BlueCross BlueShield
Federal Employee Program

BYLVAY
(odevixibat)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Bylvay (odevixibat) is an inhibitor of the ileal bile acid transporter (IBAT). IBAT is almost completely responsible for the reabsorption of bile acid from the ileum, returning biliary products to systemic circulation. Inhibition of this process promotes elimination of bile acid and reduces pruritus associated with cholestatic disease (1).

Regulatory Status

FDA-approved indication: Bylvay is an ileal bile acid transporter (IBAT) inhibitor indicated for: (1)

- Progressive Familial Intrahepatic Cholestasis (PFIC)
    - the treatment of pruritus in patients 3 months of age and older with progressive familial intrahepatic cholestasis (PFIC).
    - Limitation of Use: Bylvay may not be effective in a subgroup of PFIC type 2 patients with ABCB11 variants resulting in non-functional or complete absence of the bile salt export pump protein.
- Alagille Syndrome (ALGS)
    - the treatment of cholestatic pruritus in patients 12 months of age and older with Alagille syndrome (ALGS).

Patients with PFIC and ALGS may have impaired hepatic function at baseline. The efficacy and safety in PFIC and ALGS patients with clinically significant portal hypertension, and in patients with decompensated cirrhosis have not been established (1).

Bylvay has warnings regarding the following: diarrhea, liver test abnormalities, and Fat-Soluble Vitamin (FSV) deficiency. Patients should obtain baseline levels of liver function and fat-soluble vitamins and be monitored for abnormalities in liver function and for FSV deficiency throughout treatment (1).

The Rare Disease Database includes diagnostic criteria for Alagille syndrome, including characteristic symptoms, bile duct paucity, and genetic testing (2).

The safety and effectiveness of Bylvay in pediatric patients less than 3 months of age with PFIC

have not been established. The safety and effectiveness of Bylvay in pediatric patients less than 12 months of age with ALGS have not been established (1).

Summary

Bylvay is an ileal bile acid transport (IBAT) inhibitor indicated for the treatment of pruritus associated with progressive familial intrahepatic cholestasis (PFIC) or cholestatic pruritus associated with Alagille syndrome (ALGS). Bylvay may not be effective in PFIC type 2 patients with ABCB11 variants resulting in absence or non-function of bile salt export pump protein. Current warnings include diarrhea, liver test abnormalities, and fat-soluble vitamin deficiency. Bylvay was not evaluated in patients with cirrhosis and treatment should be discontinued permanently if patient progresses to portal hypertension or has a hepatic decompensation event (1).

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

References

1. Bylvay [package insert]. Cambridge, MA: Ipsen Biopharmaceuticals, Inc.; February 2024.
2. National Organization for Rare Disorders (NORD). Alagille syndrome. Rare Disease Database. https://rarediseases.org. Published 2023.