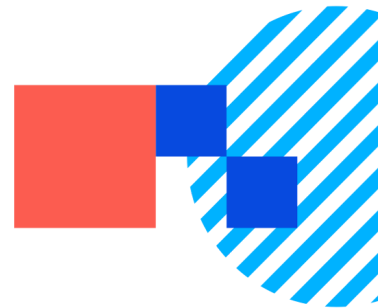




Phenylketonuria Prior Authorization

Drug(s) Applied:	sapropterin dihydrochloride
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Criteria:

Drug(s) Applied will be approved when the requested medication is being used for an FDA approved indication and all of the following criteria are met:

I. Initial Therapy Criteria

A. Phenylketonuria (PKU) as indicated by chart notes within past 180 days

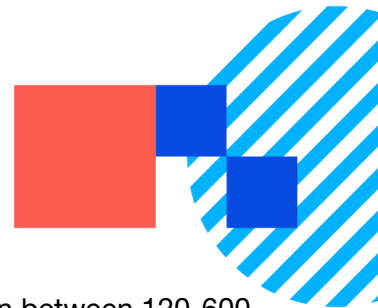
1. Phenylalanine (Phe) levels cannot be maintained within the recommended maintenance range with dietary intervention (Phe-restriction) despite strict compliance **and**
2. A Phe-restricted diet will continue while being treated with the requested agent **and**
3. ONE of the following:
 - a) Patient is less than 12 years of age AND has a pre-treatment blood Phe level greater than or equal to 360 micromol/L (6 mg/dL) **or**
 - b) Patient is 12 years of age or over AND has a pre-treatment blood Phe level greater than 600 micromol/L (10 mg/dL) **or**
 - c) Patient is planning on becoming pregnant or is currently pregnant AND has a pre-treatment Phe level greater than 360 micromol/L (6 mg/dL) **and**
4. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., metabolic disorders) **and**
5. Chart notes and/or prescriber do not show documentation that patient will be using the requested agent in combination with Palynziq (pegvaliase-pqpz) or Sepience (sepiapterin) **and**
6. Initial dose does not exceed 20 mg/kg/dose once daily

Approval Duration: For sapropterin with an initial dose of less than 20 mg/kg/day, 2 months. For sapropterin with an initial dose of 20 mg/kg/day, 1 month.

II. Continued Therapy Criteria

A. Phenylketonuria (PKU) as indicated by chart notes within past 12 months

1. Patient meets the initial therapy criteria above **and**
2. Patient's on-treatment blood Phe levels fall within acceptable ranges:
 - (1) Less than 12 years of age, then between 120-360 micromol/L (2-6 mg/dL) **or**



- (2) Greater than or equal to 12 years of age, then between 120-600 micromol/L (2-10 mg/dL) **or**
- (3) If currently pregnant or planning on becoming pregnant, then between 120-360 micromol/L (2-6 mg/dL) **or**
- 3. Patient has had at least a 30% decrease in blood Phe level from pre-treatment Phe level **and**
- 4. Patient is currently on a Phe-restricted diet and will continue while being treated with the requested agent **and**
- 5. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., metabolic disorders)

Approval Duration: 12 months

References:

1. Kuvan (sapropterin) prescribing information. BioMarin Pharmaceuticals, Inc. February 2021.
2. ACOG. Management of Women With Phenylalanine Hydroxylase Deficiency (Phenylketonuria). June 2015.
<https://www.acog.org/clinical/clinical-guidance/committee-opinion/articles/2020/04/management-of-women-with-phenylalanine-hydroxylase-deficiency-phenylketonuria>
3. Smith W. E. et al. Phenylalanine hydroxylase deficiency diagnosis and management: A 2023 evidence-based clinical guideline of the American College of Medical Genetics and Genomics (ACMG). January 2025.
<https://www.sciencedirect.com/science/article/pii/S1098360024002235#bib55>
4. Van Wegberg, A.M.J. et al. European guidelines on diagnosis and treatment of phenylketonuria: First revision. Molecular Genetics and Metabolism. June 2025.

Policy Owned by: Curative PBM team