Pharmacy Medical Necessity Guidelines: Sapropterin

Effective: December 9, 2025

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: ☒ Tufts Health RITogether – A Rhode Island Medicaid Plan		**Fax Numbers:** RXUM: 617-673-0939	

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

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

Sapropterin is a phenylalanine hydroxylase activator indicated to reduce blood phenylalanine (Phe) levels in adult and pediatric patients one month of age and older with hyperphenylalaninemia due to tetrahydrobiopterin-responsive phenylketonuria. Sapropterin is to be used in conjunction with a Phe-restricted diet.

COVERAGE GUIDELINES

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

Initial Authorization Criteria

1. Prescribed by or in consultation with a metabolic disease specialist

AND

2. Tetrahydrobiopterin (BH4) deficiency has been ruled out

AND

3. Documentation of baseline blood phenylalanine concentration greater than 600 micromol/L

AND

4. Documentation sapropterin will be used in conjunction with a phenylalanine-restricted diet

AND

5. Documentation sapropterin will not be used in combination with Palynziq

Reauthorization Criteria

1. Prescribed by or in consultation with a metabolic disease specialist

AND

2. The Member has demonstrated at least a 30% reduction in phenylalanine levels compared to baseline

AND

3. Documentation sapropterin will be used in conjunction with a phenylalanine-restricted diet

AND

4. Documentation sapropterin will not be used in combination with Palynziq

LIMITATIONS

- Initial coverage of sapropterin will be authorized for 8 weeks. Reauthorization of a sapropterin will be provided for 12-month intervals.
- Members new the plan stable on sapropterin should be reviewed against Reauthorization Criteria.
- 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. August 2024.
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

September 13, 2022: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:

- December 12, 2023: No changes
- November 12, 2024: No changes (eff 11/12/24)
- December 9, 2025: 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. 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.