Pharmacy Medical Necessity Guidelines: Orfadin® (nitisinone)

**Effective:** July 11, 2017

<table>
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<th>Prior Authorization Required</th>
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<th>Type of Review – Care Management</th>
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This Pharmacy Medical Necessity Guideline applies to the following:

**Tufts Health Plan Commercial Plans**
- ✓ Tufts Health Plan Commercial Plans – large group plans
- ✓ Tufts Health Plan Commercial Plans – small group and individual plans

**Tufts Health Public Plans**
- ✓ Tufts Health Direct – Health Connector
- ✓ Tufts Health Together – A MassHealth Plan
- ✓ Tufts Health RITogether – A Rite Care + Rhody Health Partners Plan

**Tufts Health Freedom Plan products**
- ✓ Tufts Health Freedom Plan – large group plans
- ✓ Tufts Health Freedom Plan – small group plans

**Fax Numbers:**
- RXUM: 617.673.0988

**Note:** For Tufts Health Plan Medicare Preferred Members, please refer to the Tufts Health Plan Medicare Preferred Prior Authorization Criteria. Background, applicable product and disclaimer information can be found on the last page.

**OVERVIEW**

The Food and Drug Administration (FDA) approved the orphan drug Orfadin (nitisinone) for the treatment of hereditary tyrosinemia type 1 (HT-1), a rare genetic metabolic disorder that causes progressive liver disease and renal tubular dysfunction. The disorder is caused by fumarylacetoacetate hydrolase deficiency, the last enzyme in the degradation pathway of the amino acid tyrosine, resulting in the accumulation of toxic metabolites. HT-1 is present at birth and manifests within weeks or months in the failure of the infant to thrive and by symptoms of hepatomegaly, edema, ascites, melena, and hemorrhagic diathesis.

Orfandin (nitisinone) inhibits 4-hydroxyphenylpyruvate dioxygenase and prevents the formation of toxic metabolites involved in hepatic and renal lesions. Because Orfandin (nitisinone) inhibits the breakdown of tyrosine which leads to increase plasma levels of tyrosine, patients receiving Orfandin (nitisinone) must restrict their dietary intake of tyrosine and phenylalanine.

**FDA-APPROVED INDICATIONS**

Orfadin (nitisinone) is indicated for the treatment of HT-1 in combination with dietary restriction of tyrosine and phenylalanine.

**COVERAGE GUIDELINES**

The plan may authorize coverage of Orfadin (nitisinone) when the following criterion is met:

1. Documentation of genetic tyrosinemia Type-1 (hereditary tyrosinemia Type-1)

**LIMITATIONS**

1. Orfadin (nitisinone) will not be covered for any indication other than tyrosinemia Type-1 (hereditary tyrosinemia Type-1).

**CODES**

None

**REFERENCES**

**APPROVAL HISTORY**

November 2002: Reviewed by Pharmacy & Therapeutics Committee.

Subsequent endorsement date(s) and changes made:
- August 9, 2005: No changes
- September 14, 2004: No changes
- July 11, 2006: No changes
- July 10, 2007: No changes
- July 08, 2008: No changes
- July 14, 2009: No changes
- January 1, 2010: Removal of Tufts Medicare Preferred language (separate criteria have been created specifically for Tufts Medicare Preferred)
- July 13, 2010: No changes
- July 12, 2011: No changes
- April 10, 2012: No changes
- March 12, 2013: No changes
- March 11, 2014: No changes
- March 10, 2015: No changes
- January 1, 2016: Administrative change to rebranded template.
- February 9, 2016: No changes
- July 12, 2016: No changes
- April 11, 2017: Administrative update, Adding Tufts Health RITogether to the template.
- July 11, 2017: No changes

**BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION**

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. They are used in conjunction with a Member's benefit document and in coordination with the Member's physician(s). The plan makes coverage decisions on a case-by-case basis considering the individual Member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

This Pharmacy Medical Necessity Guideline does not apply to Uniformed Services Family Health Plan Members or to certain delegated service arrangements. Unless otherwise noted in the Member's benefit document or applicable Pharmacy Medical Necessity Guideline, Pharmacy Medical Necessity Guidelines do not apply to CareLinkSM Members. For self-insured plans, drug coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a coverage guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern. Applicable state or federal mandates will take precedence.

For Tufts Health Plan Medicare Preferred, please refer to Tufts Health Plan Medicare Preferred Prior Authorization Criteria.

Treating providers are solely responsible for the medical advice and treatment of Members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to Member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.