Harvard Pilgrim Health Care | TUFTS Health Plan

Pharmacy Medical Necessity Guidelines:

Nitisinone Products: Orfadin (nitisinone)

Effective: December 9, 2025

Guideline Type	☒ Prior Authorization ☐ Non-Formulary ☐ Step-Therapy ☐ Administrative

Applies to:

Commercial Products

☒ Harvard Pilgrim Health Care Commercial products; Fax: 617-673-0988

☒ Tufts Health Plan Commercial products; Fax: 617-673-0988

CareLink℠ – Refer to CareLink Procedures, Services and Items Requiring Prior Authorization

Public Plans Products

☒ Tufts Health Direct – A Massachusetts Qualified Health Plan (QHP) (a commercial product); Fax: 617-673-0988

Note: While you may not be the provider responsible for obtaining prior authorization, as a condition of payment you will need to ensure that prior authorization has been obtained.

Overview

Food and Drug Administration – Approved Indications

Orfadin (nitisinone capsules/suspension) is a hydroxy-phenylpyruvate dioxygenase inhibitor indicated for the treatment of adult and pediatric patients with hereditary tyrosinemia type 1 (HT-1) in combination with dietary restrictions of tyrosine and phenylalanine.

Clinical Guideline Coverage Criteria

The plan may authorize coverage of Orfadin (nitisinone) suspension or nitisinone capsules Members when **ALL** of the following criteria are met:

1. Documented diagnosis of hereditary tyrosinemia type 1

AND

2. Documented used as an adjunct to dietary restriction of tyrosine and phenylalanine

Limitations

1. For a non-formulary medication request, please refer to the Pharmacy Medical Necessity Guidelines for Formulary Exceptions and submit a formulary exception request to the plan as indicated

Codes

None

References

1. Orfadin (nitisinone) [prescribing information]. Waltham, MA: Sobi Inc; November 2021.
2. Grompe, M. Disorders of Tyrosine Metabolism. In: UpToDate, TePas, E (ed), Waltham, MA, 2021. Accessed October 6, 2021.

Approval And Revision History

September 13, 2022: Reviewed by the Pharmacy & Therapeutics Committee.

- August 8, 2023: No changes
- December 10, 2024: Removed Nityr from the Medical Necessity Guideline as coverage will be non-formulary effective January 1, 2025 (eff 1/1/25).
- December 9, 2025: 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. 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.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

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.