



STANDARD COMMERCIAL DRUG FORMULARY  
PRIOR AUTHORIZATION GUIDELINES

NITISINONE

Generic	Brand	HICL	GCN	Medi-Span	Exception/Other
NITISINONE	ORFADIN, NITYR, NITISINONE	23253		GPI-10 (3090404500)	

**GUIDELINES FOR USE**

**INITIAL CRITERIA (NOTE: FOR RENEWAL CRITERIA SEE BELOW)**

1. Does the patient have a documented diagnosis of hereditary tyrosinemia type 1 (HT-1) and meet **ALL** of the following criteria?
  - The patient has elevated urinary or plasma succinylacetone (SA) levels OR a mutation in the fumarylacetoacetate hydrolase (FAH) gene
  - Therapy is prescribed by or in consultation with a prescriber specializing in inherited metabolic diseases
  - The patient has been counseled on maintaining dietary restriction of tyrosine and phenylalanine

If yes, continue to #2.

If no, do not approve.

**DENIAL TEXT:** See the initial denial text at the end of the guideline.

2. Is the request for Nityr tablets; brand Orfadin 2mg, 5mg, 10 mg, 20 mg capsules; or Orfadin suspension **AND** the patient meets the following criterion?
  - The patient had a trial of or contraindication to generic nitisinone capsule

If yes, **approve the requested drug for 6 months by GPID or GPI-14.**

If no, do not approve.

**DENIAL TEXT:** See the initial denial text at the end of the guideline.

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INITIAL CRITERIA (CONTINUED)

**INITIAL DENIAL TEXT:** \*Some terms are already pre-defined in parenthesis. Please use these definitions if the particular text you need to use does not already have definition(s) in it.

Our guideline named **NITISINONE (Orfadin, Nityr)** requires the following rule(s) be met for approval:

- A. You have hereditary tyrosinemia type 1 (HT-1: a type of genetic disorder where you cannot breakdown an important component in proteins)
- B. Your diagnosis is confirmed by elevated urinary or plasma succinylacetone levels (a chemical that is present in hereditary tyrosinemia) OR a mutation in the fumarylacetoacetate hydrolase gene
- C. Therapy is prescribed by or in consultation with a prescriber specializing in inherited metabolic diseases
- D. You have been counseled on maintaining dietary restriction of tyrosine and phenylalanine
- E. **If you are requesting Nityr tablets; brand Orfadin 2mg, 5mg, 10 mg, 20 mg capsules; or Orfadin oral suspension, approval also requires:**
  1. You have tried or have a contraindication (harmful for) to generic nitisinone capsules

Your doctor told us **[INSERT PT SPECIFIC INFO PROVIDED]**. We do not have information showing you **[INSERT UNMET CRITERIA]**. This is why your request is denied. Please work with your doctor to use a different medication or get us more information if it will allow us to approve this request.

RENEWAL CRITERIA

1. Does the patient have a diagnosis of hereditary tyrosinemia type 1 **AND** meet the following criterion?
  - The patients urinary or plasma succinylacetone (SA) levels have decreased from baseline while on treatment with nitisinone.

If yes, **approve for 12 months by GPID or GPI-14 for all strengths of the requested formulation.**

If no, do not approve.

**DENIAL TEXT:** See the renewal denial text at the end of the guideline.

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NITISINONE

RENEWAL CRITERIA (CONTINUED)

**RENEWAL DENIAL TEXT:** \*Some terms are already pre-defined in parenthesis. Please use these definitions if the particular text you need to use does not already have definition(s) in it.

Our guideline named **NITISINONE (Orfadin, Nityr)** requires the following rule(s) be met for renewal:

- A. You have hereditary tyrosinemia type 1 (HT-1: a type of genetic disorder where you cannot breakdown an important component in proteins)
- B. Your urinary or plasma succinylacetone levels (a chemical that is present in hereditary tyrosinemia) have decreased from baseline while on treatment with nitisinone

Your doctor told us [INSERT PT SPECIFIC INFO PROVIDED]. We do not have information showing you [INSERT UNMET CRITERIA]. This is why your request is denied. Please work with your doctor to use a different medication or get us more information if it will allow us to approve this request.

**RATIONALE**

For further information, please refer to the Prescribing Information and/or Drug Monograph for Orfadin and Nityr.

**REFERENCES**

- Orfadin [Prescribing Information]. Waltham, MA: Sobi, Inc.; May 2019.
- Nityr [Prescribing Information]. Cambridge, UK: Cycle Pharmaceuticals Ltd.; September 2020.

Library	Commercial	NSA
Yes	Yes	No

Part D Effective: N/A

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