

Fabry Disease

Goal(s):

- Ensure medically appropriate use of drugs for Fabry Disease

Length of Authorization:

- Up to 12 months

Requires PA:

- Agalsidase beta (pharmacy and physician administered claims) and migalastat

Covered Alternatives:

- Current PMPDP preferred drug list per OAR 410-121-0030 at www.orpdl.org
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is this an FDA approved indication?	Yes: Go to #3	No: Pass to RPh. Deny; medical appropriateness
3. Is this a request for continuation of therapy?	Yes: Go to Renewal Criteria	No: Go to #4
4. Is the provider a specialist in managing Fabry disease?	Yes: Go to #5	No: Pass to RPh. Deny; medical appropriateness
5. Is the request for migalastat?	Yes: Go to #6	No: Go to #9
6. Does the patient have a mutation that is amenable to migalastat therapy as confirmed by a genetic specialist?	Yes: Got to #7	No: Pass to RPh. Deny; medical appropriateness
7. Is the patient currently receiving agalsidase beta?	Yes: Pass to RPh. Deny; medical appropriateness	No: Go to #8
8. Is the patient 18 years of age or older?	Yes: Approve for 6 months	No: Pass to RPh. Deny; medical appropriateness. Migalastat is only FDA-approved for use in adults.

Approval Criteria		
9. Is the patient a male at least 2 years of age with diagnosis of Fabry disease confirmed by genetic testing or deficiency in alpha-galactosidase A enzyme activity in plasma or leukocytes?	Yes: Go to #10	No: Go to #11
10. Does the patient have end stage renal disease requiring dialysis?	Yes: Pass to RPh. Deny; medical appropriateness	No: Approve for 12 months
11. Is the patient a female at least 2 years of age and a documented Fabry disease carrier confirmed by genetic testing with significant clinical manifestations of Fabry disease such as: <ul style="list-style-type: none"> • Uncontrolled pain that interferes with quality of life • Gastrointestinal symptoms that are significantly reducing quality of life and not attributable to other pathology • Mild to moderate renal impairment (GFR > 30 mL/min) • Cardiac disease (left ventricular hypertrophy, conduction abnormalities, ejection fraction < 50%, arrhythmias) • Previous stroke or TIA with retained neurologic function 	Yes: Approve for 6 months	No: Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Has the patient's condition improved as assessed by the prescribing provider and provider attests to patient's improvement in one of the following: <ul style="list-style-type: none"> • Renal function • Pain Scores • Quality of Life measurement • Cardiac function • Neurologic status • Growth and development in children 	Yes: Approve for 12 months. Document baseline assessment and provider attestation received.	No: Pass to RPh. Deny; medical appropriateness