

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: MM/DD/YYYY ____/____/____ Age _____
 Address _____ City, State _____ ZIP _____
 Sex 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 (B₆) _____ MM/DD/YYYY ____/____/____ Magnesium _____ MM/DD/YYYY ____/____/____
 Genetic Mutation _____ MM/DD/YYYY ____/____/____ Vitamin D _____ MM/DD/YYYY ____/____/____

Diagnosis

Hypophosphatasia Dx: _____ Onset Type: _____
 MM/DD/YYYY ____/____/____ 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		History of/ currently has:	Onset <age 18
Skeletal			Muscular/Rheumatologic		
Hypomineralization	<input type="checkbox"/>	<input type="checkbox"/>	Muscle weakness	<input type="checkbox"/>	<input type="checkbox"/>
Skeletal deformities	<input type="checkbox"/>	<input type="checkbox"/>	Hypotonia	<input type="checkbox"/>	<input type="checkbox"/>
Fractures/pseudofractures	<input type="checkbox"/>	<input type="checkbox"/>	Muscle/joint pain	<input type="checkbox"/>	<input type="checkbox"/>
Craniosynostosis	<input type="checkbox"/>	<input type="checkbox"/>	Waddling gait	<input type="checkbox"/>	<input type="checkbox"/>
Rachitic chest	<input type="checkbox"/>	<input type="checkbox"/>	Difficulty walking	<input type="checkbox"/>	<input type="checkbox"/>
Rickets	<input type="checkbox"/>	<input type="checkbox"/>	Neurologic		
Bowing	<input type="checkbox"/>	<input type="checkbox"/>	Vitamin B ₆ -responsive seizures	<input type="checkbox"/>	<input type="checkbox"/>
Bone pain	<input type="checkbox"/>	<input type="checkbox"/>	Increased intracranial pressure	<input type="checkbox"/>	<input type="checkbox"/>
Osteomalacia	<input type="checkbox"/>	<input type="checkbox"/>	Growth/Development		
Respiratory			Failure to thrive	<input type="checkbox"/>	<input type="checkbox"/>
Respiratory insufficiency	<input type="checkbox"/>	<input type="checkbox"/>	Delayed/missed motor milestones	<input type="checkbox"/>	<input type="checkbox"/>
Respiratory failure	<input type="checkbox"/>	<input type="checkbox"/>	Short stature	<input type="checkbox"/>	<input type="checkbox"/>
Respiratory support	<input type="checkbox"/>	<input type="checkbox"/>	Functional Disabilities		
Dental			Wheelchair	<input type="checkbox"/>	<input type="checkbox"/>
Premature tooth loss/nontraumatic tooth loss (with root intact)	<input type="checkbox"/>	<input type="checkbox"/>	Uses walking device	<input type="checkbox"/>	<input type="checkbox"/>
Poor/abnormal dentition	<input type="checkbox"/>	<input type="checkbox"/>			

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

INDICATION & IMPORTANT SAFETY INFORMATION for STRENSIQ® (asfotase alfa) 40mg/mL vial

INDICATION

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

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

- **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, skin erythema, rash, pruritus and oral hypoesthesia.

Inform patients and/or caregivers of the signs and symptoms of hypersensitivity reactions and have them seek immediate medical care should signs and symptoms occur. If a severe hypersensitivity reaction occurs, discontinue STRENSIQ treatment and initiate appropriate medical treatment. Consider the risks and benefits of re-administering STRENSIQ to individual patients following a severe reaction. If the decision is made to re-administer the product, monitor patients for a reoccurrence of signs and symptoms of a severe hypersensitivity reaction.

- **Lipodystrophy**: Localized lipodystrophy, including lipoatrophy (depression in the skin) and lipohypertrophy (enlargement or thickening of tissue), 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 or not 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

Overall, the most common adverse reactions ($\geq 10\%$) reported were injection site reactions (63%). Other common adverse reactions included 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 and may be unreliable for clinical decision making.

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.

Please see accompanying full Prescribing Information for STRENSIQ (asfotase alfa), also available at https://alexion.com/Documents/Strensiq_USPI.



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