PHARMACY POLICY STATEMENT
Arkansas PASSE

DRUG NAME	Kuvan (sapropterin)
BILLING CODE	Must use valid NDC
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
STATUS	Prior Authorization Required

Kuvan, a synthetic form of the cofactor tetrahydrobiopterin, is a phenylalanine hydroxylase (PAH) activator approved by the FDA in 2007 for the treatment of tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). It is indicated for pediatric and adult patients, and it is supplied as tablets and powder for oral solution. Patients must also maintain a phenylalanine (Phe) restricted diet as part of treatment. PKU results from a deficiency of PAH, leading to increased concentrations of Phe. If untreated, this excess accumulation causes neuropsychiatric and neurocognitive symptoms.

Kuvan (sapropterin) will be considered for coverage when the following criteria are met:

Phenylketonuria (PKU)

For **initial** authorization:
1. Member is at least 1 month of age; AND
2. Member has a diagnosis of phenylketonuria; AND
3. Kuvan will be used in conjunction with a compliant Phe-restricted diet; AND
4. Kuvan will not be prescribed in combination with Palynziq.
5. **Dosage allowed/Quantity limit:** Up to 20 mg/kg once daily. Discontinue after 1 month at this dose if Phe has not decreased.

If all the above requirements are met, the medication will be approved for 2 months.

For **reauthorization**:
1. Chart notes must show at least a 30% reduction of Phe, and/or
2. Neuropsychiatric symptoms have improved, and/or
3. Member has shown an increase in Phe tolerance.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Kuvan (sapropterin) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/05/2021	New policy for Kuvan created.
12/21/2021	Removed prescriber specialty requirement and Phe level documentation.

References:

1. Kuvan [prescribing information]. Novato, CA: Biomarin Phermaceutical Inc.; February 2021.
2. Vockley J, Andersson HC, Antshel KM, et al. Phenylalanine hydroxylase deficiency: diagnosis and management guideline [published correction appears in Genet Med. 2014 Apr;16(4):356]. *Genet Med*. 2014;16(2):188-200. doi:10.1038/gim.2013.157
3. van Spronsen FJ, van Wegberg AM, Ahring K, et al. Key European guidelines for the diagnosis and management of patients with phenylketonuria. *Lancet Diabetes Endocrinol*. 2017;5(9):743-756. doi:10.1016/S2213-8587(16)30320-5
4. Camp KM, Parisi MA, Acosta PB, et al. Phenylketonuria Scientific Review Conference: state of the science and future research needs. *Mol Genet Metab*. 2014;112(2):87-122. doi:10.1016/j.ymgme.2014.02.013
5. Somaraju UR, Merrin M. Sapropterin dihydrochloride for phenylketonuria. *Cochrane Database Syst Rev*. 2015;2015(3):CD008005. Published 2015 Mar 27. doi:10.1002/14651858.CD008005.pub4

Effective date: 01/01/2022
Revised date: 12/21/2021