

# Drug Policy

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| <b>Policy:</b> | <b>Tiopronin Products</b> <ul style="list-style-type: none"> <li>tiopronin tablets – generic</li> <li>tiopronin delayed-release tablets – generic</li> <li>Venxxiva® (tiopronin delayed-release tablets – Torrent [generic only])</li> </ul> | <b>Annual Review Date:</b><br><b>02/20/2025</b><br><br><b>Last Revised Date:</b><br><b>02/20/2025</b> |
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## OVERVIEW

Tiopronin products are indicated, in combination with high fluid intake, alkali, and diet modification, for severe homozygous **cystinuria**, for the prevention of cystine kidney stone formation in adults and pediatric patients  $\geq 20$  kg, who are not responsive to these measures alone.<sup>1-3</sup> Amongst the tiopronin delayed-release formulations, Venxxiva is a branded generic product and is supplied in the same dosage strength as Thiola EC.

## POLICY STATEMENT

This policy involves the use of tiopronin products. Prior authorization is recommended for pharmacy benefit coverage of tiopronin products. Approval is recommended for those who meet the conditions of coverage in the **Criteria and Initial/Extended Approval** for the diagnosis provided. **Conditions Not Recommended for Approval** are listed following the recommended authorization criteria. Requests for uses not listed in this policy will be reviewed for evidence of efficacy and for medical necessity on a case-by-case basis.

All approvals for initial therapy are provided for the initial approval duration noted below; if reauthorization is allowed, a response to therapy is required for continuation of therapy unless otherwise noted below.

## RECOMMENDED AUTHORIZATION CRITERIA

Coverage of tiopronin products is recommended in those who meet the following criteria:

1. **Cystinuria.** Approve for 1 year if the patient meets ALL of the following (A, B, C, and D):
  - A) Patient weighs  $\geq 20$  kg; AND
  - B) Diagnosis of cystinuria has been confirmed based on laboratory testing (e.g., urinary cystine crystals present on microscopy, quantitative urine cystine assay); AND
  - C) According to the prescriber, the patient has had an inadequate response to high fluid intake, dietary modification, and urinary alkalization; AND
  - D) The medication is prescribed by or in consultation with a nephrologist, urologist, or physician who specializes in the treatment of cystinuria.

## Initial Approval/ Extended Approval.

- A) *Initial Approval:* 6 months
- B) *Extended Approval:* 6 months

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## CONDITIONS NOT RECOMMENDED FOR APPROVAL

Thiola, Thiola EC, and generic tiopronin has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions. (Note: This is not an exhaustive list of Conditions Not Recommended for Approval).

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

## Documentation Requirements:

The Company reserves the right to request additional documentation as part of its coverage determination process. The Company may deny reimbursement when it has determined that the drug provided or services performed were not medically necessary, investigational or experimental, not within the scope of benefits afforded to the member and/or a pattern of billing or other practice has been found to be either inappropriate or excessive. Additional documentation supporting medical necessity for the services provided must be made available upon request to the Company. Documentation requested may include patient records, test results and/or credentials of the provider ordering or performing a service. The Company also reserves the right to modify, revise, change, apply and interpret this policy at its sole discretion, and the exercise of this discretion shall be final and binding.

## REFERENCES

1. Thiola® tablets [prescribing information]. San Antonio, TX: Mission Pharmacal; June 2019.
2. Thiola® EC delayed-release tablets [prescribing information]. San Antonio, TX: Mission Pharmacal; March 2021.
3. Cystinuria. National Organization for Rare Disorders. Updated 2020. Available at: <https://rarediseases.org/rare-diseases/cystinuria/>. Accessed on January 31, 2025.
4. Castro Pereira DJ, Schoolwerth AC, Pais VM. Cystinuria: current concepts and future directions. *Clin Nephrol*. 2015;83(3):138-146.
5. Pearle MS, Goldfarb DS, Assimos DG, et al. American Urological Association. Medical management of kidney stones: AUA guideline. *J Urol*. 2014;192(2):316-24.
6. Venxxiva® delayed-release tablets [prescribing information]. Cambridge, UK: Torrent; December 2024.
7. Azer SM, Goldfarb DS. A Summary of Current Guidelines and Future Directions for Medical Management and Monitoring of Patients with Cystinuria. *Healthcare (Basel)*. 2023 Feb 24;11(5):674.