

APPLICANT (stamp or sticker acceptable) **PATIENT NHI:** **REFERRER** Reg No:

Reg No: First Names: First Names:

Name: Surname: Surname:

Address: DOB: Address:

..... Address:

.....

Fax Number: Fax Number:

Alglucosidase Alfa

Initial application

Applications only from a metabolic physician. Approvals valid for 12 months.

Prerequisites(tick boxes where appropriate)

- 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

Renewal

Current approval Number (if known):.....

Applications only from a metabolic physician. Approvals valid for 12 months.

Prerequisites(tick boxes where appropriate)

- 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
- There is no evidence of life threatening progression of respiratory disease as evidenced by the needed for > 14 days of invasive ventilation
- and
- There is no evidence of new or progressive cardiomyopathy

I confirm the above details are correct and that in signing this form I understand I may be audited.

Signed: Date:

Post application to Ministry of Health, Private Bag 3015, Wanganui – email: customerservice@health.govt.nz