CVS Caremark®

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| Reference number(s) |
| 6863-A |

# Specialty Guideline Management Ctexli

## Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name |
| --- | --- |
| Ctexli | chenodiol |

## 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 Indications1

Ctexli is indicated for the treatment of cerebrotendinous xanthomatosis (CTX) in adults.

All other indications are considered experimental/investigational and not medically necessary.

## Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who is experienced in the treatment of cerebrotendinous xanthomatosis (CTX) (e.g., neurologist, geneticist, endocrinologist, gastroenterologist).

## Documentation

Submission of the following information is necessary to initiate the prior authorization review:

### Initial Requests:

* Genetic testing confirming pathogenic variants in the CYP27A1 gene.
* Laboratory results, chart notes, or medical record documentation of elevated pretreatment plasma cholestanol level.
* Laboratory results, chart notes, or medical record documentation of elevated levels of bile alcohol (i.e., 23s-pentol) in the urine.
* Chart notes or medical record documentation confirming signs and symptoms of CTX.
* Laboratory results, chart notes, or medical record documentation of baseline liver transaminase (i.e., alanine aminotransferase [ALT], aspartate aminotransferase [AST]) and bilirubin levels.

### Continuation Requests:

* Laboratory results, chart notes, or medical record documentation supporting positive clinical response.
* Laboratory results, chart notes, or medical record documentation of current liver transaminase (i.e., ALT, AST) and bilirubin levels.

## Coverage Criteria

### Cerebrotendinous Xanthomatosis1-4

Authorization of 6 months may be granted for treatment of cerebrotendinous xanthomatosis (CTX) in adult members when all of the following criteria are met:

* Diagnosis of CTX is confirmed by genetic testing indicating pathogenic variants in the CYP27A1 gene.
* Member has an elevated pretreatment plasma cholestanol level.
* Member has elevated levels of bile alcohol (i.e., 23s-pentol) in the urine.
* Member has signs and symptoms of CTX (e.g., bilateral cataracts, intractable diarrhea, progressive neurological signs and symptoms, tendon xanthomas).
* Member has a baseline liver transaminase (i.e., ALT, AST) level of less than or equal to 3 times the upper limit of normal (ULN).
* Member has a baseline bilirubin level of less than or equal to 2 times the upper limit of normal (ULN).
* Member has been assessed for malabsorption disorder or other confounding gastrointestinal conditions.
* The requested medication will not be used in combination with bile acid sequestering agents (e.g., cholestyramine, colestipol, aluminum-based antacid).

## Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in adult members requesting reauthorization for cerebrotendinous xanthomatosis (CTX) when all of following criteria are met:

* Member has not experienced signs and symptoms of hepatoxicity (e.g., abdominal pain, bruising, dark-colored urine, jaundice).
* Member has a confirmed liver transaminase (i.e., ALT, AST) level of less than or equal to 3 times the upper limit of normal (ULN).
* Member has confirmed bilirubin level of less than or equal to 2 times the upper limit of normal (ULN).
* The requested medication will not be used in combination with bile acid sequestering agents (e.g., cholestyramine, colestipol, aluminum-based antacid).
* The member has achieved or maintained a positive clinical response as evidenced by any of the following:
* Member has experienced a decreased or stabilized level of bile alcohol (i.e., 23s-pentol) in the urine.
  + Member has experienced a reduction in plasma cholestanol level from baseline.
  + Member has demonstrated an improvement or stabilization of signs and symptoms of CTX (e.g., bilateral cataracts, intractable diarrhea, progressive neurological signs and symptoms, tendon xanthomas).

## References

1. Ctexli [package insert]. Foster City, CA: Mirum Pharmaceuticals Inc.; February 2025.
2. Merative Micromedex® (electronic version). Ann Arbor, MI. Available at: https://www.micromedexsolutions.com/ (cited: March 1, 2025).
3. Salen G, Steiner RD. Epidemiology, diagnosis, and treatment of cerebrotendinous xanthomatosis (CTX). J Inherit Metab Dis. 2017;40(6):771-781. doi:10.1007/s10545-017-0093-8
4. Nie S, Chen G, Cao X, et al. Cerebrotendinous xanthomatosis: a comprehensive review of pathogenesis, clinical manifestations, diagnosis, and management. Orphanet J Rare Dis 2014;9;179. doi.org/10.1186/s13023-014-0179-4