I. Medication Description

Fabry disease is an X-linked genetic disorder of glycosphingolipid metabolism. Deficiency of the lysosomal enzyme α-galactosidase A leads to progressive accumulation of glycosphingolipids, predominantly GL-3, in many body tissues, starting early in life and continuing over decades. Clinical manifestations of Fabry disease include renal failure, cardiomyopathy, and cerebrovascular accidents. Accumulation of GL-3 in renal endothelial cells may play a role in renal failure.

Fabrazyme (agalsidase-beta) is intended to provide an exogenous source of α-galactosidase A in Fabry disease patients. Nonclinical and clinical studies evaluating a limited number of cell types indicate that Fabrazyme will catalyze the hydrolysis of glycosphingolipids, including GL-3.

II. Position Statement

Coverage is determined through a prior authorization process with supporting clinical documentation for every request.

III. Policy

Coverage of Fabrazyme is available when the following criteria have been met:

- Member is 8 years or age or older AND
- The medication is prescribed by or in consultation with a geneticist, cardiologist, nephrologist or physician who specializes in the treatment of Fabry Disease AND
- Member has clinical features of Fabry disease (e.g. neuropathy, dermatologic manifestations, ocular findings [e.g. cornea verticillata, cataract, etc], renal dysfunction, cardiac manifestations, cerebrovascular involvement, diminished perspiration, etc) OR
- Member has a family history suggestive of Fabry disease AND
- Biochemical or genetic confirmation has been documented by one of the following:
  - Low alpha-galactosidase A (alpha-Gal A) activity in leukocytes, dried blood spots, or plasma confirming diagnosis (males only) OR
  - Detection of galactosidase alpha (GLA) gene mutation by gene sequencing AND
- Baseline value(s) for plasma globotriaosylceramide (GL-3) and/or GL-3 inclusions have been submitted, if available AND
- Fabrazyme will not be used in combination with Galafold (migalastat).
IV. **Quantity Limitations**

Coverage is available to provide sufficient quantities to allow up to 1mg/kg body weight every two weeks.

V. **Coverage Duration**

Coverage is available for 6 months and may be renewed.

VI. **Coverage Renewal Criteria**

Coverage can be renewed in 6-month increments based upon the following criteria:

- Stabilization of disease or in absence of disease progression:
  - Improvements in plasma GL-3 and/or GL-3 inclusions compared to pre-treatment baseline, if available **AND**
  - Documented member-specific improved clinical symptoms/outcomes **AND**
- Absence of unacceptable toxicity from the drug.

VII. **Billing/Coding Information**

- Fabrazyme is available as
  - 5mg single-use vial for injection
  - 35mg single use vial for injection
- J0180: 1 billable unit=1mg
- Pertinent diagnoses:
  - E75.21: Fabry (-Anderson) disease

VIII. **Summary of Policy Changes**

5/15/19: new policy

IX. **References**

2. UpToDate. Fabry Disease: Clinical features and diagnosis. Accessed March 2019

*These guidelines are not applicable to benefits covered under Medicare Advantage. Medicare Advantage benefit coverage requests are reviewed in accordance with the guidance set forth in Chapter 15 Section 50 of the Centers for Medicare & Medicaid Services Medicare Benefit Policy Manual.*
The Plan fully expects that only appropriate and medically necessary services will be rendered. The Plan reserves the right to conduct pre-payment and post-payment reviews to assess the medical appropriateness of the above-referenced therapies.

The preceding policy applies only to members for whom the above named pharmacy benefit medications are included on their covered formulary. Members with closed formulary benefits are subject to trying all appropriate formulary alternatives before a coverage exception for a non-formulary medication will be considered.

The preceding policy is a guideline to allow for coverage of the pertinent medication/product, and is not meant to serve as a clinical practice guideline.