Reference number(s)
2120-A

SPECIALTY GUIDELINE MANAGEMENT

ORFADIN (nitisinone)
NITYR (nitisinone)

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 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. REQUIRED 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

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) or DNA 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 hereditary tyrosinemia type 1 (HT-1) who are experiencing beneficial clinical response from therapy.

V. REFERENCE

1. Orfadin [package insert]. Ardmore, PA: Sobi, Inc; May 2019.
2. Nityr [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; November 2018.