SmartPA Criteria Proposal

Drug/Drug Class: Fabry Disease Clinical Edit
First Implementation Date: May 23, 2019
Revised Date: August 4, 2022
Prepared for: MO HealthNet
Prepared by: MO HealthNet/Conduent
Criteria Status: ☒ Existing Criteria
☐ Revision of Existing Criteria
☐ New Criteria

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. Treatment of Fabry disease primarily focuses upon replacing the missing or deficient enzyme (alpha-Gal A) with enzyme replacement therapy as well as treating the various symptoms and disease complications. Galafold®, an alpha-galactosidase A pharmacological chaperone, was FDA approved in 2018 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 (present in 35% to 50% of patients). 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 is an oral medication.

Due to high cost and specific approved indication, MO HealthNet will impose clinical criteria to ensure appropriate utilization of agents for Fabry Disease.

Program-Specific Information:

<table>
<thead>
<tr>
<th>Drug</th>
<th>Date Range FFS 1-1-2021 to 12-31-2021</th>
<th>Spend</th>
<th>Avg Spend per Claim</th>
</tr>
</thead>
<tbody>
<tr>
<td>FABRAZYME 5 MG VIAL</td>
<td>59</td>
<td>$254,977.98</td>
<td>$4,321.66</td>
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<tr>
<td>FABRAZYME 35 MG VIAL</td>
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<td>$993,603.58</td>
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<td>GALAFOLD 123 MG CAP</td>
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<td>$102,595.85</td>
<td>$25,648.96</td>
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</tbody>
</table>

Type of Criteria: ☒ Appropriate Indications
☐ Increased risk of ADE
☐ Preferred Drug List
☐ Clinical Edit

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Setting & Population

- Drug class for review: Agents for Fabry Disease
- Age range: All appropriate MO HealthNet participants aged 2 years or older

Approval Criteria

- Documented diagnosis of Fabry disease AND
- For Fabrazyme: participant is aged 2 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 all approval criteria are not met

Required Documentation

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