



Smart

PA

Clinical Edit Criteria Proposal

Drug/Drug Class: Palynziq™ (pegvaliase-pqpz) Clinical Edit

Date: April 18, 2019

Prepared for:

Prepared by: MO HealthNet

New Criteria

Revision of Existing Criteria

Executive Summary

Purpose: Ensure appropriate utilization and control of Palynziq™ (pegvaliase-pqpz).

Why was this Issue Selected:

Palynziq™ is a PEGylated (recombinant) phenylamine ammonia lyase enzyme indicated for the treatment of adult patients with phenylketonuria (PKU) and have uncontrolled blood phenylalanine (Phe) concentrations greater than 600 micromol/L on existing management. Current therapies available for PKU require patients to adhere to a restricted diet, which is not a requirement for patients taking Palynziq™. Palynziq™ converts phenylalanine to ammonia and *trans*-cinnamic acid and works to reduce the blood phenylalanine concentrations by substituting the deficient phenylalanine hydroxylase (PAH) enzyme in patients with PKU. PKU most commonly presents with intellectual disability and can exhibit symptoms of epilepsy, parkinsonism and eye abnormalities to name a few. The diagnosis of PKU is aided in obtaining Phe levels in the blood. This baseline Phe should also be done before initiating treatment with Palynziq™. The recommended dose of Palynziq™ is a taper schedule that involves an induction dose, a titration dose and maintenance dosage. This medication comes with a Black Box Warning for anaphylaxis that can occur at any time during treatment and must be dispensed through the restricted program of Palynziq™ REMS (Risk Evaluation & Mitigation Strategies). Doses should be administered under the supervision of a health care provider and epinephrine should be available during treatment, preferably an auto-injectable form.

| Program-specific information: | Drug | Average cost per year |
|-------------------------------|-------------|-----------------------|
| | • Palynziq™ | \$192,000 WAC |

Setting & Population: Adult patients 18 years of age and older

Type of Criteria: Increased risk of ADE Clinical Edit
 Appropriate Indications

Data Sources: Only administrative databases Databases + Prescriber-supplied

Setting & Population

- Drug for review: Palynziq™ (pegvaliase-pqpz)
- Age range: Adult patients age 18 years and older
- Gender: Male and female

Approval Criteria

- Appropriate diagnosis
 - Phenylketonuria (PKU) with blood PHE concentrations > 600 micromol/L on existing management
- Prior or current restriction of dietary Phe and protein intake
- Documented trial period on Kuvan (sapropterin)
- Adherence to prescribing within the protocol
 - Induction, titration, and maintenance phases

Denial Criteria

- Therapy will be denied if approval criteria is not met

References

1. Prescribing Information, BioMarin Pharmaceutical Inc. Novato, CA 94949 2018
2. Rush, Eric T et al. Phenylketonuria (PKU) May 31, 2018 Medscape