BlueCross BlueShield
Federal Employee Program

LIVMARLI
(maralixibat)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Livmarli (maralixibat) 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 indications: Livmarli is an ileal bile acid transporter (IBAT) inhibitor indicated for: (1)

- the treatment of cholestatic pruritus in patients 3 months of age and older with Alagille syndrome (ALGS).
- the treatment of cholestatic pruritus in patients 12 months of age and older with progressive familial intrahepatic cholestasis (PFIC).
    - Limitations of Use: Livmarli is not recommended in a subgroup of PFIC type 2 patients with specific ABCB11 variants resulting in non-functional or complete absence of bile salt export pump (BSEP) protein.

Livmarli treatment is associated with a potential for drug-induced liver injury. Liver tests should be obtained at baseline and monitored during treatment. Livmarli is contraindicated in patients with prior or active hepatic decompensation events (1).

Livmarli also has warnings regarding gastrointestinal adverse reactions and fat-soluble vitamin (FSV) deficiency. Patients should obtain baseline levels of fat-soluble vitamins and be monitored for FSV deficiency throughout treatment (1).

Livmarli may increase the risk of propylene glycol toxicity in pediatric patients less than 5 years of age. The total daily intake of propylene glycol should be considered for managing the risk of propylene glycol toxicity. Patients should be monitored for signs of potential propylene glycol toxicity, including hemolysis, hyperosmolarity with anion gap metabolic acidosis, acute kidney injury, and CNS toxicity. Discontinue Livmarli if toxicity is suspected (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 Livmarli in patients less than 3 months of age with ALGS have not been established. The safety and effectiveness of Livmarli in pediatric patients less than 12 months of age with PFIC have not been established (1).

Summary

Livmarli is an ileal bile acid transport (IBAT) inhibitor indicated for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) or progressive familial intrahepatic cholestasis (PFIC). Current warnings include gastrointestinal adverse reactions, hepatotoxicity, and fat-soluble vitamin deficiency. Livmarli is contraindicated in patients with prior or active hepatic decompensation events. The safety and effectiveness of Livmarli in patients less than 3 months of age with ALGS have not been established. The safety and effectiveness of Livmarli in pediatric patients less than 12 months of age with PFIC have not been established (1).

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

References

1. Livmarli [package insert]. Foster City, CA: Mirum Pharmaceuticals, Inc.; April 2025.
2. National Organization for Rare Disorders (NORD). Alagille syndrome. Rare Disease Database. https://rarediseases.org. Published 2020.