RATIONALE FOR INCLUSION IN PA PROGRAM

Background
Myozyme is indicated for the treatment for patients with Pompe disease, a rare but severely debilitating disease. Pompe disease is caused by the deficiency of lack of the enzyme acid alpha-glucosidase, which is essential for normal muscle development and function. The disease, which usually results in death from respiratory failure, is rapidly fatal in newborn babies. Myozyme provides an exogenous source of GAA (acid alpha glucosidase), replacing the deficient GAA (1).

Regulatory Status
FDA-approved indication: Myozyme (alglucosidase alfa) is a lysosomal glycogen-specific enzyme indicated for use in patients with Pompe disease (GAA deficiency).

Myozyme carries a boxed warning regarding the risk of cardiorespiratory failure. Appropriate medical support should be readily available to initiate treatment for life threatening anaphylactic, severe allergic and immune mediated reactions. Medical support should also be available due to the risk for acute cardio respiratory failure (1).

Pediatric patients aged 1 month to 3.5 years at time of first infusion have been treated with Myozyme in clinical trials. Other open-label clinical trials of Myozyme have been performed in older pediatric patients ranging from 2 to 16 years at the initiation of treatment (juvenile-onset Pompe disease); however, the risks and benefits of Myozyme treatment have not been established in the juvenile-onset Pompe disease population (1).

Summary
Pompe disease is a rare, inherited, and often fatal disorder that disables the heart and muscles. It is caused by mutations in a gene that makes an enzyme called alpha-glucosidase (GAA). The severity of the disease and the age of onset are related to the degree of enzyme deficiency. Enzyme replacement therapy has been developed that has shown, in clinical trials with infantile-onset patients, to decrease heart size, maintain normal heart function, improve muscle function, tone, and strength, and reduce glycogen accumulation (1).

Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Myozyme while maintaining optimal therapeutic outcomes.
MYOZYME
(alglucosidase alfa)

References