I. Medication Description

Pegvaliase-pqpz is a PEGylated phenylalanine ammonia lyase (PAL) enzyme that converts phenylalanine to ammonia and trans-cinnamic acid. It substitutes for the deficient phenylalanine hydroxylase (PAH) enzyme activity in patients with phenylketonuria (PKU) and reduces blood phenylalanine concentrations.

Palynziq is associated with a risk of serious hypersensitivity reactions or anaphylaxis that may occur at any time during treatment. The initial dose of Palynziq should be administered under the supervision of a healthcare provider equipped to manage anaphylaxis and the patient should be closely observed for at least 60 minutes following injection. Prior to self-injection, patients should be prescribed auto-injectable epinephrine, instructed on its appropriate use, and instructed to carry it with them at all times during Palynziq treatment. Palynziq is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the Palynziq REMS.

II. Position Statement

Coverage is determined through a prior authorization process with supporting clinical documentation for every request.

III. Policy

Coverage of Palynziq is available when the following criteria have been met:

- Member is at least 18 years of age AND
- Prescribed by a metabolic disease specialist AND
- Member has a diagnosis of phenylketonuria (PKU) AND
- When requesting coverage of a brand medication for which a plan-preferred A/B rated generic is available, coverage will be provided when there is sufficient evidence that the use of the A/B rated generic equivalent has resulted in inadequate results AND
- Member has uncontrolled blood phenylalanine (Phe) concentrations defined as levels above 600 micromol/L (10mg/dL) on existing management including:
  - Restriction of dietary phenylalanine intake AND
  - Coverage will be provided when the member has experienced intolerance or therapeutic failure with the plan-preferred medication (Kuvan) first OR when at least ONE of the following criteria have been met:
• The plan-preferred medications are contraindicated or will likely cause an adverse reaction by or physical or mental harm to the member.
• The plan-preferred medications are expected to be ineffective based on the known clinical history and conditions of the member and the member’s prescription drug regimen.
• The member has tried the plan-preferred medications or another prescription drug in the same pharmacologic class or with the same mechanism of action and such prescription drug was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event.
• The member is stable on the medication selected by their healthcare professional for the medical condition under consideration (where “stable” is defined as receiving the medication for an adequate period of time, have achieved optimal response, and continued favorable outcomes are expected UNLESS the medication was initially selected solely due to the availability of a drug sample or a coupon card and the member does not otherwise meet the definition of “stable”).
• The plan-preferred medication is not in the best interest of the member because it will likely cause a significant barrier to the member’s adherence or to compliance with the member’s plan of care, will likely worsen a comorbid condition of the member, or will likely decrease the member’s ability to achieve or maintain reasonable functional ability in performing daily activities **AND**
  • Member has been prescribed an epinephrine auto-injectable to carry with them at all times and instructed on its appropriate use

IV. **Quantity Limitations**

Coverage is available for a quantity sufficient to allow for FDA-approved dosing.

V. **Coverage Duration**

Coverage is available for 6 months and may be renewed.

VI. **Coverage Renewal Criteria**

Coverage can be renewed based upon the following criteria:
  • Documentation of at least a 20% reduction in blood phenylalanine concentration from pre-treatment baseline **OR**
  • A blood phenylalanine concentration <600micromol/L (<10mg/dL) **AND**
  • Absence of unacceptable toxicity from the drug

VII. **Billing/Coding Information**

• Palynziq is available as 2.5mg/0.5mL, 10mg/0.5mL, and 20mg/mL single-dose prefilled syringes
• J3590: 1 billable unit = 1 syringe
• C9399: 1 billable unit = 1 syringe
• Pertinent indications:
  o Classical phenylketonuria: E70.0
  o Other hyperphenylalaninemas: E70.1

VIII. Summary of Policy Changes

• 8/15/18: new policy
• 5/15/19: updated billing/coding information

IX. References


*These guidelines are not applicable to benefits covered under Medicare Advantage. Medicare Advantage benefit coverage requests are reviewed in accordance with the guidance set forth in Chapter 15 Section 50 of the Centers for Medicare & Medicaid Services Medicare Benefit Policy Manual.

The Plan fully expects that only appropriate and medically necessary services will be rendered. The Plan reserves the right to conduct pre-payment and post-payment reviews to assess the medical appropriateness of the above-referenced therapies.

The preceding policy applies only to members for whom the above named pharmacy benefit medications are included on their covered formulary. Members with closed formulary benefits are subject to trying all appropriate formulary alternatives before a coverage exception for a non-formulary medication will be considered.

The preceding policy is a guideline to allow for coverage of the pertinent medication/product, and is not meant to serve as a clinical practice guideline.