Effective Date: 02/01/2022
Reviewed: 11/2021, 07/2023, 6/2024, 6/2025, 4/2026 Scope: Medicaid

CHENODIOL PRODUCTS: CHENODAL and CTEXLI

POLICY

I. 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 Indication
Chenodal (chenodiol) is indicated for patients with radiolucent stones in well-opacifying gallbladders, in whom selective surgery would be undertaken except for the presence of increased surgical risk due to systemic disease or age. The likelihood of successful dissolution is far greater if the stones are floatable or small. For patients with nonfloatable stones, dissolution is less likely and added weight should be given to the risk that more emergent surgery might result from a delay due to unsuccessful treatment. Safety of use beyond 24 months is not established. Chenodal will not dissolve calcified (radiopaque) or radiolucent bile pigment stones.

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

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

II. CRITERIA FOR INITIAL APPROVAL

Radiolucent stones in well-opacifying gallbladders
[Note: Chenodal will not dissolve calcified (radiopaque) or radiolucent bile pigment stones.]

Authorization of 6 months may be granted for treatment of members with radiolucent stones in well-opacifying gallbladders when all of the following criteria are met:

A. Request is for Chenodal
B. Member is 18 years of age or older.
C. Medication is prescribed by, or in consultation with, a gastroenterologist
D. Documentation that the member has an increased surgical risk due to systemic disease or age.
E. Documentation that the member experienced an inadequate treatment response after a 6-month trial or intolerance to ursodiol.
F. Documentation that the member will not exceed a dose of 16 mg/kg/day. Member's current weight and prescribed dose must be provided.
G. Documentation that the member has not received more than 24 months of therapy with Chenodal.

Cerebrotendinous Xanthomatosis (CTX)
Authorization of 6 months may be granted for treatment of members with CTX when all of the following criteria are met:
A. Member is 18 years of age or older.
B. Medication is prescribed by, or in consultation with a physician who is experienced in the treatment of CTX (e.g., neurologist. geneticist, endocrinologist, gastroenterologist)
C. Documentation of confirmed diagnosis of CTX by genetic testing indicating pathogenic variants in the CYP27A1 gene.
D. Documentation that the member has an elevated pretreatment plasma cholestanol level.
E. Documentation that the member has elevated levels of bile alcohol (i.e., 23s-pentol) in the urine.
F. Documentation that the member has signs and symptoms of CTX (e.g., bilateral cataracts, intractable diarrhea, progressive neurological signs and symptoms, tendon xanthomas).
G. Documentation that the 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).
H. Documentation that the member has a baseline bilirubin level of less than or equal to 2 times the upper limit of normal (ULN).
I. Documentation that the member has been assessed for malabsorption disorder or other confounding gastrointestinal conditions.
J. The requested medication will not be used in combination with bile acid sequestering agents (e.g., cholestyramine, colestipol, aluminum-based antacid) or Cholbam.
K. Dosing will not exceed 250 mg three times daily (three 250 mg tablets per day).

III. CONTINUATION OF THERAPY

Radiolucent stones in well-opacifying gallbladders
Authorization of 6 months may be granted for continued treatment in members requesting reauthorization when all of the following criteria are met:
A. Documentation that the member has experienced partial (or complete) dissolution of stones, or patient has not experienced a partial dissolution and provider will discontinue therapy with the requested drug if response is not seen by 18 months of treatment.
B. There is no evidence of unacceptable toxicity.
C. Documentation that the member will not exceed a dose of 16 mg/kg/day. Member's current weight and prescribed dose must be provided.
D. Documentation that the member has not received more than 24 months of therapy with Chenodal.

Cerebrotendinous Xanthomatosis
Authorization of 6 months may be granted for continued treatment in members requesting reauthorization when all of the following criteria are met:
A. Member has not experienced signs and symptoms of hepatotoxicity (e.g., abdominal pain, bruising, dark-colored urine, jaundice).
B. Documentation that the member has a confirmed liver transaminase (i.e., ALT, AST) level of less than or equal to 3 times the upper limit of normal.
C. Documentation that the member has a confirmed bilirubin level of less than or equal to 2 times the upper limit of normal.
D. The requested medication will not be used in combination with bile acid sequestering agents (e.g., cholestyramine, colestipol, aluminum-based antacid) or Cholbam.

E. Documentation that the member has achieved or maintained a positive clinical response as evidenced by any of the following:
    i. Decreased or stabilized level of bile alcohol (23s-pentol) in the urine
    ii. Reduction in plasma cholestanol level from baseline
    iii. Improvement or stabilization of signs and symptoms of CTX (e.g., bilateral cataracts, intractable diarrhea, progressive neurological signs and symptoms, tendon xanthomas).
F. Dosing will not exceed 250 mg three times daily (three 250 mg tablets per day).

IV. REFERENCES

1. Chenodal [package insert]. San Diego, CA: Retrophin, Inc.; December 2025. Accessed March 2026.
2. Ctexli [package insert]. Foster City, CA: Mirum Pharmaceuticals Inc.; December 2025. Accessed: March 2026.
3. Micromedex® (electronic version). IBM Watson Health, Greenwood Village, Colorado, USA. Available at: https://www.micromedexsolutions.com/ (cited: November 23, 2020).
4. Ursodiol [package insert]. Irvine, CA: Nexgen Pharma, Inc.; August 2020.