Pharmacy Medical Necessity Guidelines: Sapropterin

Effective: January 18, 2021

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RXUM

These pharmacy medical necessity guidelines apply to the following:

Commercial Products

☒ Tufts Health Plan Commercial products – large group plans
☒ Tufts Health Plan Commercial products – small group and individual plans
☒ Tufts Health Freedom Plan products – large group plans
☒ Tufts Health Freedom Plan products – small group plans
- CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Tufts Health Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product)
☒ Tufts Health Together – MassHealth MCO Plan and Accountable Care Partnership Plans
☒ Tufts Health RITogether – A Rhode Island Medicaid Plan

Fax Numbers:

RXUM: 617.673.0988

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

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

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

Initial Therapy

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

Reauthorization Criteria

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

AND

3. Documentation that the Member is following a phenylalanine-restricted diet

LIMITATIONS

- Initial coverage of sapropterin will be authorized for 8 weeks.
- Members new the plan stable on sapropterin should be reviewed against Reauthorization Criteria.
- The plan will not cover sapropterin unless used in conjunction with a phenylalanine-restricted diet.
- Sapropterin will not be authorized in combination with Palynziq (pegvalise-pqpz).
- The plan does not cover brand Kuvan. Refer to the Pharmacy Medical Necessity Guidelines for non-covered medications.

CODES

None

REFERENCES

1. Kuvan (sapropterin) [prescribing information]. Novato, CA: BioMarin Pharmaceutical, Inc. 2019 February.

2. Blau N, Erlandsen H et al. The metabolic and molecular bases of tetrahydrobiopterin-responsive phenylalanine hydroxylase deficiency. *Molecular Genetics and Metabolism*. 2004;82:101-11.
3. Doggrell SA. Is sapropterin treatment suitable for all subjects with phenylketonuria? *Expert Opinion Pharmacotherapy*. 2008;9(1):145-7.
4. Giovannini M, Verduci E, Salvatici E et al. Phenylketonuria: dietary and therapeutic challenges. *J Inherit Metab Dis*. 2007;30:145-52.
5. Palynziq (pegvaliase-pqpz) [prescribing information]. Novato, CA: BioMarin Pharmaceutical Inc.; 2018 May.

APPROVAL HISTORY

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

Subsequent endorsement date(s) and changes made:
1. May 12, 2009: No changes
2. January 1, 2010: Removal of Tufts Medicare Preferred language (separate criteria have been created specifically for Tufts Medicare Preferred)
3. May 11, 2010: Addition of note to convert phenylalanine level to mg/dl, divide micromol/L by 60
4. May 10, 2011: No changes
5. March 13, 2012: No changes
6. March 12, 2013: No changes
7. February 11, 2014: No changes
8. February 10, 2015: No changes
9. January 1, 2016: Administrative change to rebranded template.
10. 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.
11. May 10, 2016: No changes. Effective 10/1/16, Medical Necessity Guideline applies to Tufts Health Together.
12. February 14, 2017: No changes
13. April 11, 2017: Administrative update. Effective 6/1/2017, Medical Necessity Guideline applies to Tufts Health RITogether.
14. February 13, 2018: No changes
15. November 13, 2018: Effective January 8, 2019, added the following limitation: Kuvan (sapropterin) will not be authorized in combination with Palynziq (pegvalise-pgnz).
16. September 10, 2019: No changes
17. June 9, 2020: Administrative update to add the Limitation "Members new the plan stable on Kuvan (sapropterin) should be reviewed against Reauthorization Criteria."
18. January 12, 2021: Administrative updates. Due to the generic availability of sapropterin, updated the title of the Medical Necessity Guideline from "Kuvan (sapropterin)" to "Sapropterin" and added the following Limitation to be line with current coverage of multi-source brand Kuvan "The plan does not cover brand Kuvan. Refer to the Pharmacy Medical Necessity Guidelines for non-covered medications."

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. 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.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

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.