CareSource Rxinnovations

PHARMACY POLICY STATEMENT
Georgia Medicaid

DRUG NAME	Palynziq (pegvaliase-pqpz)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Palynziq, approved by the FDA in 2018, is a phenylalanine (Phe)-metabolizing enzyme indicated to reduce blood Phe concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood Phe concentrations greater than 600 micromol/L on existing management. Palynziq is only available through a REMS program due to a risk of anaphylaxis.
PKU results from a deficiency of phenylalanine hydroxylase (PAH) enzyme, leading to increased concentrations of Phe. If untreated, this excess accumulation causes neuropsychiatric and neurocognitive symptoms. Palynziq is a PEGylated phenylalanine ammonia lyase (PAL) enzyme that converts phenylalanine to ammonia and trans-cinnamic acid. It works as an enzyme substitution therapy as PAL substitutes for the deficient PAH enzyme activity. Standard of care for PKU is a Phe-restricted diet.

Palynziq (pegvaliase-pqpz) will be considered for coverage when the following criteria are met:

Phenylketonuria (PKU)

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with specialist experienced in metabolic or genetic diseases; AND
3. Member has a diagnosis of phenylketonuria; AND
4. Member has uncontrolled blood phenylalanine (Phe) concentrations greater than 600 micromol/L on existing management with Kuvan* (requires prior authorization) in conjunction with following recommended dietary modifications; AND
5. Palynziq will not be prescribed in combination with Kuvan.
6. **Dosage allowed/Quantity limit:** Initial, 2.5 mg subQ once weekly x 4 weeks. Titrate over at least 5 weeks to 20 mg once daily. May increase to 40 mg daily after 24 weeks on 20 mg/day if control not achieved. May increase to 60 mg daily if control not achieved with 40 mg/day after 16 weeks. Discontinue after 16 weeks of 60 mg/day if adequate response not achieved. (Max dose 60 mg/day). QL: 90 syringes per 30 days

**Note*: *A trial of Kuvan is not necessary if there is documentation of 2 null mutations. However, a trial and failure of compliant diet management is still required.*

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must show at least one of the following:
   a) Member has achieved at least a 20% reduction in blood phenylalanine concentration from pretreatment baseline
   b) Member has achieved a blood phenylalanine concentration of 600 micromol/L or less.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Palynziq (pegvaliase-pqpz) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
07/27/2018	New policy for Palynziq created.
04/30/2021	Updated references. Added requirements for dietary management and Kuvan. Removed exclusion criteria that were from clinical trial. Abbreviated dosing information and updated to reflect label change with new max. Amended renewal criteria.
10/31/2022	Transferred to new template. Added QL. Changed initial auth duration from 12 months to 6 months. Split renewal criteria into 2 bullets for readability.
06/14/2024	Updated references.

References:

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3. van Wegberg AMJ, MacDonald A, Ahring K, et al. The complete European guidelines on phenylketonuria: diagnosis and treatment. Orphanet J Rare Dis. 2017;12(1):162. Published 2017 Oct 12. doi:10.1186/s13023-017-0685-2
4. van Spronsen FJ, van Wegberg AM, Ahring K, et al. Key European guidelines for the diagnosis and management of patients with phenylketonuria. Lancet Diabetes Endocrinol. 2017;5(9):743-756. doi:10.1016/S2213-8587(16)30320-5
5. Thomas J, Levy H, Amato S, et al. Pegvaliase for the treatment of phenylketonuria: Results of a long-term phase 3 clinical trial program (PRISM). Mol Genet Metab. 2018;124(1):27-38. doi:10.1016/j.ymgme.2018.03.006
6. Harding CO, Amato RS, Stuy M, et al. Pegvaliase for the treatment of phenylketonuria: A pivotal, double-blind randomized discontinuation Phase 3 clinical trial. Mol Genet Metab. 2018;124(1):20-26. doi:10.1016/j.ymgme.2018.03.003
7. Longo N, Dimmock D, Levy H, et al. Evidence- and consensus-based recommendations for the use of pegvaliase in adults with phenylketonuria. Genet Med. 2019;21(8):1851-1867. doi:10.1038/s41436-018-0403-z
8. Adams AD, Fiesco-Roa MÓ, Wong L, et al. Phenylalanine hydroxylase deficiency treatment and management: A systematic evidence review of the American College of Medical Genetics and Genomics (ACMG). *Genet Med*. 2023;25(9):100358. doi:10.1016/j.gim.2022.12.005

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Revised date: 06/14/2024