UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2025 P 1185-10
Program	Prior Authorization/Notification
Medication	Orfadin® (nitisinone)
P&T Approval Date	5/2016, 5/2017, 5/2018, 5/2019, 5/2020, 5/2021, 5/2022, 5/2023, 5/2024, 5/2025
Effective Date	8/1/2025

1. Background:

Orfadin (nitisinone) is a hydroxy-phenylpyruvate dioxygenase inhibitor 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. Initial Authorization

1. **Orfadin** will be approved based on **both** of the following criteria:

   a. Diagnosis of hereditary tyrosinemia type 1

   -AND-

   b. Orfadin is being used as an adjunct to diet modification

   Authorization will be issued for 12 months.

B. Reauthorization

1. **Orfadin** will be approved based on the following criterion:

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

   Authorization will be issued for 24 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 be in place.

4. References:

1. Orfadin [prescribing information]. Waltham, MA. Sobi, Inc. November 2021.

Program	Prior Authorization/Notification – Orfadin (nitisinone) capsules, for oral use, and oral suspension
Change Control	
5/2016	New program
5/2017	Annual review. Added criteria to align with package insert (used as adjunct to diet modification). Updated reauthorization verbiage to align with standard verbiage (patient shows evidence of). Updated references.
5/2018	Annual review. Updated references.
5/2019	Annual review. No changes to coverage criteria.
5/2020	Annual review with no changes to clinical criteria. Updated reference.
5/2021	Annual review. No changes to coverage criteria.
5/2022	Annual review. No changes to coverage criteria.
5/2023	Annual review. Added state mandate footnote. Updated reference.
5/2024	Annual review. No changes to clinical criteria.
5/2025	Annual review. No changes to clinical criteria.