Palynziq

Description

Palynziq (pegvaliase-pqpz)

Background
Palynziq (pegvaliase-pqpz) is a phenylalanine-metabolizing enzyme indicated to reduce blood phenylalanine concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood phenylalanine concentrations greater than 600 micromol/L. Prolonged high blood phenylalanine (Phe) levels are neurotoxic and lead to impairment of intelligence and other brain functions, such as attentiveness. Reduction of blood Phe levels through dietary control is an important determinant of long-term neurologic outcome in phenylketonuria (PKU) patients, and reduction of blood Phe levels in patients with PKU has been shown to decrease the long-term risk of neurologic injury. Palynziq substitutes for the deficient phenylalanine hydroxylase (PAH) enzyme activity in patients with PKU and reduces blood phenylalanine concentrations (1).

Regulatory Status
FDA-approved indication: Palynziq is a phenylalanine-metabolizing enzyme indicated to reduce blood phenylalanine concentrations in adult patients with phenylketonuria who have uncontrolled blood phenylalanine concentrations greater than 600 micromol/L on existing management (1).

Palynziq has a boxed warning that anaphylaxis may occur at any time during Palynziq treatment (1).
Administration of the initiation dose of Palynziq must be under the supervision of a healthcare provider equipped to manage anaphylaxis, and patients must be closely observed for at least 60 minutes following injection. Prior to self-administration, confirm patient competency with self-administration, and patient's and observer's (if applicable) ability to recognize signs and symptoms of anaphylaxis and to administer auto-injectable epinephrine, if needed (1).

The prescriber should prescribe an auto-injectable epinephrine and provide instructions on it appropriate use. The patient should be advised to carry the epinephrine injector at all times while on Palynziq and if used, to seek follow up medical care (1).

Palynziq is available only through a restricted program called the Palynziq REMS Program (1).

The safety and effectiveness of Palynziq in pediatric patients have not been established (1).

Related policies
Kuvan

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Palynziq may be considered medically necessary for patients 18 years and older for phenylketonuria and if the conditions indicated below are met.

Palynziq may be considered investigational in patients less than 18 years of age and for all other indications.

Prior-Approval Requirements

Age
18 years of age and older

Diagnosis

Patient must have the following:

Phenylketonuria (PKU)
AND ALL of the following:
1. Blood phenylalanine concentration > 600 micromol/L after a trial of sapropterin dihydrochloride (Kuvan)
2. Physician agrees to assess patient tolerability, blood phenylalanine concentration, and dietary protein and phenylalanine intake throughout treatment
3. Prescriber and patient must be enrolled with the Palynziq REMS Program
4. Auto-injectable epinephrine has been prescribed and the patient instructed in its use
5. NOT to be used in combination with sapropterin dihydrochloride (Kuvan)

Prior–Approval Renewal Requirements

Age
18 years of age and older

Diagnosis

Patient must have the following:

Phenylketonuria (PKU)
1. NOT to be used in combination with sapropterin dihydrochloride (Kuvan)
2. Auto-injectable epinephrine has been prescribed and the patient instructed in its use

AND ONE of the following:

a. Reduction from baseline phenylalanine concentration of 20% or greater
b. Reduction in blood phenylalanine concentration to less than or equal to 600 micromol/L

Policy Guidelines

Pre–PA Allowance
None

Prior–Approval Limits
Duration  6 months

Prior–Approval *Renewal* Limits

Duration  12 months

**Rationale**

**Summary**
Palynziq (pegvaliase-pqpz) is a phenylalanine-metabolizing enzyme indicated to reduce blood phenylalanine concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood phenylalanine concentrations greater than 600 micromol/L. Palynziq substitutes for the deficient phenylalanine hydroxylase (PAH) enzyme activity in patients with PKU and reduces blood phenylalanine concentrations. The safety and effectiveness of Palynziq in pediatric patients have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Palynziq while maintaining optimal therapeutic outcomes.

**References**

**Policy History**

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**Keywords**
This policy was approved by the FEP® Pharmacy and Medical Policy Committee on December 6, 2019 and is effective on January 1, 2020.