

| Clinical Policy Title: | migalastat |
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| Policy Number: | RxA.149 |
| Drug(s) Applied: | Galafold® |
| Original Policy Date: | 02/07/2020 |
| Last Review Date: | 08/28/2024 |
| Line of Business Policy Applies to: | All lines of business (except Medicare) |

Criteria

١. **Initial Approval Criteria**

A. Fabry Disease (must meet all):

- 1. Diagnosis of Fabry disease confirmed by one of the following (a or b):
 - a. Enzyme assay demonstrating a deficiency of alpha-galactosidase activity;
 - b. DNA testing;
- 2. Presence of at least one amenable GLA variant (mutation), as confirmed by one of the following resources (a, b, or c):
 - Galafold[®] Prescribing Information brochure (package insert; Section 12, Table 2);
 - b. Amicus Fabry GLA Gene Variant Search Tool: <u>http://www.galafoldamenabilitytable.com/hcp</u>;
 - c. Amicus Medical Information at 1-877-4AMICUS or medinfousa@amicusrx.com;
- 3. Galafold[®] is not prescribed concurrently with Fabrazyme[®].

Approval Duration

All Lines of Business (except Medicare): 6 months

II. **Continued Therapy Approval**

- A. Fabry Disease (must meet all):
 - 1. Auto-approval based on lookback functionality within the past 120 days as a proxy for member responding positively to therapy.

Approval Duration All Lines of Business (except Medicare): 12 months

References

1. Hopkin RJ, Jefferies JL, Laney DA, et al. on behalf of the Fabry Pediatric Expert Panel. The management and treatment of children with Fabry disease: A United States-based perspective. Molecular Genetics and Metabolism. February 2016; 117(2): 104-113. Available at: https://doi.org/10.1016/j.ymgme.2015.10.007. Accessed August 28, 2024.

| Review/Revision History | Review/Revised Date | P&T Approval Date |
|--|---------------------|-------------------|
| Policy established. | 1/2020 | 02/07/2020 |
| Policy was reviewed:1. Continued Therapy criteria II.A.1 was rephrased to "Currently receiving medication that has been | 06/15/2020 | 09/14/2020 |

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.

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| authorized by RxAdvance"2. References reviewed and updated. | | |
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| Policy was reviewed: 1. Continued therapy approval criteria II.A.1 was updated to "Member is currently receiving medication". 2. References updated. | 03/31/2021 | 06/10/2021 |
| Policy was reviewed: 1. Initial Approval Criteria I.A.2 was updated to add nephrologist, or a physician who specializes in the treatment of Fabry disease. 2. References were reviewed and updated. | 01/10/2022 | 04/18/2022 |
| Policy was reviewed: 1. Initial Approval Criteria, I.A.1: Updated diagnostic criteria from Diagnosis of Fabry disease to Diagnosis of Fabry disease confirmed by one of the following (a or b): a. Enzyme assay demonstrating a deficiency of alphagalactosidase activity; b. DNA testing. 2. Initial Approval Criteria, I.A.2: Updated prescriber criteria from Prescribed by or in consultation with a clinical geneticist, nephrologist or a physician who specializes in the treatment of Fabry disease to Prescribed by or in consultation with a clinical geneticist, cardiologist, nephrologist, neurologist, or a physician who specializes in the treatment of Fabry disease. 3. References were reviewed and updated. | 01/18/2023 | 04/13/2023 |
| Policy was reviewed. | 10/19/2023 | 10/19/2023 |
| Policy was reviewed: Removed age restrictions. Removed prescriber restrictions. Removed dose restrictions. Updated Continued therapy approval with auto- approval based on lookback functionality within the past 120 days. Removed reauthorization requirement for positive response to therapy. Updated approval duration verbiage. References were reviewed and updated. | 08/28/2024 | 09/13/2024 |