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Section:	Prescription Drugs	**Effective Date:**	July 1, 2026
Subsection:	Endocrine and Metabolic Drugs	**Original Policy Date:**	March 20, 2026
Subject:	Harliku	**Page:**	1 of 4

Last Review Date: June 11, 2026

Harliku

Description

Harliku (nitisinone)

Background

Harliku (nitisinone) is a competitive inhibitor of hydroxyphenyl-pyruvate dioxygenase, an enzyme upstream of homogentisate 1,2-dioxygenase (HGD) in the tyrosine catabolic pathway. In patients with alkaptonuria, homogentisic acid (HGA) accumulates in various tissues and urine. Harliku treatment resulted in reduction of urinary HGA concentrations in patients with AKU (1-2).

Regulatory Status

FDA-approved indication: Harliku is a hydroxyphenyl-pyruvate dioxygenase inhibitor indicated for the reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU) (1).

Harliku contains warnings regarding ocular symptoms and hyperkeratotic plaques due to elevated plasma tyrosine levels and leukopenia and severe thrombocytopenia. Assess plasma tyrosine levels in patients presenting with ocular signs and symptoms. Obtain slit-lamp examination prior to treatment and regularly thereafter. Implement diet restriction and/or treatment interruption as appropriate. Monitor platelet and white blood cell counts during Harliku therapy (1).

The safety and effectiveness of Harliku in pediatric patients less than 18 years of age have not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Harliku may be considered **medically necessary** if the conditions indicated below are met.

Harliku may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

1. Alkaptonuria (AKU)

AND ALL of the following:

a. Used for the reduction of urine homogentisic acid (HGA)
b. Diagnosis is confirmed by biochemical testing [e.g., detection of increased levels of homogentisic acid (HGA) in urine], enzyme assay, or genetic testing
c. Prescribed by, or in consultation with, a geneticist, metabolic disease specialist, or rheumatologist

Prior – Approval *Renewal* Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

1. Alkaptonuria (AKU)

AND ALL of the following:

a. Used for the reduction of urine homogentisic acid (HGA)
b. Patient has demonstrated a positive clinical response to therapy (e.g.,

decrease in urine HGA)

Policy Guidelines

Pre - PA Allowance

None

Prior - Approval Limits

Quantity 2 mg per day

Duration 12 months

Prior – Approval *Renewal* Limits

Same as above

Rationale

Summary

Harliku is a hydroxyphenyl-pyruvate dioxygenase inhibitor indicated for the reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU). Harliku contains warnings regarding ocular symptoms and hyperkeratotic plaques due to elevated plasma tyrosine levels and leukopenia and severe thrombocytopenia. The safety and effectiveness of Harliku in pediatric patients less than 18 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Harliku while maintaining optimal therapeutic outcomes.

References

1. Harliku [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd; June 2025.

Policy History

Date	Action
March 2026	Addition to PA
June 2026	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 11, 2026 and is effective on July 1, 2026.