Executive Summary

Purpose: Ensure appropriate utilization and control of agents for Fabry Disease

Why Issue Selected: Fabry disease is a rare, progressive genetic disorder characterized by a defective gene, galactosidase alpha gene (GLA), that causes a deficiency of the enzyme, alpha-galactosidase A (alpha-Gal A). This enzyme is responsible for breaking down specific lipids in lysosomes, including globotriaosylceramide (GL-3). The accumulation of GL-3 in blood vessels, kidneys, heart, nerves and other organs leads to cell damage with consequences from mild-to-severe symptoms including kidney failure, myocardial infarctions, and strokes that can be fatal. Galafold® is an alpha-galactosidase 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. Galafold binds to and stabilizes specific mutant forms of alpha-Gal A, thereby facilitating proper trafficking of the enzyme to lysosomes and increasing enzyme activity. Fabry disease affects approximately 3,000 people in the United States and has only one other current treatment option, Fabrazyme®. Galafold is unlike Fabrazyme, an enzyme replacement therapy, in that it increases the activity of the deficient enzyme rather than replacing it and it’s an oral option. Due to the highly specific patient population that would benefit from treatment and high cost, MO HealthNet recommends adding a clinical edit to ensure appropriate patient selection.

Program-Specific Information:

<table>
<thead>
<tr>
<th>Drug</th>
<th>Date Range FFS 1-1-2019 to 12-31-2019</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
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</tr>
<tr>
<td>GALAFOLD 123MG CAP</td>
<td>Claim: 0, Spend: $1,784.45, Cost per unit (MAC): $323,211.00</td>
</tr>
<tr>
<td>FABRAZYME 5 MG VIAL</td>
<td>Claim: 51, Spend: $6,314.62, Cost per year (WAC): $371,492.00</td>
</tr>
<tr>
<td>FABRAZYME 35 MG VIAL</td>
<td>Claim: 71, Spend: $901.94, Cost per year (WAC): $371,492.00</td>
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</tbody>
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Type of Criteria: ☐ Increased risk of ADE, ☑ Appropriate Indications, ☐ Preferred Drug List, ☑ Clinical Edit

Data Sources: ☐ Only Administrative Databases, ☑ Databases + Prescriber-Supplied
Setting & Population

- Drug class for review: agents for Fabry Disease
- Age range: All appropriate MO HealthNet participants aged 8 years or older

Approval Criteria

- Documented diagnosis of Fabry disease AND
- For Fabrazyme: participant is aged 8 years or older
- For Galafold:
  - Participant is aged 18 years or older AND
  - Documented genetic testing confirming participant has an amenable GLA variant AND
  - Claim does not exceed 14 capsules for 28 days of therapy

Denial Criteria

- Therapy will be denied if no approval criteria are met

Required Documentation

<table>
<thead>
<tr>
<th>Laboratory Results:</th>
<th>x</th>
<th>Progress Notes:</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>MedWatch Form:</td>
<td></td>
<td>Other:</td>
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</table>

Disposition of Edit

Denial: Exception code “0682” (Clinical Edit)
Rule Type: CE

Default Approval Period

6 months

References