Galafold (migalastat)

Background
Galafold (migalastat) is a pharmacological chaperone that 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. This binding stabilizes alpha-Gal A allowing its trafficking into the lysosome where it exerts its action. Certain GLA variants (mutations) causing Fabry disease result in the production of abnormally folded and less stable forms of the alpha-Gal A protein which, however, retain enzymatic activity. Those GLA variants, referred to as amenable variants, produce alpha-Gal A proteins that may be stabilized by Galafold thereby restoring their trafficking to lysosomes and their intralysosomal activity (1).

Regulatory Status
FDA-approved indication: Galafold 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 (1).

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 (1).

The safety and effectiveness of Galafold in pediatric patients have not been established (1).
This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Galafold may be considered medically necessary in patients 18 years of age and older with Fabry disease and if the conditions indicated below are met.

Galafold is considered investigational in patients less than 18 years of age and for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

The patient must have the following:

   Fabry disease

   AND the following:
   Patient has an amenable galactosidase alpha gene (GLA) variant based on an in vitro assay

Prior – Approval Renewal Requirements

Age 18 years of age and older

Diagnosis

The patient must have the following:

   Fabry disease

Policy Guidelines

Pre - PA Allowance

None
Prior - Approval Limits

Quantity  42 capsules per 84 days

Duration  12 months

Prior – Approval Renewal Limits
Same as above

Rationale

Summary
Galafold 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. The safety and effectiveness of Galafold in pediatric patients have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate and cost-effective use of Galafold while maintaining optimal therapeutic outcomes.

References

Policy History

<table>
<thead>
<tr>
<th>Date</th>
<th>Action</th>
</tr>
</thead>
<tbody>
<tr>
<td>August 2018</td>
<td>Addition to PA</td>
</tr>
<tr>
<td>October 2018</td>
<td>Changed quantity limits to match available blister packs</td>
</tr>
<tr>
<td>November 2018</td>
<td>Annual review</td>
</tr>
<tr>
<td>December 2019</td>
<td>Annual review</td>
</tr>
</tbody>
</table>

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.