curative

Phenylketonuria Prior Authorization Drug List A

Drug(s) Applied:	**sapropterin dihydrochloride, Javygtor** (sapropterin), **Zelvysia** (sapropterin), **Palynziq** (pegvaliase-pqpz)

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. If for Javygtor, Zelvysia, or Palynziq, must have documented failure of sapropterin dihydrochloride **and**
  2. Phenylalanine (Phe) levels cannot be maintained within the recommended maintenance range with dietary intervention (Phe-restriction) despite strict compliance **and**
  3. A Phe-restricted diet will continue while being treated with the requested agent **and**
  4. If for sapropterin, 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**
  5. If for Palynziq, patient has a pre-treatment blood Phe level greater than or equal to 600 micromol/L (10 mg/dL) **and**
  6. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., metabolic disorders) **and**
  7. 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. For Palynziq, 9 months.

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. If for sapropterin, patient's on-treatment blood Phe levels fall within acceptable ranges confirmed by at least ONE of the following:
    (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**
    (4) Patient has had at least a 30% decrease in blood Phe level from pre-treatment Phe level **and**
3. If for Palynziq, then patient meets the following:
    a) ONE of the following:
        (1) Patient's blood Phe level is less than or equal to 600 micromol/L (10 mg/dL) **or**
        (2) Patient has had at least a 20% decrease in blood Phe level from pre-treatment Phe level **and**
    b) Chart notes and/or prescriber do not show documentation that patient has received 16 weeks of therapy at the maximum recommended dose in approved labeling (60 mg once daily) **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