**Pharmacy Medical Necessity Guidelines: Kuvan® (sapropterin)**

*Effective: June 1, 2017*

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<th>Prior Authorization Required</th>
<th>✓ Type of Review – Care Management</th>
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<td>Pharmacy (RX) or Medical (MED) Benefit</td>
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This Pharmacy Medical Necessity Guideline applies to the following:

**Tufts Health Plan Commercial Plans**
- ✓ Tufts Health Plan Commercial Plans – large group plans
- ✓ Tufts Health Plan Commercial Plans – small group and individual plans

**Tufts Health Public Plans**
- ✓ Tufts Health Direct – Health Connector
- ✓ Tufts Health Together – A MassHealth Plan
- ✓ Tufts Health RITogether – A Rite Care + Rhode Health Partners Plan

**Tufts Health Freedom Plan products**
- ✓ Tufts Health Freedom Plan - large group plans
- ✓ Tufts Health Freedom Plan - small group plans

**Fax Numbers:**
- RXUM: 617.673.0988

**Note:** For Tufts Health Plan Medicare Preferred Members, please refer to the Tufts Health Plan Medicare Preferred Prior Authorization Criteria. Background, applicable product and disclaimer information can be found on the last page.

**OVERVIEW**

Phenylketonuria (PKU) is a genetic disorder in which the enzyme phenylalanine hydroxylase (PAH), which helps our bodies break down phenylalanine (Phe), an amino acid found in foods, does not function properly. The result is high levels of Phe in the blood. High levels of Phe are toxic to the brain and can lead to mental retardation, behavioral abnormalities, seizures, an inability to focus and organize information, and other neurologic complications. PKU disease occurs in one out of every 12,000 to 15,000 live births in the United States.

Kuvan (sapropterin) is a synthetic preparation of the dihydrochloride salt of naturally occurring tetrahydrobiopterin (BH4), the cofactor for the enzyme PAH. PAH hydroxylates Phe through an oxidative reaction to form tyrosine. In patients with PKU, PAH activity is absent or deficient. Treatment with BH4 can activate residual PAH enzyme, improve the normal oxidative metabolism of Phe, and decrease Phe levels in some patients.

**FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS**

Kuvan (sapropterin) is indicated to reduce blood Phe levels in patients with hyperphenylalaninemia due to BH4 responsive PKU and is to be used in conjunction with a Phe-restricted diet.

**COVERAGE GUIDELINES**

**Initial Therapy**

The plan may authorize coverage of Kuvan (sapropterin) for an initial 8 weeks of therapy for Members when the following criteria are met:

1. The prescribing physician is a metabolic disease specialist  
   AND
2. Tetrahydrobiopterin (BH4) deficiency has been ruled out  
   AND
3. The Member has a baseline phenylalanine level ≥ 600 μmol/L  
   Note: to convert phenylalanine level to mg/dl, divide micromol/L by 60  
   AND
4. Documentation that the Member is following a phenylalanine-restricted diet

**Continuing Therapy**

The plan may authorize coverage of Kuvan (sapropterin) for continuing therapy for Members when the following criteria are met:

1. The prescribing physician is a metabolic disease specialist  
   AND
2. The Member has demonstrated at least a 30% reduction in phenylalanine levels compared to baseline
3. Documentation that the Member is following a phenylalanine-restricted diet

LIMITATIONS

1. Initial authorization of coverage for Kuvan (sapropterin) will be limited to 8 weeks.
2. The plan will not cover Kuvan (sapropterin) unless used in conjunction with a phenylalanine-restricted diet.

CODES

None

REFERENCES


APPROVAL HISTORY

May 13, 2008: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

- May 12, 2009: No changes.
- January 1, 2010: Removal of Tufts Medicare Preferred language (separate criteria have been created specifically for Tufts Medicare Preferred)
- May 11, 2010: Addition of note to convert phenylalanine level to mg/dl, divide micromol/L by 60
- May 10, 2011: No changes.
- March 13, 2012: No changes.
- March 12, 2013: No changes.
- February 11, 2014: No changes.
- February 10, 2015: No changes.
- January 1, 2016: Administrative change to rebranded template.
- February 9, 2016: Added the existing limitation of approval requiring documentation of a phenylalanine-restricted diet into the criteria for initial and continuing therapy. Added the requirement of the prescribing physician must be a metabolic specialist to the criteria for continuing therapy.
- May 10, 2016: No changes. Effective 10/1/16, Medical Necessity Guideline applies to Tufts Health Together.
- February 14, 2017: No changes.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. They are used in conjunction with a Member's benefit document and in coordination with the Member's physician(s). The plan makes coverage decisions on a case-by-case basis considering the individual Member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

This Pharmacy Medical Necessity Guideline does not apply to Uniformed Services Family Health Plan Members or to certain delegated service arrangements. Unless otherwise noted in the Member's benefit document or applicable Pharmacy Medical Necessity Guideline, Pharmacy Medical Necessity
Guidelines do not apply to CareLink℠ Members. For self-insured plans, drug coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a coverage guideline and a self-insured Member’s benefit document, the provisions of the benefit document will govern. Applicable state or federal mandates will take precedence.

For Tufts Health Plan Medicare Preferred, please refer to Tufts Health Plan Medicare Preferred Prior Authorization Criteria.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to Member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.