

PHARMACY POLICY STATEMENT

North Carolina Marketplace

DRUG NAME	Ctexli (chenodiol)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Ctexli, approved by the FDA in 2025, is a bile acid indicated for treatment of adults with cerebrotendinous xanthomatosis (CTX), a neurodegenerative lipid storage disorder and bile acid synthesis disorder.

CTX is caused by pathogenic variants in the *CYP27A1* gene which lead to deficiency of the enzyme sterol 27-hydroxylase, preventing cholesterol from converting to bile acids, particularly depleting chenodeoxycholic acid (chenodiol, CDCA). This causes abnormal deposition of cholesterol and cholestanol in various tissues, and increased excretion of bile alcohols in urine. Symptoms are variable and age-dependent and include diarrhea, cataracts, xanthomas, and neurologic dysfunction.

Ctexli replaces deficient levels of the endogenous CDCA bile acid. This results in reductions of plasma cholestanol and urine 23S-pentol concentrations to slow CTX progression. Exogenous CDCA is the first-line treatment and is given lifelong.

Chenodiol was originally approved as brand name Chenodal in 2009 for the treatment of gallbladder stones and has been used off-label for CTX.

Ctexli (chenodiol) will be considered for coverage when the following criteria are met:

Cerebrotendinous Xanthomatosis (CTX)

For **initial** authorization:

1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a neurologist or metabolic specialist; AND
3. Member has a documented diagnosis of CTX confirmed by genetic test results that show mutation of the *CYP27A1* gene; AND
4. Biochemical test results show elevated plasma cholestanol at baseline; AND
5. Baseline liver transaminase and total bilirubin levels have been or will be obtained before starting.
6. **Dosage allowed/Quantity limit:** 250 mg orally three times daily. QL: 90 tablets per 30 days

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:

1. Chart notes must show positive clinical response to therapy such as reduced plasma cholestanol or urine 23S-pentol levels, or symptom improvement from baseline.

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Ctexli (chenodiol) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
03/17/2025	New policy for Ctexli created.

References:

1. Ctexli [prescribing information]. Mirum Pharmaceuticals, Inc.; 2025.
2. Stelten BML, Dotti MT, Verrips A, et al. Expert opinion on diagnosing, treating and managing patients with cerebrotendinous xanthomatosis (CTX): a modified Delphi study. *Orphanet J Rare Dis*. 2021;16(1):353. Published 2021 Aug 6. doi:10.1186/s13023-021-01980-5
3. Federico A, Gallus GN. Cerebrotendinous Xanthomatosis. 2003 Jul 16 [Updated 2024 Nov 14]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1409/>
4. Patni N, Wilson DP. Cerebrotendinous Xanthomatosis. [Updated 2023 Mar 8]. In: Feingold KR, Ahmed SF, Anawalt B, et al., editors. Endotext [Internet]. South Dartmouth (MA): MDText.com, Inc.; 2000-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK395578/>
5. Carson BE, De Jesus O. Cerebrotendinous Xanthomatosis. [Updated 2023 Aug 23]. In: StatPearls [Internet]. Treasure Island (FL): StatPearls Publishing; 2025 Jan-. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK564330/>

Effective date: 10/01/2025

Revised date: 03/17/2025