Reference number(s)
2012-A

SPECIALTY GUIDELINE MANAGEMENT

KUVAN (sapropterin dihydrochloride)
Javygtor (sapropterin dihydrochloride)
sapropterin dihydrochloride (generic)

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/Javygtor/sapropterin is indicated to reduce blood phenylalanine (Phe) levels in adult and pediatric patients one month of age and older with hyperphenylalaninemia (HPA) due to tetrahydrobiopterin- (BH4-) responsive phenylketonuria (PKU). Kuvan/Javygtor/saproprterin is to be used in conjunction with a Phe-restricted diet.

B. Compendial Uses
1. Autosomal dominant guanine triphosphate (GTP) cyclohydrolase deficiency (Segawa disease)
2. Autosomal recessive guanine triphosphate (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 not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: enzyme assay, genetic testing, or phenylalanine level results supporting diagnosis.

III. CRITERIA FOR INITIAL APPROVAL

A. Phenylketonuria (PKU)
Authorization of 60 days may be granted for members with a diagnosis of phenylketonuria who have a baseline phenylalanine level greater than or equal to 360 micromol/L (6 mg/dL) with dietary interventions alone.

Note: If Kuvan is initiated in a member currently receiving Palynziq for phenylketonuria (PKU), then Palynziq will be discontinued after an appropriate period of overlap.

B. Biopterin Metabolic Defects
Authorization of 6 months may be granted for members who have any of the following biopterin metabolic defects:

1. Autosomal dominant guanine triphosphate (GTP) cyclohydrolase deficiency (Segawa disease)
2. Autosomal recessive guanine triphosphate (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)

IV. CONTINUATION OF THERAPY

A. Phenylketonuria (PKU)

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for phenylketonuria (PKU) who meet any of the following criteria:

1. Achieve or maintain a 30% decrease in phenylalanine levels from baseline; or
2. Phenylalanine levels are in an acceptable range (less than 360 micromol/L or 6 mg/dL); or
3. Demonstrate an improvement in neuropsychiatric symptoms.

Note: Kuvan should not be used concomitantly with Palynziq for phenylketonuria (PKU).

B. Biopterin Metabolic Defects

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for any biopterin metabolic defect listed in Section III who are experiencing benefit from therapy as evidenced by disease stability or disease improvement.

V. REFERENCES

1. Kuvan [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; February 2021.
2. Vockley J, Andersson HC, Antshel KM, 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.
4. Sapropterin dihydrochloride [package insert]. Chestnut Ridge, NY: Par Pharmaceutical; April 2020.
5. Javygtor [package insert]. Princeton, NJ: Dr. Reddy's Laboratories, Inc.; May 2022.