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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 or Zelvysia, must have documented failure of sapropterin dihydrochloride **and**
  2. Phenylalanine levels cannot be maintained within the recommended maintenance range with dietary intervention (phenylalanine-restriction) despite strict compliance AND a Phe-restricted diet will continue while being treated with the requested agent **and**
  3. If for sapropterin, ONE of the following:
     a) Patient is less than 12 years of age AND has a baseline (prior to therapy for the requested indication) 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 baseline (prior to therapy for the requested indication) 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 baseline (prior to therapy for the requested indication) Phe level greater than 360 micromol/L (6 mg/dL) **and**
  4. If for Palynziq, patient has a baseline (prior to therapy for the requested indication) blood Phe level greater than or equal to 600 micromol/L (10 mg/dL) **and**
  5. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., metabolic disorders) **and**
  6. Chart notes and/or prescriber do not show documentation that patient will be using the requested agent in combination with folate synthesis inhibitors (e.g., methotrexate, valproic acid, phenobarbital, or trimethoprim), or drugs affecting nitric oxide-mediated vasorelaxation (e.g., PDE-5 inhibitors) **and**
  7. Initial dose does not exceed 20 mg/kg/dose once daily

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Approval Duration: For sapropterin with an initial dose of 10 mg/kg/day up to 20mg/kg/day, 2 months. For sapropterin with an initial dose of 20mg/kg/day or more, 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, BOTH of the following:
   a) Patient's blood Phe levels continue to remain above acceptable range:
      (1) Less than 12 years of age, then 120-360 micromol/L (2-6 mg/dL) **or**
      (2) Greater than or equal to 12 years of age, then 120-600 micromol/L (2-10 mg/dL)] **or**
      (3) If currently pregnant or planning on becoming pregnant, then 120-360 micromol/L (2-6 mg/dL) **and**
   b) Patient has had at least a 30% decrease in blood Phe level from baseline (prior to therapy for the requested indication) **and**
3. If for Palynziq, BOTH of 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 baseline (prior to therapy for the requested indication) **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 phenylalanine (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

Policy Owned by: Curative PBM team