Pharmacy Policy Bulletin: J-0463 Xuriden (uridine triacetate) – Commercial and Healthcare Reform	
Number: J-0463	**Category:** Prior Authorization
Line(s) of Business: ☒ Commercial ☒ Healthcare Reform ☐ Medicare	**Benefit(s):** **Commercial:** **Prior Authorization (1.):** 1. Miscellaneous Specialty Drugs Oral = Yes w/ Prior Authorization **Healthcare Reform:** Not Applicable
Region(s): ☒ All ☐ Delaware ☐ New York ☐ Pennsylvania ☐ West Virginia	**Additional Restriction(s):** None
Version: J-0463-011	**Original Date:** 12/02/2015
Effective Date: 10/28/2024	**Review Date:** 10/02/2024

Drugs Product(s):	• Xuriden (uridine triacetate)
FDA-Approved Indication(s):	• Adult and pediatric patients for the treatment of hereditary orotic aciduria.

Background:	• Xuriden is an acetylated form of uridine. It provides uridine in the systemic circulation of patients with hereditary orotic aciduria who cannot synthesize adequate quantities of uridine on their own due to a genetic enzymatic deficiency of uridine monophosphate synthase (UMPS).
	• Hereditary orotic aciduria is an ultra-rare genetic condition that is caused by variations in the uridine monophosphate synthetase (*UMPS)* gene, and results in lack of uridine due to deficiency of the UMPS enzyme. Clinical manifestations caused by lack of uridine include developmental delay, failure to thrive, and megaloblastic anemia. Further, urinary orotic acid levels are increased because orotic acid is a substrate of the deficient enzyme. Without the enzyme, orotic acid accumulates and is excreted in the urine. Uridine replacement reduces orotic acid overproduction through feedback inhibition, which decreases its excretion into the urine.
	• In clinical trials, endpoints evaluated were stability or improvement in hematologic parameters (individualized) and urine orotic acid levels.
	• ICD-10 Code Information:
	  ○ ICD-10: E79.8 “Other disorders of purine and pyrimidine metabolism” may apply to Xuriden; however, the prescriber must confirm the member has a diagnosis of hereditary orotic aciduria.
	• Prescribing Considerations:
	  ○ Xuriden should be prescribed by a metabolic disease specialist.

Approval Criteria

I. Initial Authorization

When a benefit, coverage of Xuriden may be approved when the following criterion is met **(A.)**:

A. The member has a diagnosis of hereditary orotic aciduria (no ICD-10 code) supported by one (1) of the following **(1. or 2.)**:

1. Genetic testing confirming mutation of the *UMPS* gene.

2. Elevated levels of orotic acid in the urine over the laboratory reference range.

II. Reauthorization

When a benefit, reauthorization of Xuriden may be approved when the following criterion is met **(A.)**:

A. The member has experienced a positive clinical response to therapy defined as one (1) of the following **(1. or 2.)**:

1. Decreased urinary orotic acid levels from baseline.

2. Stabilization or improvement of hematologic parameters (e.g., neutrophil count, neutrophil percent, white blood cell count, mean corpuscular volume) from baseline.

III. An exception to some or all of the criteria above may be granted for select members and/or circumstances based on state and/or federal regulations.

Limitations of Coverage

I. Coverage of drug(s) addressed in this policy for disease states outside of the FDA-approved indications should be denied based on the lack of clinical data to support effectiveness and safety in other conditions unless otherwise noted in the approval criteria.

II. For Commercial or HCR members with a closed formulary, a non-formulary product will only be approved if the member meets the criteria for a formulary exception in addition to the criteria outlined within this policy.

Authorization Duration

- Commercial and HCR Plans: If approved, up to a 12 month authorization may be granted.

Automatic Approval Criteria

None

References:

1. Xuriden [prescribing information]. Rockville, MD: Wellstat Therapeutics; August 2023.
2. U.S. Department of Health & Human Services. Genetic and Rare Diseases Information Center. Orotic aciduria type 1. Available at: https://rarediseases.info.nih.gov/diseases/5429/orotic-aciduria-type-1#ref_13805. Accessed August 12, 2021.
3. National Organization for Rare Disorders. Hereditary Orotic Aciduria. Available at: https://rarediseases.org/rare-diseases/hereditary-orotic-aciduria. Accessed August 29, 2022.