my professional judgment of medical necessity.

Provider's Name (printed) _____ License # _____ NPI # _____

Address _____ City, State _____ ZIP _____

Phone _____ Fax _____ Email _____

Provider's Signature _____ MM/DD/YYYY ___/___/___

PLEASE ATTACH COPIES OF RECENT LAB REPORTS

STRENSIQ® (asfotase alfa) INDICATION & IMPORTANT SAFETY INFORMATION INCLUDING BOXED WARNING

INDICATION

STRENSIQ® is indicated for the treatment of patients with perinatal/infantile- and juvenile-onset hypophosphatasia (HPP).

IMPORTANT SAFETY INFORMATION

WARNING: HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS

Patients treated with enzyme replacement therapies have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Anaphylaxis has occurred during the early course of enzyme replacement therapy and after extended duration of therapy.

Initiate STRENSIQ under the supervision of a healthcare provider with appropriate medical monitoring and support measures. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue STRENSIQ and immediately initiate appropriate medical treatment, including use of epinephrine. Inform patients of the symptoms of life-threatening hypersensitivity reactions, including anaphylaxis and to seek immediate medical care should symptoms occur *[see Warnings and Precautions (5.1)]*.

WARNINGS AND PRECAUTIONS

- **Life-threatening hypersensitivity reactions, including anaphylaxis**, have been reported in STRENSIQ-treated patients. Signs and symptoms consistent with anaphylaxis included difficulty breathing, choking sensation, nausea, periorbital edema, and dizziness. These reactions have occurred within minutes after subcutaneous administration of STRENSIQ and have been observed more than 1 year after treatment initiation. Other hypersensitivity reactions have also been reported in STRENSIQ-treated patients, including vomiting, fever, headache, flushing, irritability, chills, erythema, rash, pruritus, and oral hypoesthesia. Consider the risks and benefits of re-administering STRENSIQ following a severe reaction. If the decision is made to re-administer, monitor patients for a reoccurrence of signs and symptoms of a severe hypersensitivity reaction.
- **Lipodystrophy:** Localized lipodystrophy, including lipoatrophy and lipohypertrophy has been reported at injection sites after several months in patients treated with STRENSIQ in clinical trials. Advise patients to follow proper injection technique and to rotate injection sites.
- **Ectopic Calcifications:** Patients with HPP are at increased risk for developing ectopic calcifications. Events of ectopic calcification, including ophthalmic (conjunctival and corneal) and renal (nephrocalcinosis, nephrolithiasis), have been reported in the clinical trial experience with STRENSIQ. There was insufficient information to determine whether the reported events were consistent with the disease or due to STRENSIQ. No visual changes or changes in renal function were reported resulting from the occurrence of ectopic calcifications.

  Ophthalmology examinations and renal ultrasounds are recommended at baseline and periodically during treatment with STRENSIQ to monitor for signs and symptoms of ophthalmic and renal ectopic calcifications and for changes in vision or renal function.
- **Possible Immune-Mediated Clinical Effects:** In clinical trials, most STRENSIQ-treated patients developed anti-asfotase alfa antibodies and neutralizing antibodies which resulted in reduced systemic exposure of asfotase alfa. In postmarketing reports, some STRENSIQ-treated patients with initial therapeutic response subsequently developed recurrence and worsening in disease-associated laboratory and radiographic biomarkers (some in association with neutralizing antibodies) suggesting possible immune-mediated effects on STRENSIQ's pharmacologic action resulting in disease progression. The effect of anti-asfotase alfa antibody formation on the long-term efficacy of STRENSIQ is unknown. There are no marketed anti-asfotase alfa antibody tests. If patients experience progression of HPP symptoms or worsening of disease-associated laboratory and imaging biomarkers after a period of initial therapeutic response to STRENSIQ, consider obtaining anti-asfotase alfa antibody testing by contacting STRENSIQ Medical Information at Alexion at 1-888-765-4747 or by email at medinfo@alexion.com. Close clinical follow up is recommended.

ADVERSE REACTIONS

In clinical trials, the most common adverse reactions (≥ 10%) reported were injection site reactions (63%), lipodystrophy (28%), ectopic calcifications (14%), and hypersensitivity reactions (12%). Possible immune-mediated clinical effects have been identified during post-approval use of STRENSIQ.

DRUG INTERACTIONS

Drug Interference with Laboratory Tests:

- Laboratory tests utilizing alkaline phosphatase (ALP) as a detection reagent could result in erroneous test results for patients receiving treatment due to the presence of asfotase alfa in clinical laboratory samples. Inform laboratory personnel that the patient is being treated with STRENSIQ and discuss use of an alternative testing platform which does not utilize an ALP-conjugated test system.
- Elevated serum ALP measurements detected through clinical laboratory testing are expected in patients receiving STRENSIQ due to circulating concentrations of asfotase alfa. Do not rely on serum ALP measurements for clinical decision making in patients treated with STRENSIQ.

SPECIAL POPULATIONS

- **Pregnancy & Lactation:** There are no available data on STRENSIQ use in pregnant women, the presence of STRENSIQ in human milk, or the effects on the breastfed infant or on milk production, to inform a drug associated risk.

To report SUSPECTED ADVERSE REACTIONS, contact Alexion Pharmaceuticals, Inc. at 1-844-259-6783 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch

Please see accompanying full Prescribing Information for STRENSIQ (asfotase alfa), including Boxed WARNING regarding hypersensitivity reactions including anaphylaxis, also available at strensiq-hcp.com.

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ALEXION®
AstraZeneca Rare Disease