MEDICAL MUTUAL®

Drug Policy

Policy:	Thiola (tiopronin) Thiola EC (tiopronin delayed-release)	Annual Review Date: 05/18/2023 Last Revised Date: 05/18/2023

OVERVIEW

Thiola and Thiola EC are active reducing and complexing thiol compounds. These drugs are indicated for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria with urinary cystine greater than 500 mg/day who are resistant to treatment with conservative measures of high fluid intake, urinary alkalization and dietary modification, or who have adverse reactions to d-penicillamine. Cystine stones typically occur in patients who excrete abnormal amounts of cystine in urine. The stone formation is a result of poor aqueous solubility of cystine.

POLICY STATEMENT

This policy involves the use of Thiola, Thiola EC, and generic tiopronin. Prior authorization is recommended for pharmacy benefit coverage of Thiola, Thiola EC, and generic tiopronin. 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 Thiola, Thiola EC, and generic tiopronin is recommended in those who meet the following criteria:

1. <u>Cystinuria</u>

Criteria. *Patient must meet the following criteria*

- **A.** The patient is 9 years of age or older; AND
- **B.** The patient weighs 20 kg or greater; AND
- **C.** The patient has severe homozygous cystinuria with urinary cystine greater than 500 mg/day; AND
- **D.** The patient meets one of the following:
  - **a.** The patient has tried and failed conservative treatment including high fluid intake, urinary alkalization and dietary modifications; OR
  - **b.** The patient has had adverse reactions to d-penicillamine; AND
- **E.** The patient does NOT have a prior history of developing agranulocytosis, aplastic anemia, or thrombocytopenia; AND

F. The patient is NOT pregnant OR the prescriber attests that the anticipated benefit of therapy to inhibit stone formation clearly outweighs the possible hazards of treatment; AND
G. The medication is prescribed by or in consultation with a nephrologist, urologist, or physician who specializes in the treatment of cystinuria; AND
H. The patient will continue high fluid intake, urinary alkalization, and dietary modification while using the requested agent; AND
I. If the request is for brand Thiola (non-EC formulation), the patient has tried generic tiopronin and the brand is requested due to formulation differences in the inactive ingredients [e.g. preservatives, fillers, dyes]

Initial Approval/ Extended Approval.
A) *Initial Approval:* 3 months
B) *Extended Approval:* 6 months

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. Tiopronin. In: DRUGDEX [online database]. Truven Health Analytics; Greenwood Village, CO. Last updated 17 June 2021. Accessed on 18 May 2022.
2. Thiola [prescribing information]. San Antonio, TX: Mission Pharmacal Company; January 2021.
3. Thiola EC [prescribing information]. San Antonio, TX: Mission Pharmacal Company; June 2019.
4. Cystinuria. National Organization for Rare Disorders. Updated 2020. Available at: http://rarediseases.org/rare-disesaes/cystinuria/. Accessed 18 May 2022.