

Palynziq® (pegvaliase-pqpz)



When requesting Palynziq® (pegvaliase-pqpz), the individual requiring treatment must be diagnosed with an FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved Indication

To reduce blood phenylalanine concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood phenylalanine concentrations greater than 600 micromol/L despite treatment.

Coverage Guidelines

The individual must meet **all** of the following criteria for initial approval or reauthorization if the patient has gotten Palynziq (pegvaliase-pqpz) for less than a year:

- Has blood phenylalanine concentration of 600 micromol/L or greater despite existing treatment(s) (e.g., restriction of dietary phenylalanine and protein intake, prior treatment with Kuvan® [sapropterin dihydrochloride]); AND
- Prescribed by or in consultation with a metabolic disease specialist; AND
- Is 18 years of age or older; AND

The individual must meet the following criteria for reauthorization:

- Has achieved at least a 20% reduction in blood phenylalanine concentration from pre-treatment baseline; OR
- Has current blood phenylalanine concentration level less than or equal to 600 micromol/L; AND
- The patient is not receiving concomitant therapy with sapropterin dihydrochloride (Kuvan); AND
- Is 18 year of age or older.

Approval duration: 12 months

Dosing Recommendations

The initial induction dose is 2.5 mg administered subcutaneously once weekly for 4 weeks.

Approve up to a maximum dose of 40mg administered subcutaneously no more frequently than once daily.

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_Hydroxylase_Deficiency_Practice_Guideline_AOP_Jan_2013.pdf. Accessed on June 5, 2020.
3. van Wegberg AMJ, MacDonald A, Ahring A, et al. The complete European guidelines on phenylketonuria: diagnosis and treatment. *Orphanet J Rare Dis*. 2017;12:162.