CVS/caremark™

SPECIALTY GUIDELINE MANAGEMENT

FABRAZYME (agalsidase beta)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Fabrazyme is indicated for use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types.

All other indications are considered experimental/investigational and are not a covered benefit.

II. REQUIRED DOCUMENTATION

The following information is necessary to initiate the prior authorization review: enzyme assay or genetic testing results supporting diagnosis.

III. CRITERIA FOR INITIAL APPROVAL

A. Fabry disease

Authorization for 12 months may be granted for treatment of Fabry disease when all of the following criteria are met:

1. Diagnosis of Fabry disease was confirmed by enzyme assay demonstrating a deficiency of alpha-galactosidase enzyme activity or by genetic testing, or the member is an obligate female carrier with a first degree male relative diagnosed with Fabry disease.
2. Member exhibits clinical signs and symptoms of Fabry disease (see Appendix)

IV. CONTINUATION OF THERAPY

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

V. DOSAGE AND ADMINISTRATION

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

VI. APPENDIX: Classic Manifestations of Fabry disease

- Intermittent paresthesia and acroparesthesia (eg, chronic, burning, tingling pain in the hands and/or feet)
- Episodic Fabry crises (ie, severe, incapacitating pain, lasting from days to weeks; rare in female carriers)
- Angiokeratomas (ie, small, slightly raised, purplish-red, nonblanching telangiectases)
- Whorled corneal opacity
- Gastrointestinal problems (eg, diarrhea, abdominal discomfort, vomiting, nausea)
- Hypohidrosis or anhidrosis
- Heat, cold, and/or exercise intolerance
- Renal dysfunction (ranging from mild proteinuria to end-stage renal disease)
- Cardiovascular dysfunction (eg, myocardial infarction, valvular abnormalities, arrhythmias, left ventricular hypertrophy)
- Cerebrovascular complications (eg, stroke, hemiplegia, hemianesthesia, and transient ischemic attacks)
- Pulmonary complications (eg, airflow obstruction, dyspnea)

VII. REFERENCES

1. Fabrazyme [package insert]. Cambridge, MA: Genzyme Corporation; May 2010.
2. Desnick RJ, Brady RO. Fabry disease in childhood. *J Pediatr*. 2004;144(5 Suppl):S20-S26.