



## 5.50.042

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|--------------------|------------------------|------------------------------|---------------|
| <b>Section:</b>    | Prescription Drugs     | <b>Effective Date:</b>       | April 1, 2026 |
| <b>Subsection:</b> | Gastrointestinal Agent | <b>Original Policy Date:</b> | April 4, 2025 |
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**Last Review Date:** March 6, 2026

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## Ctexli

### Description

#### Ctexli (chenodiol)

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#### 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).

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

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## 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

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## Prior-Approval *Renewal* Requirements

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**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

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### 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).

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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.; December 2025.

## Policy History

| Date       | Action                             |
|------------|------------------------------------|
| April 2025 | Addition to PA                     |
| June 2025  | Annual review                      |
| March 2026 | Annual review and reference update |

## Keywords

**This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 6, 2026 and is effective on April 1, 2026.**