

Pharmacy Medical Necessity Guidelines: Fabrazyme® (agalsidase beta)

Effective: September 15, 2020

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	MED	Department to Review	PRECERT / MM
<p>These pharmacy medical necessity guidelines apply to the following:</p> <p>Commercial Products</p> <ul style="list-style-type: none"> <input checked="" type="checkbox"/> Tufts Health Plan Commercial products – large group plans <input checked="" type="checkbox"/> Tufts Health Plan Commercial products – small group and individual plans <input checked="" type="checkbox"/> Tufts Health Freedom Plan products – large group plans <input checked="" type="checkbox"/> Tufts Health Freedom Plan products – small group plans • CareLinkSM – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization <p>Tufts Health Public Plans Products</p> <ul style="list-style-type: none"> <input checked="" type="checkbox"/> Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product) <input checked="" type="checkbox"/> Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans <input checked="" type="checkbox"/> Tufts Health RITogether – A Rhode Island Medicaid Plan 		<p>Fax Numbers:</p> <p>All plans except Tufts Health Public Plans: Precert: 617.972.9409</p> <p>Tufts Health Public Plans only: MM: 888.415.9055</p>	

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

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

Fabrazyme (agalsidase beta) is indicated for use in patients with Fabry disease. Fabrazyme reduces globotriaosylceramide (GL-3) deposition in capillary endothelium of the kidney and certain other cell types.

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 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.

Fabrazyme (agalsidase beta) is intended to provide an exogenous source of the enzyme, deficient in patients with Fabry disease, responsible for breaking down glycosphingolipids including GL-3.

COVERAGE GUIDELINES

The plan may authorize coverage of Fabrazyme (agalsidase beta) for Members when the all of the following criteria are met:

1. The Member must have the definitive diagnosis of Fabry disease
- AND**
2. The prescribing physician must be a nephrologist, cardiologist, or from a physician specializing in metabolic disorders or genetics

LIMITATIONS

- Fabrazyme (agalsidase beta) will not be authorized in combination with Galafold (migalastat).

CODES

The following HCPCS/CPT code(s) are:

Code	Description
J0180	Injection, agalsidase beta, 1 mg

REFERENCES

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18. Schiffmann R, Martin RA, Reimschisel T et al. Four-year prospective clinical trial of agalsidase alfa in children with Fabry disease. *J Pediatr*. 2010 May; 156(5):832-7, 837.e1.
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20. Wanner C, Arad M, Baron R et al. European expert consensus statement on therapeutic goals in Fabry disease. *Mol Genet Metab*. 2018; 124(3):189-203.
21. Wyatt K, Henley W, Anderson L et al. The effectiveness and cost-effectiveness of enzyme and substrate replacement therapies: a longitudinal cohort study of people with lysosomal storage disorders. *Health Technol Assess*. 2012; 16(39):1-543.

APPROVAL HISTORY

January 2004: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

1. January 11, 2005: No changes
2. January 10, 2006: No changes
3. December 12, 2006: No changes
4. November 13, 2007: No changes
5. November 11, 2008: No changes
6. November 10, 2009: No changes
7. January 1, 2010: Removal of Tufts Medicare Preferred language (separate criteria have been created specifically for Tufts Medicare Preferred)
8. September 14, 2010: No changes
9. July 12, 2011: No changes

10. June 12, 2012: No changes
11. May 14, 2013: No changes
12. May 13, 2014: No changes
13. May 12, 2015: No changes
14. January 1, 2016: Administrative change to rebranded template.
15. April 12, 2016: No changes
16. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
17. March 13, 2018: No changes
18. December 11, 2018: Added the following limitation: Fabrazyme (agalsidase beta) will not be authorized in combination with Galafold (migalastat).
19. September 10, 2019: No changes
20. September 15, 2020: 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.