Effective Date: 6/2019
Revised: 10/2019, 06/2020 Reviewed: 6/2019, 10/2019, 6/2020, 3/2021, 3/2022, 3/2023, 3/2024, 3/2025, 3/2026, 5/2026 Scope: Medicaid

Nitisinone (generic Orfadin) capsules
Nityr (nitisinone) tablets
Orfadin (nitisinone) suspension
Harliku (nitisinone) tablets

POLICY

I. INDICATIONS

Nitisinone is 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.
- the reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU).

II. CRITERIA FOR INITIAL APPROVAL

Authorization of 6 months may be granted for treatment of hereditary tyrosinemia type 1 (HT-1) when all of the following are met:

1. Documentation that diagnosis is confirmed by biochemical testing (e.g., detection of succinylacetone in urine), enzyme assay, or genetic testing
2. The requested medication is being used as an adjunct to dietary restriction of tyrosine and phenylalanine; OR

Authorization of 6 months may be granted for treatment of alkaptonuria (AKU) when all of the following are met:

1. Member is 18 years of age or older
2. Documentation that diagnosis of AKU is confirmed by urinary HGA excretion of greater than 0.4 g/24 hours
3. Member has clinical manifestations of AKU such as ochronosis or chronic joint pain

If requesting brand Nityr (nitisinone) tablets, documentation provided of intolerable adverse event to nitisinone capsules, and the adverse event was not an expected adverse event attributed to the active ingredient as described in the prescribing information.

If requesting brand Orfadin (nitisinone), documentation provided of intolerable adverse event to nitisinone capsules AND Nityr (nitisinone) tablets, and the adverse event was not an expected adverse event attributed to the active ingredient as described in the prescribing information.

If requesting Orfadin oral suspension, documentation provided that the member is unable to tolerate/swallow the oral tablet AND capsule.

If requesting Harliku (nitisinone) tablets, documentation provided of intolerable adverse event to nitisinone capsules AND Nityr (nitisinone) tablets, and the adverse event was not an expected adverse event attributed to the active ingredient as described in the prescribing information.

III. CONTINUATION OF THERAPY

Authorization of 6 months for all members (including new members) requesting authorization for continuation of therapy must meet all initial authorization criteria and documentation is submitted that the member shows evidence of positive clinical response (e.g. decrease in urinary/plasma succinylacetone and alpha-1-microglobulin levels for HT-1, decrease in urinary homogentisic acid levels for AKU) while on therapy.

IV. REFERENCES

1. Orfadin [package insert]. Ardmore, PA: Sobi, Inc; November 2021. Accessed March 2026.
2. Nityr [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; December 2025. Accessed March 2026.
3. Harliku (nitisinone) tablets [Prescribing Information]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd; June 2025.