

Protocol for Livmarli® (maralixibat)

Approved April 2023

Background: *Alagille syndrome (ALGS) is an inherited multisystem disorder typically manifesting as cholestasis, and potentially leading to end-stage liver disease and death.*

Livmarli is an ileal bile acid transporter (IBAT) inhibitor indicated for the treatment of cholestatic pruritus in patients with Alagille syndrome

Criteria for approval:

1. Diagnosis of Alagille syndrome
2. Patient is 3 months of age or older
3. Patient has symptoms of moderate to severe pruritus
4. Patient has cholestasis, as indicated by at least ONE of the following:
 - a. Total serum bile acid >3x the upper limit of normal (ULN) for age
 - b. Conjugated bilirubin >1 mg/dL
 - c. Fat soluble vitamin deficiency that is otherwise unexplainable
 - d. Gamma Glutamyl Transferase (GGT) >3 × ULN for age
 - e. Intractable pruritus explainable only by liver disease
5. Medication is prescribed by or in consultation with a hepatologist, gastroenterologist, or other specialist with experience in the treatment of the disease
6. Patient has no history of liver transplant or clinical evidence of decompensated cirrhosis
7. Patient has had inadequate response to at least one medication used to treat pruritus (e.g., Ursodiol [ursodeoxycholic acid], cholestyramine, rifampin, naltrexone) unless all are contraindicated
8. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Initial Approval: 6 months

Continuation of therapy:

1. Documentation of positive clinical response to therapy (e.g., reduced pruritus, reduced serum bile acids from baseline) in chart notes or medical records
2. Medication is prescribed by or in consultation with a hepatologist, gastroenterologist, or other specialist in the treated disease state

Renewal Approval: 12 months

References

1. Livmarli [prescribing information]. Mirum Pharmaceuticals Inc. Foster City, CA. September 2021
2. Kamath BM, et al. Systematic review: The epidemiology, natural history, and burden of Alagille syndrome. *J Pediatr Gastroenterol Nutr.* 2018; 67:148-156.
3. *Clinical Pharmacology® Gold Standard Series* [Internet database]. Tampa FL. Elsevier 2019. Updated periodically