

**Galafold (migalastat)  
Effective 12/01/2019**

<b>Plan</b>	<input checked="" type="checkbox"/> MassHealth <input type="checkbox"/> Commercial/Exchange	<b>Program Type</b>	<input checked="" type="checkbox"/> Prior Authorization <input type="checkbox"/> Quantity Limit <input type="checkbox"/> Step Therapy
<b>Benefit</b>	<input checked="" type="checkbox"/> Pharmacy Benefit <input type="checkbox"/> Medical Benefit (NLX)		
<b>Specialty Limitations</b>	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
<b>Contact Information</b>	<b>Specialty Medications</b>		
	All Plans	Phone: 866-814-5506	Fax: 866-249-6155
	<b>Non-Specialty Medications</b>		
	MassHealth	Phone: 877-433-7643	Fax: 866-255-7569
	Commercial	Phone: 800-294-5979	Fax: 888-836-0730
	Exchange	Phone: 855-582-2022	Fax: 855-245-2134
	<b>Medical Specialty Medications (NLX)</b>		
	All Plans	Phone: 844-345-2803	Fax: 844-851-0882
<b>Exceptions</b>	N/A		

### Overview

Migalastat is FDA indicated for Fabry disease. It is an oral pharmacological chaperone that stabilizes certain mutant variants of alpha-galactosidase to increase enzyme trafficking to lysosomes. Migalastat reversibly binds to the active site of the alpha-galactosidase A (alpha-Gal A) protein (encoded by the galactosidase alpha gene, GLA), which is deficient in Fabry disease. Binding to the active site stabilizes alpha-Gal A allowing trafficking from the endoplasmic reticulum into the site of action, the lysosome.

### Coverage Guidelines

Authorizations will be granted for members who are currently receiving treatment with Galafold, excluding when the product is obtained as samples or via manufacturer’s patient assistance programs  
**OR**

Authorizations will be granted if the member meets all the following criteria and documentation has been submitted:

1. The member is at least 18 years of age
2. The member is diagnosed with Fabry disease
3. The prescriber is a clinical genetics specialist or nephrologist or consult with either specialist is provided
4. Results from enzyme assay test showing reduced or absent  $\alpha$ -galactosidase A ( $\alpha$ -GAL) enzyme activity in plasma, leukocytes, tears, or biopsied tissue are submitted
5. The member has GLA mutations which are amenable to treatment with Galafold

### Limitations

1. Approvals will be granted for 12 months
2. Galafold will not be authorized in combination with enzyme replacement therapy (ERP)



## References

1. Galafold (migalastat) [prescribing information]. Cranbury, NJ: Amicus Therapeutics US Inc; March 2020
2. 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):545-555
3. 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; 117:104
4. Terryn W, Cochat P, Froissart R, et al. Fabry nephropathy: indications for screening and guidance for diagnosis and treatment by the European Renal Best Practice. *Nephrol Dial Transplant* 2013; 28:505
5. McCafferty EH, Scott LJ. Migalastat: A Review in Fabry Disease. *Drugs* 2019; 79:543
6. Benjamin ER, Della Valle MC, Wu X, et al. The validation of pharmacogenetics for the identification of Fabry patients to be treated with migalastat. *Genet Med* 2017; 19:430

## Review History

09/18/19 – Reviewed

09/16/20 – Reviewed at P&T

## Disclaimer

AllWays Health Partners complies with applicable federal civil rights laws and does not discriminate or exclude people on the basis of race, color, national origin, age, disability, or sex.