Clinical Oversight Review Board (CORB) Criteria for Prescribing

Alglucosidase alfa (Lumizyme)

Non-Formulary **alglucosidase alfa (Lumizyme)** requires a clinical review. Appropriateness of therapy will be based on the following criteria:

Initiation (new start) criteria: Non-formulary **alglucosidase alfa (Lumizyme)** will be covered on the prescription drug benefit for 12 months when the following criteria are met:

- Prescriber is metabolic specialist or geneticist.
- Patient has a diagnosis of Pompe disease (Acid alpha-glucosidase (GAA) deficiency)
- Diagnosis has been confirmed by one of the following:
  - Deficiency of acid alpha-glucosidase (GAA) enzyme activity; OR
  - Detection of biallelic pathogenic variants in the GAA gene by molecular genetic testing.
- Documented baseline values for one or more of the following:
  - Infantile-onset disease: muscle weakness, motor function, respiratory function, cardiac involvement, percent predicted forced vital capacity (FVC), or 6 minute walk test (6MWT)
  - Late-onset (non-infantile) disease: FVC or 6MWT.
- Will not be used in combination with other enzyme replacement therapies (i.e., avalglucosidase alfa-ngpt)

Criteria for *new members* entering Kaiser Permanente already taking the medication who have not been reviewed previously: Non-formulary **alglucosidase alfa (Lumizyme)** will be covered on the prescription drug benefit when the following criteria are met:

- Prescriber is metabolic specialist or geneticist.
- Patient has a diagnosis of Pompe disease (Acid alpha-glucosidase (GAA) deficiency)
- Will not be used in combination with other enzyme replacement therapies (i.e., avalglucosidase alfa-ngpt)

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Continued use criteria (12 months after initiation): Non-formulary **alglucosidase alfa (Lumizyme)** will continue to be covered on the prescription drug benefit for 12 months when the following criteria are met:

- Patient has demonstrated a beneficial response to therapy compared to pretreatment baseline in one or more of the following:
  - Infantile-onset disease: stabilization or improvement in muscle weakness, motor function, respiratory function, cardiac involvement, FVC, and/or 6MWT
  - Late-onset (non-infantile) disease: stabilization or improvement in FVC and/or 6MWT