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**Title:** Sapropterin dihydrochloride (Kuvan®)

**Policy #:** Rx.01.234

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***Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.***

***This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.***

***Members are advised to use participating pharmacies in order to receive the highest level of benefits.***

**Intent:**

The intent of this policy is to communicate the medical necessity criteria for **Kuvan® (sapropterin dihydrochloride)** as provided under the member's prescription drug benefit.

**Description:**

Phenylketonuria (PKU) is an inherited disorder that causes elevated blood levels of phenylalanine. PKU is caused by mutations of the PAH gene, which encodes for the enzyme phenylalanine hydroxylase. Phenylalanine hydroxylase converts phenylalanine to tyrosine. Patients with PKU cannot appropriately process phenylalanine from the diet, thus building up to potentially toxic levels in the blood. Excessive amounts of phenylalanine can cause brain and nerve damage if left untreated.

Kuvan® (sapropterin dihydrochloride) 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-(BH4-) responsive Phenylketonuria when used in combination with a low phenylalanine diet.

**Policy:**

**INITIAL CRITERIA:** Sapropterin dihydrochloride (Kuvan®) is approved when ALL the following are met:

1. Diagnosis of phenylketonuria (PKU); and
2. Used in conjunction with phenylalanine (Phe)-restricted diet; and
3. For Brand Kuvan® only: inadequate response or inability to tolerate generic sapropterin dihydrochloride

Initial authorization duration: 2 years

**REAUTHORIZATION CRITERIA:** Sapropterin dihydrochloride (Kuvan®) is re-approved when ALL the following are met:

1. Member has had a positive clinical response to therapy (i.e. there is a reduction of phenylalanine (Phe) blood levels from baseline); and
2. Used in conjunction with phenylalanine (Phe)-restricted diet

Reauthorization duration: 2 years

**Black Box Warning as shown in the drug Prescribing Information:**

N/A

**Guidelines:**

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

## BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

### References:

Phenylketonuria. US National Library of Medicine website. Updated April 7, 2021. Accessed September 29, 2021. [Medlineplus.gov/genetics/condition/phenylketonuria/#causes](https://medlineplus.gov/genetics/condition/phenylketonuria/#causes)

Kuvan® (sapropterin dihydrochloride) [package insert]. Novato, CA: BioMartin Pharmaceutical Inc.; March 2020. Available from: [https://www.kuvan.com/hcp/wp-content/file/KUVAN\\_Prescribing\\_Information1.pdf](https://www.kuvan.com/hcp/wp-content/file/KUVAN_Prescribing_Information1.pdf). Accessed September 29, 2021.

### Applicable Drugs:

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
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Kuvan	Sapropterin dihydrochloride
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### Cross References:

Rx.01.33 Off Label Use

Rx.01.221 Drugs Exceeding Claim Dollar Limit Threshold

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<b>Policy Version Number:</b>	2.00
<b>P&amp;T Approval Date:</b>	September 23, 2021
<b>Policy Effective Date:</b>	January 01, 2022
<b>Next Required Review Date:</b>	September 23, 2022

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The Policy Bulletins on this web site were developed to assist the Company in administering the provisions of the respective benefit programs, and do not constitute a contract. If you have coverage through the Company, please refer to your specific benefit program for the terms, conditions, limitations and exclusions of your coverage. Company does not provide health care services, medical advice or treatment, or guarantee the outcome or results of any medical services/treatments. The facility and professional providers are responsible for providing medical advice and treatment. Facility and professional providers are independent contractors and are not employees or agents of the Company. If you have a specific medical condition, please consult with your doctor. The Company reserves the right at any time to change or update its Policy Bulletins.

