Galafold (migalastat)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the Galafold (migalastat) drug policy is to ensure appropriate selection of patients for therapy based on product labeling, clinical guidelines and clinical studies. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Galafold is 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.

POLICY

Required Documentation

1. Medical records, enzyme assay, or genetic testing confirming diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data
2. Submission of medical records (e.g. chart notes, laboratory values) documenting positive clinical response to therapy as defined in continuation criteria

Criteria for Initial Approval

A. Galafold (migalastat) may be considered medically necessary for the treatment of Fabry disease when ALL of the following criteria are met:
    1. Must be prescribed by or in consultation with a geneticist, nephrologist, or a physician who specializes in the management of Fabry disease
    2. Must be 16 years of age or older

3. The 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 a symptomatic obligate carrier
4. Patient has an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data
5. Patient must not have severe renal impairment (eGFR <30 mL/minute/1.73 m^2) or end-stage renal disease requiring dialysis
6. Patient will not be receiving Galafold in combination with Fabrazyme (agalsidase beta)

Approval will be for **12 months**

Continuation of Therapy

A. Galafold (migalastat) may be considered medically necessary for the continuation of treatment of Fabry disease when ALL of the following criteria are met:
   1. Patient has a documented positive clinical response to therapy (e.g.,lab results demonstrating a reduction in plasma globotriaosylceramide [GL-3] or GL-3 inclusions, progress notes documenting improvement and/or stabilization in clinical symptoms, improvement and/or stabilization in renal function, pain reduction).
   2. Patient is not receiving Galafold in combination with Fabrazyme (agalsidase beta)

Approval will be for **12 months**

Dosing and Administration

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

Quantity Limit

14 capsules/28 days

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

REFERENCES

- Galafold [package insert]. Cranbury, NJ: Amicus Therapeutics U.S., Inc.; December 2021.
- Desnick RJ, Brady RO. Fabry disease in childhood*. J Pediatr*. 2004;144(5 Suppl):S20-S26.
- Biegstraaten M, Arngrimsson R, Barbey F, et al. Recommendations for initiation and cessation of enzyme replacement therapy in patients with Fabry disease: the European Fabry Working Group consensus document. *Orphanet J Rare Dis.* 2015; 1036.
- Desnick RJ, Brady R, Barranger J, et al. Fabry disease, an under-recognized multisystemic disorder: expert recommendations for for diagnosis, and enzyme repalacement therapy. *Ann Intern Med.* 2003; 138(4):338.
- Sirrs, S, Bichet DG, Iwanochko RM, et al. Canadian Fabry disease treatment guidelines 2016.
- Feldt-Rasmussen U, Hughes D, Sunder-Plassmann G et al. Long-term efficacy and safety of migalastat treatment in Fabry disease: 30-month results from the open-label extension of the randomized, phase 3 ATTRACT study. *Mol Genet Metab*. 2020;131(1-2):219-228. Accessed December 21, 2020.

*Some content reprinted from CVSHealth

POLICY HISTORY

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