UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2025 P 2385-1
Program	Prior Authorization/Medical Necessity
Medication	Harliku™ (nitisinone)
P&T Approval Date	11/1/2025
Effective Date	2/1/2026

1. Background:

Harliku™ (nitisinone) is a hydroxyphenyl-pyruvate dioxygenase inhibitor indicated for the reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU).

2. Coverage Criteria[a]:

A. Initial Authorization

1. **Harliku** will be approved based on **all** of the following criteria:

   a. Diagnosis of alkaptonuria

   -AND-

   b. Patient is ≥ 18 years old

   -AND-

   c. Submission of medical records (e.g., chart notes, laboratory values) confirming **one** of the following:

      (1) Urinary homogentisic acid (HGA) excretion > 0.4g/24 hours
      (2) Biallelic mutation in *homogentisate 1,2-dioxygenase (HGD)* gene confirmed by genetic testing

   -AND-

   d. Harliku will not be used in combination with **any** of the following:

      (1) Generic nitisinone
      (2) Nityr
      (3) Orfadin

   -AND-

   e. Prescribed by or in consultation with a geneticist, metabolic disease specialist, or rheumatologist.

Authorization will be issued for 12 months.

B. Reauthorization

1. **Harliku** will be approved based on **all** of the following criteria:

   a. Documentation of positive clinical response to Harliku therapy (e.g., reduced urinary HGA levels, improvement in joint symptoms)

   -AND-

   b. Harliku will not be used in combination with **any** of the following:

      (1) Generic nitisinone
      (2) Nityr
      (3) Orfadin

   -AND-

   c. Prescribed by or in consultation with a geneticist, metabolic disease specialist, or rheumatologist

Authorization will be issued for 12 months.

[a] State mandates may apply. Any federal regulatory requirements and the member specific benefit plan coverage may also impact coverage criteria. Other policies and utilization management programs may apply.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.
- Supply limits may also be in place.

4. References:

1. Harliku [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; June 2025.
2. Ranganath LR, Milan AM, Hughes AT, et al. Suitability of nitisinone in alkaptonuria 1 (SONIA 1): an international, multicentre, randomised, open-label, no-treatment controlled, parallel-group, dose-response study to investigate the effect of once daily nitisinone on 24-h urinary homogentisic acid excretion in patients with alkaptonuria after 4 weeks of treatment. *Ann Rheum Dis*. 2016;75(2):362-367.
3. Ranganath LR, Psarelli EE, Arnoux JB, et al. Efficacy and safety of once-daily nitisinone for patients with alkaptonuria (SONIA 2): an international, multicentre, open-label, randomised controlled trial. *Lancet Diabetes Endocrinol*. 2020;8(9):762-772.
4. Alkaptonuria. National Organization for Rare Disorders. https://rarediseases.org/rare-diseases/alkaptonuria/. Published June 26, 2017. Accessed on September 23, 2025.

Program	Prior Authorization/Medical Necessity - Harliku (nitisinone)
Change Control	
11/2025	New program