

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:

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

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 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 that the above details are correct:

Signed: Date: