HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	Kuvan (sapropterin)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Kuvan, a synthetic form of the cofactor tetrahydrobiopterin (BH4), is a phenylalanine hydroxylase (PAH) activator approved by the FDA in 2007 indicated to reduce blood phenylalanine (Phe) levels in adult and pediatric patients one month of age and older with hyperphenylalaninemia (HPA) due to BH4-responsive Phenylketonuria (PKU). Patients must also maintain a Phe-restricted diet as part of treatment. Kuvan is supplied as tablets and powder for oral solution.
PKU results from a deficiency of phenylalanine hydroxylase (PAH) enzyme, leading to increased concentrations of Phe. If untreated, this excess accumulation causes neuropsychiatric and neurocognitive symptoms. Standard of care for PKU is a Phe-restricted diet.

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. Medication must be prescribed by or in consultation with a specialist experienced in metabolic or genetic diseases; AND
3. Member has a diagnosis of phenylketonuria; AND
4. Member has documentation of current blood Phe level sustained above 360 µmol/L despite dietary management; AND
5. Kuvan will be used in conjunction with a compliant Phe-restricted diet; AND
6. Kuvan will not be prescribed in combination with Palynziq.
7. **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 document at least one of the following compared to baseline:
   a) At least 30% reduction of Phe
   b) Phe decrease to between 120 and 360 µmol/L
   c) Improved neuropsychiatric symptoms
   d) Increase in Phe dietary tolerance

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

HAP 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.
10/31/2022	Annual review; no updates.
06/12/2024	Added reference. Added “despite dietary management” with Phe >360. Added Phe 120-360 as an option to qualify for continuation.

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
6. Adams AD, Fiesco-Roa MÓ, Wong L, et al. Phenylalanine hydroxylase deficiency treatment and management: A systematic evidence review of the American College of Medical Genetics and Genomics (ACMG). *Genet Med*. 2023;25(9):100358. doi:10.1016/j.gim.2022.12.005

Effective date: 01/01/2025
Revised date: 06/12/2024