Effective Date 06/07/2019
Reviewed: 6/2019, 6/2020, 10/2020, 4/2021, 3/2022, 5/2023, 4/2024, 5/2025, 11/2025 Scope: Medicaid

Palynziq (pegvaliase-pqpz)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met, and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication
Indicated to reduce blood Phenylalanine (Phe) concentrations in adult patients with phenylketonuria who have uncontrolled blood Phe concentrations greater than 600 μmol/L on existing management

All other indications are considered experimental/investigational and not medically necessary.

II. CRITERIA FOR APPROVAL

An authorization of 3 months may be granted when all the following criteria are met:

A. Member is ≥ 18 years of age
B. The medication is prescribed by, or in consultation with, a physician who specializes in the treatment of metabolic disease and/or phenylketonuria (PKU).
C. Documentation that member has a diagnosis of phenylketonuria (PKU)
D. Prescriber must be certified with Palynziq REMS program
E. Documentation that member is currently uncontrolled on existing management and has a blood phenylalanine concentration greater than 600 μmol/L (labs to be provided)
F. Member has had a documented inadequate treatment response or intolerance to a formulary sapropterin product in addition to compliance with dietary restriction of Phe
G. Member must always have a prescription of auto-injectable epinephrine on hand
H. Palynziq will not be used in combination with sapropterin (e.g., Kuvan, Javygtor) or sepiapterin (Sephience)

III. CONTINUATION OF THERAPY

A. Palynziq is not being used in combination with a sapropterin product (Kuvan, Javygtor, etc.) or sepiapterin (Sephience) **AND**
B. Current documentation has been provided of blood phenylalanine concentration less than or equal to 600 μmol/L **AND**
C. The request is for less than or equal to 20 mg daily. Note: Member has been titrated over 3 months to the lowest effective tolerated dose **OR**
D. The request is for 40 mg daily and documentation has been provided that the member has been adherent to 20 mg daily for at least 24 weeks **OR**
E. The request is for 60 mg daily and documentation has been provided that member has been adherent to 40 mg daily for at least 16 weeks

IV. QUANTITY LIMIT

- Palynziq 2.5mg/0.5 ml: 8 syringes per 28 days (daily dose of 0.143 ml)

- Palynziq 10mg/0.5 ml: 30 syringes per 30 days (daily dose of 0.5 ml)
- Palynziq 20mg/ml: 60 syringes per 30 days (daily dose of 2 ml), with post-exception limit for 90 syringes per 30 days (daily dose of 3 ml) if 60 mg dose approved.

V. COVERAGE DURATION

- Initial 3 months
- Renewal:
  - Dose of 20mg once daily: 6 months
  - Dose of 40mg or 60mg once daily: 4 months

VI. DOSING

Treatment	Palynziq Dosage	Duration
Induction	2.5 mg once weekly	4 weeks
Titration	2.5 mg twice weekly	1 week
	10 mg once weekly	1 week
	10 mg twice weekly	1 week
	10mg four times weekly	1 week
	10mg once a daily	1 week
Maintenance	20mg once daily	24 weeks
	40mg once daily	16 weeks
Maximum	60mg once daily	16 weeks

VII. REFERENCES

Palynziq [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; December 2022.