Reference number(s)
2120-A

SPECIALTY GUIDELINE MANAGEMENT

NITYR (nitisinone)
ORFADIN (nitisinone)
nitisinone (generic)

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
Nityr/Orfadin/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.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review: biochemical testing, enzyme assay, or genetic testing results supporting diagnosis.

III. CRITERIA FOR INITIAL APPROVAL

Hereditary Tyrosinemia Type 1 (HT-1)
Authorization of 12 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.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section III who are experiencing beneficial clinical response from therapy.

V. REFERENCES

1. Orfadin [package insert]. Waltham, MA: Sobi, Inc; November 2021.
2. Nityr [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; June 2021.
3. nitisinone [package insert]. Chestnut Ridge, NY: Par Pharmaceutical; October 2019.