BlueCross BlueShield Federal Employee Program

PALYNZIQ (pegvaliase-pqpz)

RATIONALE FOR INCLUSION IN PA PROGRAM

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 its 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).

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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

1. Palynziq [package insert]. Novato, CA. BioMarin Pharmaceutical, Inc.; November 2020.