

Clinical Policy Title:	maralixibat
Policy Number:	RxA.709
Drug(s) Applied:	Livmarli®
Original Policy Date:	12/07/2021
Last Review Date:	12/11/2025
Line of Business Policy Applies to:	All lines of business (except Medicare)

Criteria

I. Initial Approval Criteria

A. Cholestatic pruritus associated with Alagille syndrome (Tablets, 9.5 mg/mL solution) (must meet all):

1. Diagnosis of cholestatic pruritus in patients with Alagille syndrome;
2. Diagnosis confirmed by presence of the JAG1 or Notch2 gene mutation;
3. Symptoms of moderate to severe pruritus;
4. Cholestasis, as indicated by at least one (1) of the following:
 - a. Total serum bile acid > 3 times 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 times ULN for age;
 - e. Intractable pruritus explainable only by liver disease;
5. Trial and failure of at least two (2) of the following medications used to treat pruritus, unless contraindicated or clinically significant adverse effects are experienced:
 - a. Ursodiol (ursodeoxycholic acid);
 - b. Bile acid sequestrants (e.g., Questran, Colestid, Welchol, cholestyramine);
 - c. Rifampin.

B. Cholestatic pruritus associated with progressive familial intrahepatic cholestasis (Tablets, 19 mg/mL solution) (must meet all):

1. Diagnosis of cholestatic pruritus in patients with progressive familial intrahepatic cholestasis;
2. Diagnosis confirmed by presence of ATP8B1, ABCB4, TJP2, NR1H4, or MYO5B mutation;
3. Symptoms of moderate to severe pruritus;
4. Trial and failure of one of the following treatments for pruritis (a, b, c, or d):
 - a. Ursodeoxycholic acid;
 - b. Antihistamines;
 - c. Rifampin;
 - d. Bile acid sequestrant.

Approval Duration

All Lines of Business (except Medicare): 12 months

II. Continued Therapy Approval

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.

A. All Indications (must meet all):

1. Member is currently receiving medication in the past 120 days that has been authorized by RxAdvance or the member has met the initial approval criteria.

Approval Duration

All Lines of Business (except Medicare): 12 months

References

1. Mirum Pharmaceuticals, Inc. Long-Term, Open-Label Study with a Double-Blind, Placebo-Controlled, Randomized Drug Withdrawal Period of Lum001 (Maralixibat), an Apical Sodium-Dependent Bile Acid Transporter Inhibitor (Asbti), in Patients with Alagille Syndrome. [clinicaltrials.gov](https://clinicaltrials.gov/ct2/show/NCT02160782); 2021. Available at: <https://clinicaltrials.gov/ct2/show/NCT02160782>. Accessed March 24, 2025.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	10/19/2021	12/07/2021
Policy was reviewed: <ol style="list-style-type: none"> 1. Initial Approval Criteria, I.A.1: Updated diagnostic criteria from Diagnosis of cholestatic pruritus in patients with Alagille syndrome (ALGS) to Diagnosis of ALGS-associated pruritus with molecular genetic testing confirmed mutations in the JAG1 or NOTCH2 gene. 2. Initial Approval Criteria, I.A.6: Updated to remove prior disease criteria "Patient does not have chronic diarrhea requiring ongoing intravenous fluid or nutritional intervention". 3. Initial Approval Criteria, I.A.7: Updated to remove prior surgery criteria "No history of surgical interruption of enterohepatic circulation (for example, partial external biliary diversion [PEBD] surgery)". 4. Initial Approval Criteria, I.A.8: Updated to remove prior disease criteria "No clinical evidence of decompensated cirrhosis". 5. Initial Approval Criteria, I.A.6.b: Updated trial and failure criteria: Cholestyramine to Bile acid sequestrants (e.g., 	9/6/2022	10/19/2022

<p>Questran, Colestid, Welchol, cholestyramine).</p> <p>6. Initial Approval Criteria, I.A.6.d: Updated to remove prior trial and failure criteria "Naltrexone".</p> <p>7. Initial Approval Criteria, I.A.6.e: Updated to remove prior trial and failure criteria "Sertraline".</p> <p>8. Initial Approval Criteria, I.A.6.d: Updated to include new trial and failure criteria Antihistamines (e.g., diphenhydramine, hydroxyzine).</p> <p>9. Initial Approval Criteria, I.A.7: Updated to include new diagnostic criteria Documentation of member's current weight in kilograms.</p> <p>10. Initial Approval Criteria, I.A: Updated approval duration criteria from 6 months to 12 months.</p> <p>11. Continued Therapy Approval Criteria, II.A.3: Updated to include new diagnostic criteria Documentation of member's current weight in kilograms.</p> <p>12. References were reviewed and updated.</p>		
<p>Policy was reviewed:</p> <p>1. Initial Approval Criteria, I.A.1: Updated diagnostic criteria from Diagnosis of ALGS-associated pruritis with molecular genetic testing confirmed mutations in the JAG1 or NOTCH2 gene to Diagnosis of ALGS-associated pruritis confirmed by presence of the JAG1 or Notch2 gene mutation.</p> <p>2. Initial Approval Criteria, I.A.3: Updated age criteria from Age ≥ 1 years to Age ≥ 3 months and ≤ 18 years at therapy</p>	<p>06/05/2023</p>	<p>07/13/2023</p>

<p>initiation.</p> <p>3. Initial Approval Criteria, I.A.4: Updated diagnostic criteria from Symptoms of moderate to very severe pruritus to Symptoms of moderate to severe pruritus.</p> <p>4. Initial Approval Criteria, I.A.6.d: Updated trial and failure criteria to remove “Antihistamines (e.g., diphenhydramine, hydroxyzine)”.</p> <p>5. Continued Therapy Approval Criteria, II.A.2: Updated diagnostic criteria from Member is responding positively to therapy; (eg tolerating therapy and documentation of improvement in pruritis); to Member is responding positively to therapy</p> <p>6. Continued Therapy Approval Criteria, II.A.3: Updated to remove prior documentation criteria "Documentation of member’s current weight in kilograms"</p> <p>7. References were reviewed and updated.</p>		
<p>Policy was reviewed.</p>	<p>10/19/2023</p>	<p>10/19/2023</p>
<p>Policy was reviewed:</p> <ol style="list-style-type: none"> 1. Removed prescriber criteria. 2. Removed dosing criteria. 3. Updated approval duration verbiage. 4. Updated continuation of therapy language. 5. Removed reauthorization requirement for positive response to therapy. 	<p>03/24/2025</p>	<p>04/10/2025</p>
<p>Policy was reviewed:</p> <ol style="list-style-type: none"> 1. Specified what strength solution goes with which indication. 	<p>5/22/2025</p>	<p>5/22/2025</p>

2. Removed prescriber criteria.		
Policy reviewed.	12/11/2025	12/11/2025