HOSPITAL MEDICINES LIST RESTRICTIONS CHECKLIST

Use this checklist to determine if a patient meets the restrictions for funding in the hospital setting. For more details, refer to Section H of the Pharmaceutical Schedule. For community funding, see the Special Authority Criteria.

PRESCRIBER	PATIENT:	
Name:	Name:	
Ward:	NHI:	

Alglucosidase Alfa

INITIATION Re-assessment required after 12 months Prerequisites (tick boxes where appropriate) Prescribed by, or recommended by a metabolic physician, or in accordance with a protocol or guideline that has been endorsed by the Health NZ Hospital. and () The patient is aged up to 24 months at the time of initial application and has been diagnosed with infantile Pompe disease and () Diagnosis confirmed by documented deficiency of acid alpha-glucosidase by prenatal diagnosis using chorionic villus biopsies and/or cultured amniotic cells or Documented deficiency of acid alpha-glucosidase, and urinary tetrasaccharide testing indicating a diagnostic elevation of glucose tetrasaccharides or Documented deficiency of acid alpha-glucosidase, and documented molecular genetic testing indicating a disease-causing mutation in the acid alpha-glucosidase gene (GAA gene) or Documented urinary tetrasaccharide testing indicating a diagnostic elevation of glucose tetrasaccharides, and molecular genetic testing indicating a disease-causing mutation in the GAA gene and) Patient has not required long-term invasive ventilation for respiratory failure prior to starting enzyme replacement therapy (ERT) and () Patient does not have another life-threatening or severe disease where the prognosis is unlikely to be influenced by ERT or might be reasonably expected to compromise a response to ERT and Alglucosidase alfa to be administered at doses no greater than 20 mg/kg every 2 weeks

CONTINUATION Re-assessment required after 12 months Prereguisites (tick boxes where appropriate)			
and	O Prescribed by, or recommended by a metabolic physician, or in accordance with a protocol or guideline that has been endorsed by the Health NZ Hospital.		
	O	The treatment remains appropriate for the patient and the patient is benefiting from treatment	
	and	Alglucosidase alfa to be administered at doses no greater than 20 mg/kg every 2 weeks	
	and	Patient has not had severe infusion-related adverse reactions which were not preventable by appropriate pre-medication and/or adjustment of infusion rates	
	and	Patient has not developed another life threatening or severe disease where the long term prognosis is unlikely to be influenced by ERT	
	and	Patient has not developed another medical condition that might reasonably be expected to compromise a response to ERT	
	and O	There is no evidence of life threatening progression of respiratory disease as evidenced by the needed for > 14 days of invasive ventilation	
		There is no evidence of new or progressive cardiomyopathy	