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

SPECIALTY GUIDELINE MANAGEMENT

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) or DNA testing.

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

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.
2. Nityr [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; January 2024.