UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2026 P 1517-1
Program	Prior Authorization/Notification
Medication	Nitisinone Products: Harliku™ (nitisinone) tablets, nitisinone (generic Orfadin) capsules, Nityr® (nitisinone)* tablets, Orfadin® (nitisinone) capsules and oral suspension
P&T Approval Date	2/2026
Effective Date	5/1/2026

1. Background:

Nitisinone is a hydroxyphenyl-pyruvate dioxygenase inhibitor. Harliku™ (nitisinone) is indicated for the reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU). Nitisinone (generic Orfadin®), Nityr® (nitisinone), and Orfadin® (nitisinone) are indicated for the treatment of adult and pediatric patients with hereditary tyrosinemia type 1 (HT-1) in combination with dietary restriction of tyrosine and phenylalanine.

2. Coverage Criteria[a]:

A. Alkaptonuria

1. **Initial Authorization**

   a. **Harliku** will be approved based on **both** of the following criteria:

      (1) Diagnosis of alkaptonuria

      -AND-

      (2) Harliku will not be used in combination with another nitisinone product

   Authorization will be issued for 12 months.

2. **Reauthorization**

   a. **Harliku** will be approved based on **both** of the following criteria:

      (1) Documentation of positive clinical response [e.g., decreased urinary homogentisic acid (HGA) levels] to Harliku therapy

      -AND-

      (2) Harliku will not be used in combination with another nitisinone product

   Authorization will be issued for 12 months.

B. Hereditary Tyrosinemia Type 1

1. **Initial Authorization**

   a. **Nityr*** and **Orfadin** will be approved based on **all** of the following criteria:

      (1) Diagnosis of hereditary tyrosinemia type 1

      -AND-

      (2) Prescribed in conjunction with a tyrosine- and phenylalanine- restricted diet

      -AND-

      (3) The requested drug will not be used in combination with another nitisinone product

   Authorization will be issued for 12 months.

2. **Reauthorization**

   a. **Nityr* and Orfadin** will be approved based on **all** of the following criteria:

      (1) Documentation of positive clinical response (e.g., decrease in urinary/plasma succinylacetone and alpha-1-microglobulin levels) while on therapy

      -AND-

      (2) Prescribed in conjunction with a tyrosine- and phenylalanine- restricted diet

      -AND-

      (3) The requested drug will not be used in combination with another nitisinone product

   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.

*Nityr is typically excluded from coverage. Tried/failed criteria may be in place. Please refer to plan specifics to determine exclusion status.

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 be in place.

4. References:

1. Harliku [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; June 2025.
2. Nityr [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; May 2024.
3. Orfadin [prescribing information]. Waltham, MA: Sobi, Inc.; November 2021.

Program	Prior Authorization/Notification – Nitisinone Products: Harliku tablets, nitisinone capsules, Nityr tablets, Orfadin capsules and oral suspension
Change Control	
2/2026	New program