

Tiopronin EC

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Tiopronin is indicated in combination with high fluid intake, alkali, and diet modification, for the prevention of cystine (kidney) stone formation in adult and pediatric patients 20 kg and greater with severe homozygous cystinuria who are not responsive to these measures alone.

All other indications are considered experimental/investigational and are not a covered benefit

II. CRITERIA FOR INITIAL APPROVAL

Cystinuria

Authorization of 6 months may be granted for prevention of cystine stone formation in a member with severe homozygous cystinuria when all of the following criteria are met:

- A. Documented diagnosis of cystinuria was established by one or more of the following:
 - a. Biallelic mutations/variants in the SLC3A1 or SLC7A9 gene confirmed by genetic testing
 - b. Stone analysis revealing 100 percent cystine calculi
 - c. Presence of pathognomonic hexagonal cystine crystals visualized on urine microscopy
- B. Documentation of the pre-treatment urinary cystine level is greater than 500 mg/day
- C. The requested medication is being used as an adjunct to high fluid intake, alkali, and diet modification
- D. Dosing is in accordance with the United States Food and Drug Administration approved labeling

III. CONTINUATION OF THERAPY

- A. Authorization of 6 months may be granted for continued treatment of severe cystinuria in members requesting reauthorization who have documentation of experiencing a decrease in urinary cystine levels compared to pretreatment baseline, is being used as an adjunct to high fluid intake, alkali, and diet modification and dosing is in accordance with the United States Food and Drug Administration approved labeling.

IV. REFERENCES

1. Thiola [package insert]. San Antonio, TX: Mission Pharmaceutical Company; January 2024. Accessed May 2025.
2. Biyani CS, Cartledge JJ. Cystinuria- diagnosis and management. *Eur Urology*. 2006; 4:175-183.