BlueCross BlueShield
Federal Employee Program

CTEXLI
(chenodiol)

RATIONALE FOR INCLUSION IN PA PROGRAM

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).

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.

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References

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