Commercial/Healthcare Exchange PA Criteria

Effective: February 6th, 2019

Prior Authorization: Palynziq (pegvaliase-pqpz)

Products Affected: Palynziq (pegvaliase-pqpz injection for subcutaneous use)

Medication Description: Palynziq is indicated to reduce blood phenylalanine concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood phenylalanine concentrations greater than 600 micromol/L (µmol/L) on existing management. Treatment with Palynziq should be managed by a healthcare provider experienced in the management of PKU. Baseline blood phenylalanine concentrations should be obtained before initiating treatment. Palynziq is titrated up over a period of 9 weeks to the maintenance dose of 20 mg administered subcutaneously (SC) once daily (QD). Therapeutic response may not be achieved until the patient is titrated to an effective maintenance dosage. Palynziq 20 mg SC QD should be maintanined for at least 24 weeks. The dose can be increased to a maximum dose of Palynziq 40 mg SC QD in patients who have been maintained continuously on the 20 mg QD dose for at least 24 weeks and who have not achieved either a 20% reduction in blood phenylalanine concentration from pre-treatment baseline levels or a blood phenylalanine concentration ≤ 600 µmol/L. Palynziq should be discontinued in patients who have not achieved a response after 16 weeks of continuous treatment with the maximum dosage of 40 mg QD. In patients who experience blood phenylalanine concentrations < 30 µmol/L during the titration and maintenance phase, the dosage of Palynziq may be reduced and/or dietary protein and phenylalanine intake may be modified to maintain phenylalanine levels within a clinically acceptable range and above 30 µmol/L. Because of the risk of anaphylaxis Palynziq is available only through a restricted Risk Evaluation and Mitigation Strategy (REMS) program. It was unclear from the Palynziq clinical trials if all patients had tried and were non-responders to Kuvan.

Covered Uses: Reduction of blood phenylalanine concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood phenylalanine concentrations >600 micromol/L on existing management.

Exclusion Criteria: N/A

Required Medical Information:

1. Diagnosis of phenylketonuria (PKU)
2. Previous therapies tried

Age Restrictions: 18 years of age or older

Prescriber Restrictions: The medication is prescribed by or in consultation with a metabolic disease specialist.

Coverage Duration: 12 months

Other Criteria:

Phenylketonuria (PKU) in Adults

Initial

1. The patient has uncontrolled blood phenylalanine concentrations greater than 600 micromol/L on at least one existing treatment modality (e.g., restriction of dietary phenylalanine and protein intake or prior treatment with Kuvan® [sapropterin dihydrochloride tablets and powder for oral solution]); AND
2. Palynziq will not be used in conjunction with Kuvan.

Continuation

A. The patient's blood phenylalanine concentration is less than or equal to 600 micromol/L; OR
B. The patient has achieved a greater than or equal to 20% reduction in blood phenylalanine concentration from pre-treatment baseline (i.e., blood phenylalanine concentration before starting Palynziq therapy).

References:

1. Palynziq™ injection [prescribing information]. Novato, CA: BioMarin Pharmaceuticals; May 2018.
2. Vockley J, Andersson HC, Antshel KM, et al. Phenylalanine hydroxylase deficiency: diagnosis and management guideline. Available at: https://www.acmg.net/docs/Phenylalanine_Hydrosylase_Deficiency_Practice_Guideline_AOP_Jan_2013.pdf. Accessed on May 24, 2018.

Policy Revision history

Rev #	Type of Change	Summary of Change	Sections Affected	Date
1	New Policy	New Policy	All	1/29/19