Premier Health Plan

POLICY AND PROCEDURE

POLICY TITLE: ***Orfadin (nitisinone)***
DEPARTMENT: **Clinical Pharmacy Services- Utilization Management**
ORIGINAL DATE: ***June 2011 (as adopted from UPMC Health Plan)***

Last P & T Committee Approval Date: *February 2018*

Product Applicability: *mark all applicable products below:*

COMMERCIAL	[ ] HMO [ ] PPO *Products*: [ ] Small [ ] Indiv. [ ] Large *Exchange*: [ ] Shop [ ] Indiv. [x] All
OTHER	[x] Self-funded/ASO

PURPOSE

The purpose of this policy is to define the prior authorization process for Orfadin (nitisinone).

Orfadin (nitisinone) is a synthetic, reversible inhibitor of 4-hydroxyphenylpyruvate dioxygenase indicated for use as an adjunct to dietary restriction of tyrosine and phenylalanine in the treatment of HT-1.

DEFINITIONS

Hereditary Tyrosinemia Type 1 (HT-1): a rare metabolic disease in children. In normal, unaffected individuals, excess amounts of the amino acid tyrosine are degraded in several steps. In HT-1, however, one of the enzymes in this degradation, fumarylacetoacetase hydrolase (FAH), is deficient. Tyrosine and its toxic metabolites [fumarylacetoacetate, maleylacetoacetate, succinylacetone (SA), and succinylacetoacetate (SAA)] thus build up in the body and cause serious medical problems such as liver failure and hepatocellular carcinoma. Kidney dysfunction, skeletal changes, and neurological manifestations may also occur.

POLICY

It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, and approval by the Pharmacy & Therapeutics Committee of the criteria for prior authorization, as described in RX.002 Pharmacy and Therapeutics Committee and RX.003-Prior Authorization Process.

The drug, Orfadin (nitisinone), is subject to the prior authorization process.

PROCEDURE

Initial Authorization Criteria:

Must meet all of the criteria listed under the respective product:

1. **Orfadin (nitisinone) capsule:**
   - Must be prescribed by a physician who specializes in the treatment of inherited metabolic disorders or in consultation with this specialist
   - Must have diagnosis of HT-1
     - Must have a laboratory test of baseline SA level
   - Must be used as an adjunct to dietary restriction of tyrosine and phenylalanine
   - Must have undergone baseline liver evaluation and ophthamalogic testing

2. **Orfadin (nitisinone) oral suspension:**
   - Must meet above criteria for Orfadin capsule
   - Must have chart documentation of the clinical rationale for why Orfadin capsules cannot be used

Reauthorization Criteria:

All prior authorization renewals are reviewed on an annual basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at 1-year intervals based upon the following:

- Documentation from the prescriber that the member's disease has improved/stabilized based upon the prescriber's assessment while on therapy
- Documentation that the member is being monitored hematologic and hepatic side effects of the medication
- Documentation of laboratory test which demonstrates progressive SA suppression

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 6 months
Reauthorization	Up to 1 year

If the established criteria are not met, the request is referred to a Medical Director for review.

REFERENCES

1. Disorders of tyrosine metabolism. UpToDate. Accessed 6/27/2011.
2. http://www.orfadin.com Accessed 4/28/2011.
3. Orfadin [package insert]. Sweden. Apotek Production & Laboratories AB; June 2016.

RECORD RETENTION

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual review	*02/17, 02/18*
Criteria update	*10/16*