CareSource

PHARMACY POLICY STATEMENT **Georgia Medicaid**	
DRUG NAME	Palynziq (pegvaliase-pqpz)
BILLING CODE	Must use valid NDC code
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
COVERAGE REQUIREMENTS	Prior Authorization Required (Non-Preferred Product) Alternative preferred product includes Kuvan QUANTITY LIMIT— up to 60 mg SQ once daily
LIST OF DIAGNOSES CONSIDERED **NOT** MEDICALLY NECESSARY	Click Here

Palynziq (pegvaliase-pqpz) is a **non-preferred** product and will only be considered for coverage under the **pharmacy** benefit when the following criteria are met:

Members must be clinically diagnosed with one of the following disease states and meet their individual criteria as stated.

PHENYLKETONURIA (PKU)

For **initial** authorization:

1. Member is 18 years of age or older; AND
2. Medication must be prescribed by or in consultation with a 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:** Initial, 2.5mg subQ once weekly x 4 weeks. Titrate over at least 5 weeks to 20mg once daily. May increase to 40mg daily after 24 weeks on 20mg/day if control not achieved. May increase to 60mg daily if control not achieved with 40mg/day after 16 weeks. Discontinue after 16 weeks of 60mg/day if adequate response not achieved. (Max dose 60mg/day).

(*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 member meets all the requirements listed above, the medication will be approved for 12 months.

For **reauthorization**:

1. Member has achieved at least a 20% reduction in blood phenylalanine concentration from pre-treatment baseline or a blood phenylalanine concentration of 600 micromol/L or less.

If member meets all the reauthorization requirements above, the medication will be approved for an additional 12 months.

CareSource considers Palynziq (pegvaliase-pqpz) not medically necessary for the treatment of diseases that are not listed in this document.

DATE	ACTION/DESCRIPTION
07/27/2018	New policy for Palynziq (pegvaliase-pqpz) 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.

References:

1. Palynziq [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; November 2020.
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3. Vockley J, Andersson HC, Antshel KM, et al. Phenylalanine hydroxylase deficiency: diagnosis and management guideline [published correction appears in Genet Med. 2014 Apr;16(4):356]. *Genet Med*. 2014;16(2):188-200. doi:10.1038/gim.2013.157
4. 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
5. 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
6. 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
7. 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
8. 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

Effective date: 10/01/2021
Revised date: 04/30/2021