

**Nova Scotia Provincial Pharmacare Programs
Request for Coverage of Kanuma (sebelipase alfa)**

PATIENT INFORMATION

PATIENT SURNAME	PATIENT GIVEN NAME	HEALTH CARD NUMBER	DATE OF BIRTH
PATIENT ADDRESS			

DIAGNOSTIC INFORMATION

For the treatment of patients diagnosed with lysosomal acid lipase (LAL) deficiency who meet all of the following criteria:

- Documented biochemical evidence of deficient LAL activity and two documented pathogenic mutations in the LIPA gene **AND**
- Have onset of clinical manifestations¹ of LAL deficiency before six months of age. Baseline values _____
- OR**
- Have at least one of the following clinical manifestations¹ of LAL deficiency at 6 months of age and older:
- Persistently elevated transaminases (ALT > 1.5 x ULN² or AST > 1.5 x ULN²) as measured by two assessments three to six months apart Baseline values _____
 - Persistent dyslipidemia (LDL-c and/or TG values in the top 5th percentile based on sex and age) as measured by two assessments three to six months apart Baseline values _____
 - Any documented hepatomegaly or hepatosplenomegaly
 - Liver fibrosis confirmed by biopsy
 - Failure to thrive
 - Growth impairment³
 - Evidence of intestinal affection and/or malabsorption
- AND**
- The patient does not demonstrate any of the following:
- Increased portal vein pressures, or de novo evidence of portal hypertension on ultrasound and Doppler, or new clinical presentation of portal hypertension (e.g., esophageal varices)
 - Severe hepatic dysfunction (Child-Pugh Class C)
 - End-stage liver disease

Renewal request for patients with onset of clinical manifestations of LAL deficiency at six months of age and older:

- 1) Has the patient progressed to end-stage liver failure or multi-organ failure? **yes** **no**
- 2) Does the patient have any of the following response components compared to baseline values after 12 months of therapy?
- Less than 10% improvement in ALT or AST
 - Worsening of liver fibrosis confirmed by biopsy
 - Persisting growth impairment³ despite sebelipase alfa therapy and nutritional interventions
 - At least a 15% increase in spleen volume and/or a greater than 15% increase in liver volume on ultrasound
 - Increased portal vein pressures, or de novo evidence of portal hypertension on ultrasound and doppler, or new clinical presentation of portal hypertension (e.g., esophageal varices).

Clinical Notes:

- The physician must provide baseline values for the clinical manifestation at the time of initial request for reimbursement.
- Based on age- and- sex-specific normal values for ALT and AST
- Growth impairment is defined as decreased body weight across at least two of the major centiles on a WHO weight-for-age chart, or body weight below 10th centile and no weight gain within two weeks and/or decreased height across at least two of the major centiles on a WHO height-for-age chart.

PRESCRIBER NAME & ADDRESS:	
_____	_____
LICENCE #	PRESCRIBER'S SIGNATURE

	DATE

If you need assistance, please contact the Pharmacare Office at (902) 496-7001 or 1-800-305-5026

Please return form to: Nova Scotia Pharmacare Programs
P.O. Box 500, Halifax, NS B3J 2S1
Fax: (902) 496-4440

