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Section:	Prescription Drugs	**Effective Date:**	July 1, 2025
Subsection:	Gastrointestinal Agent	**Original Policy Date:**	April 4, 2025
Subject:	Ctexli	**Page:**	1 of 4

Last Review Date: June 12, 2025

Ctexli

Description

Ctexli (chenodiol)

Background
Endogenous chenodiol (chenodeoxycholic acid) is a primary bile acid, synthesized from cholesterol in the liver. In cerebrotendinous xanthomatosis (CTX), the major bile acid synthesis pathways are disrupted due to partial or total deficiency in sterol 27-hydroxylase encoded by the CYP27A1 gene. Ctexli (chenodiol) may act to replace deficient levels of endogenous bile acid chenodeoxycholic acid in patients with CTX. Increased chenodiol levels in enterohepatic bile acid pool restore the activation of farnesoid X receptor (FXR) and downregulate CYP7A1 leading to suppression of reduction of atypical bile acids and bile alcohols including cholestanol and 23S-pentol (1).

Regulatory Status
FDA-approved indication: Ctexli is a bile acid indicated for treatment of cerebrotendinous xanthomatosis (CTX) in adults (1).

Ctexli has been associated with hepatotoxicity. Prior to initiation, obtain baseline liver transaminase and total bilirubin levels and monitor yearly and as clinically indicated. If liver transaminase levels are elevated > 3 times ULN or total bilirubin level is >2 times ULN, interrupt treatment until levels have returned to baseline values. For persistent or recurrent liver test abnormalities, consider discontinuing Ctexli (1).

The safety and effectiveness of Ctexli in patients less than 18 years of age have not been established (1).

Related policies
Chenodal

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Ctexli may be considered **medically necessary** if the conditions indicated below are met.

Ctexli may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

Cerebrotendinous xanthomatosis (CTX)

AND ALL of the following:

1. Diagnosis has been confirmed through genetic testing documenting pathogenic variants in the CYP27A1 gene
2. Patient has elevated pretreatment plasma cholestanol level and elevated levels of bile alcohol (i.e., 23s-pentol) in urine
3. Baseline ALT, AST, and bilirubin levels will be obtained and prescriber agrees to monitor annually as clinically indicated
4. Prescribed by or recommended by a prescriber who specializes in treatment of CTX

Prior–Approval *Renewal* Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

Cerebrotendinous xanthomatosis (CTX)

AND ALL of the following:

1. Patient has achieved or maintained a positive clinical response to therapy (e.g., decreased or stabilized level of bile alcohol, reduction in signs and symptoms of CTX, or reduction in plasma cholestanol level)
2. Prescriber agrees to monitor ALT, AST, and bilirubin levels annually as clinically indicated

Policy Guidelines

Pre–PA Allowance

None

Prior–Approval Limits

Quantity 270 tablets per 90 days

Duration 12 months

Prior–Approval *Renewal* Limits

Same as above

Rationale

Summary

Ctexli (chenodiol) is a bile acid indicated for the treatment of cerebrotendinous xanthomatosis (CTX). Ctexli may cause hepatotoxicity. Liver transaminase and total bilirubin levels should be monitored. The safety and effectiveness of Ctexli in pediatric patients have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Ctexli while maintaining optimal therapeutic outcomes.

References

1. Ctexli [package insert]. Foster City, CA. Mirum Pharmaceuticals, Inc.; February 2025.

Policy History

Date	Action
April 2025	Addition to PA
June 2025	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 12, 2025 and is effective on July 1, 2025.