

## SPECIALTY GUIDELINE MANAGEMENT

### KUVAN (sapropterin dihydrochloride)

#### POLICY

##### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

###### A. FDA-Approved Indication

Kuvan is indicated to reduce blood phenylalanine (Phe) levels in patients with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). Kuvan is to be used in conjunction with a Phe-restricted diet.

###### B. Compendial Uses

1. Autosomal dominant guanine triphosphate cyclohydrolase deficiency (Segawa disease)
2. Autosomal recessive guanine (GTP) cyclohydrolase deficiency
3. 6-pyruvoyl-tetrahydropterin synthase (6-PTS) deficiency
4. Sepiapterin reductase deficiency
5. Dihydropteridine reductase (DHPR) deficiency
6. Pterin-4a-carbinolamine dehydratase deficiency (also called primapterinuria)

All other indications are considered experimental/investigational and are not a covered benefit.

##### II. CRITERIA FOR INITIAL APPROVAL

###### A. **Phenylketonuria (PKU)**

1. Authorization of 2 months may be granted for members requesting a therapeutic trial with Kuvan when the pretreatment, including before dietary management, phenylalanine level was greater than 6 mg/dL (360 micromol/L).
2. Authorization of indefinite approval may be granted following a therapeutic trial with Kuvan when the member's therapeutic trial meets either of the following:
  - a. Member experienced a reduction in blood Phe level of at least 30% during the therapeutic trial with Kuvan.
  - b. Member has demonstrated an improvement in neuropsychiatric symptoms during the therapeutic trial with Kuvan.

###### B. **Biopterin Metabolic Defects**

Authorizations of indefinite approval may be granted for members who have any of the following biopterin metabolic defects:

1. Autosomal dominant guanine triphosphate cyclohydrolase deficiency (Segawa disease)
2. Autosomal recessive guanine (GTP) cyclohydrolase deficiency
3. 6-pyruvoyl-tetrahydropterin synthase (6-PTS) deficiency
4. Sepiapterin reductase deficiency
5. Dihydropteridine reductase (DHPR) deficiency
6. Pterin-4a-carbinolamine dehydratase deficiency (also called primapterinuria)

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### **III. CONTINUATION OF THERAPY**

All members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria.

### **IV. REFERENCES**

1. Kuvan [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; August 2016.
2. Vockley J, Andersson HC, Antshel KN, et al. Phenylalanine hydroxylase deficiency: diagnosis and management guideline. *Genet Med*. 2014;16(2):188-200.
3. Singh RH, Rohr F, Frazier D, et al. Recommendations for the nutrition management of phenylalanine hydroxylase deficiency. *Genet Med*. 2014;16(2):121-131.