Reference number(s)
2120-A

Specialty Guideline Management nitisinone products

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Nityr	nitisinone
Orfadin	nitisinone
Harliku	nitisinone

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 Indications[1-3]

nitisinone (generic)/Nityr/Orfadin:

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.

Harliku:

Indicated for the reduction of urine homogentisic acid (HGA) in adult patients with alkaptonuria (AKU).

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

Documentation

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

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of enzyme or metabolic disorders.

Coverage Criteria

Hereditary Tyrosinemia Type 1 (HT-1) (nitisinone (generic)/Nityr/Orfadin Only) [1-3]

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.

Alkaptonuria (AKU) (Harliku Only)[4,5]

Authorization of 12 months may be granted for treatment of alkaptonuria (AKU) when the all of the following criteria are met:

- Member is 18 years of age or older.
- Diagnosis is confirmed by biochemical testing (e.g., detection of increased levels of homogentisic acid (HGA) in urine), enzyme assay, or genetic testing.

Continuation of Therapy

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

References

1. Orfadin [package insert]. Waltham, MA: Sobi, Inc; November 2021.
2. Nityr [package insert]. Cambridge, United Kingdom: Cycle Pharmaceuticals Ltd.; May 2024.
3. nitisinone [package insert]. Chestnut Ridge, NY: Par Pharmaceutical; October 2019.
4. Harliku [package insert]. Boston, MA: Cycle Pharmaceuticals Ltd.; June 2025.
5. Introne WJ, Perry M, Chen M. Alkaptonuria. 2003 May 9 [Updated 2021 Jun 10]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1454/