

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

**POLICY**

**I. INDICATIONS**

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met, and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

Orfadin 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.

Nityr 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.

All other indications are considered experimental/investigational and are not a covered benefit.

**II. CRITERIA FOR INITIAL APPROVAL**

Authorization of 6 months may be granted for treatment of hereditary tyrosinemia type 1 (HT-1) when the diagnosis is confirmed by biochemical testing (e.g., detection of succinylacetone in urine), enzyme assay, or genetic testing and the requested medication is being used as an adjunct to dietary restriction of tyrosine and phenylalanine.

If requesting brand Nityr (nitisinone) tablets, documentation provided of failure or intolerance to generic nitisinone capsules.

If requesting brand Orfadin (nitisinone), documentation provided of failure or intolerance to generic nitisinone capsules AND Nityr (nitisinone) tablets.

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

**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 the member shows evidence of

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Scope: Medicaid

positive clinical response (e.g. decrease in urinary/plasma succinylacetone and alpha-1-microglobulin levels) 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.