Pharmacy Medical Necessity Guidelines: Galafold™ (migalastat)

Effective: January 12, 2021

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RxUM

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

RXUM: 617.673.0988

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMISTRATION (FDA)-APPROVED INDICATIONS

Galafold (migalastat) is an alpha-galactosidase A (alpha-Gal A) pharmacological chaperone indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

Fabry disease (also referred to as Anderson-Fabry disease) is a rare genetic lysosomal disorder caused by the body's inability to produce a specific enzyme (alpha-galactosidase A) responsible for the degradation of glycosphingolipids. In affected individuals, the missing enzyme prevents the normal breakdown and recycling of cells resulting in the storage of these cell deposits in cells of the kidney, heart, skin, eyes, gastrointestinal system, and central and peripheral nervous system. As a result of the storage, cells do not perform properly and may cause progressive damage throughout the body. The signs and symptoms of this condition develop with age as more cells become damaged by the accumulation of cell deposits. The incidence of Fabry disease is reported to be 1 in 40,000 to 60,000 and is most typically seen in males.

Galafold (migalastat) is designed to restore alpha-galactosidase A activity in patients who have amenable mutations, so it can clear the accumulation of disease substrate.

COVERAGE GUIDELINES

The plan may authorize coverage of Galafold (migalasta) for Members when the following criteria are met:

1. Documented diagnosis of Fabry disease

AND

2. Documentation the Member has an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data

AND

3. The prescribing physician is a nephrologist, cardiologist, or a physician specializing in metabolic disorders or genetics

AND

4. The Member is at least 18 years of age

LIMITATIONS

1. Galafold (migalastat) will not be authorized in combination with enzyme replacement therapy.

CODES

None

REFERENCES

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6. Germain DP, Hughes DA, Nicholls K et al. Treatment of Fabry's disease with the pharmacologic chaperone migalastat. *N Engl J Med*. 2016; 375(6):545-55.
7. Hopkin RJ, Jefferies JL, Laney DA et al. The management and treatment of children with Fabry disease: a United States-based perspective. *Mol Genet Metab*. 2016; 117(2):104-13.
8. Hughes DA, Nicholls K, Shankar SP et al. Oral pharmacological chaperone migalastat compared with enzyme replacement therapy in Fabry disease: 18-month results from the randomised phase III ATTRACT study. *J Med Genet*. 2017; 54(4):288-296.
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APPROVAL HISTORY

December 11, 2018: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
1. September 10, 2019: No changes
2. June 9, 2020: No changes
3. January 12, 2021: No changes

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.