Executive Summary

Purpose: The MO HealthNet Pharmacy Program will implement a state-specific preferred drug list.

Why Issue Selected: Familial Hypercholesterolemia (FH) is a genetic disorder characterized by high cholesterol levels, specifically high levels of low-density lipoprotein-cholesterol (LDL-C) in the blood. Patients who have one abnormal copy of the low-density lipoprotein receptor (LDLR) gene have the heterozygous form while those patients who have two abnormal copies of the LDLR gene have the homozygous form. Heterozygous FH is a common genetic disorder occurring in 1:500 people while Homozygous FH (HoFH) is much rarer, occurring in 1 in a million births. Patients with HoFH have severely elevated levels of LDL-C. Physical findings of HoFH may include premature coronary artery disease (CAD) and tendon and skin xanthomas. Treatment involves early and aggressive lipid-lowering therapies and lipoprotein apheresis. Patients with HoFH are typically less responsive to standard lipid-lowering therapies including high-intensity statins and proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors. Some patients with HoFH are non-responders to standard therapy.

Evkeeza® is an angiopoietin-like 3 (ANGPTL3) inhibitor indicated as adjunct to other LDL-C lowering therapies for the treatment of adult and pediatric patients aged 5 years and older with HoFH. Juxtapid® is a branded drug product indicated as an adjunct to lipid-lowering medications, treatments, and diet to reduce LDL-C, apolipoprotein B, total cholesterol (TC) and non-high density lipoprotein-cholesterol (non HDL-C) in patients with HoFH.

Total program savings for the PDL classes will be regularly reviewed.
Setting & Population

- Drug class for review: Homozygous Familial Hypercholesterolemia (HoFH) Agents
- Age range: All appropriate MO HealthNet participants

Approval Criteria

- Documented diagnosis of homozygous familial hypercholesterolemia confirmed by:
  - Genetic testing OR
  - American Heart Association clinical criteria AND
- Documented compliance to the following therapies:
  - PCSK9 inhibitor therapy (defined as 90/120 days) AND
  - High intensity statin therapy (defined as 90/120 day) OR
  - Documented ADE/ADR to high intensity statin therapy AND
- Documentation of LDL-C lab result not meeting goal while on therapy with high-intensity statin and PCSK9 inhibitor
- For Evkeeza: Participant aged 5 years or older
- For Juxtapid: Participant aged 18 years or older

Denial Criteria

- Participant is currently pregnant
- For Juxtapid:
  - Documented diagnosis of moderate or severe hepatic impairment
  - Dose on claim exceeds 60 mg per day
- Therapy will be denied if all approval criteria are not met

Required Documentation

<table>
<thead>
<tr>
<th>Laboratory Results:</th>
<th>X</th>
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<tbody>
<tr>
<td>MedWatch Form:</td>
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<td>Other:</td>
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Disposition of Edit

Denial: Exception Code “0160” (Preferred Drug List)
Rule Type: PDL

Default Approval Period

1 year

References

• Facts and Comparisons eAnswers (online); 2021 Clinical Drug Information, LLC.