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 Indications[1]

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 Xanthomatosis[1-4]

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