

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