MIGALASTAT

Generic	Brand	HICL	GCN	Exception/Other
MIGALASTAT	GALAFOLD	44433		

GUIDELINES FOR USE

INITIAL CRITERIA (NOTE: FOR RENEWAL CRITERIA SEE BELOW)

1. Does the patient have a diagnosis of Fabry disease and meet **ALL** of the following criteria?
   - The patient is 18 years or older
   - The patient has an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data as interpreted by clinical genetics professional as pathogenic/likely pathogenic (i.e., patient does not have a benign amenable GLA variant)
   - The requested medication is prescribed by or in consultation with a nephrologist, cardiologist, or specialist physician in genetics or inherited metabolic disorders
   - The patient is NOT concurrently using enzyme replacement therapy (i.e., Fabrazyme)
   - The patient is symptomatic **OR** has evidence of injury from GL-3 to the kidney, heart, or central nervous system recognized by laboratory, histological, or imaging findings (e.g., decreased GFR for age, persistent albuminuria, cerebral white matter lesions on brain MRI, cardiac fibrosis on contrast cardiac MRI)

   If yes, continue to #2.
   If no, do not approve.
   DENIAL TEXT: See the initial denial text at the end of the guideline.

2. Is the request for a female patient who meets the following criteria?
   - Confirmation of Fabry disease via genetic test documenting galactosidase alpha gene (GLA) mutation

   If yes, **approve for 6 months by HICL with a quantity limit of #14 capsules per 28 days.**
   If no, continue to #3.

3. Is the request for a male patient who meets **ONE** of the following criteria?
   - Confirmation of Fabry disease via enzyme assay indicating deficiency of alpha galactosidase A (a-Gal -A)
   - Confirmation of Fabry disease via genetic test documenting galactosidase alpha gene (GLA) mutation

   If yes, **approve for 6 months by HICL with a quantity limit of #14 capsules per 28 days.**
   If no, do not approve.
   INITIAL DENIAL TEXT: See the initial denial text at the end of the guideline.

CONTINUED ON NEXT PAGE

MIGALASTAT

INITIAL CRITERIA (CONTINUED)

INITIAL DENIAL TEXT: The guideline named **MIGALASTAT (Galafold)** requires a diagnosis of Fabry disease. In addition, the following criteria must be met:

- The patient is 18 years or older
- The patient has an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data as interpreted by clinical genetics professional as pathogenic/likely pathogenic (i.e., patient does not have a benign amenable GLA variant)
- The requested medication is prescribed by or in consultation with a nephrologist, cardiologist, or specialist physician in genetics or inherited metabolic disorders
- The patient is NOT concurrently using enzyme replacement therapy (i.e., Fabrazyme)
- The patient is symptomatic OR has evidence of injury from GL-3 to the kidney, heart, or central nervous system recognized by laboratory, histological, or imaging findings (e.g., decreased GFR for age, persistent albuminuria, cerebral white matter lesions on brain MRI, cardiac fibrosis on contrast cardiac MRI)
- The patient meets one of the following:
  - Female patients: Confirmation of Fabry disease via genetic test documenting galactosidase alpha gene (GLA) mutation
  - Male patients: Confirmation of Fabry disease via enzyme assay indicating deficiency of alpha galactosidase A (a-Gal -A) or genetic test documenting galactosidase alpha gene (GLA) mutation

RENEWAL CRITERIA

1. Does the patient have a diagnosis of Fabry disease and meet the following criteria?
   - The prescribing provider attests that the patient **has demonstrated improvement or maintenance/stabilization** while on therapy in regards to at least **ONE** of the following:
     - Symptoms (e.g., pain, hypohidrosis/anhidrosis, exercise intolerance, GI symptoms, angiokeratomas, abnormal cornea, tinnitus/hearing loss)
     - Imaging (e.g., brain/cardiac MRI, DEXA, renal ultrasound)
     - Laboratory or histological testing (e.g., GL-3 in plasma/urine, renal biopsy)

   If yes, **approve for 12 months by HICL with a quantity limit of #14 capsules per 28 days.** If no, do not approve.
   RENEWAL DENIAL TEXT: The guideline named **MIGALASTAT (Galafold)** requires a diagnosis of Fabry disease for renewal. In addition, the following criteria must be met:
   - The prescribing provider attests that the patient has demonstrated improvement or maintenance/stabilization while on therapy in regards to at least one of the following:
     - Symptoms (e.g., pain, hypohidrosis/anhidrosis, exercise intolerance, GI symptoms, angiokeratomas, abnormal cornea, tinnitus/hearing loss)
     - Imaging (e.g., brain/cardiac MRI, DEXA, renal ultrasound)
     - Laboratory or histological testing (e.g., GL-3 in plasma/urine, renal biopsy)

CONTINUED ON NEXT PAGE

MIGALASTAT

RATIONALE

For further information, please refer to the Prescribing Information and/or Drug Monograph for Galafold.

REFERENCES

- Galafold [Prescribing Information]. Cranbury, NJ: Amicus Therapeutics; August 2018.

Library	Commercial	NSA
Yes	Yes	No

Part D Effective: N/A
Commercial Effective: 01/01/19
Created: 11/18
Client Approval: 11/18
P&T Approval: 10/18