Pharmacy Medical Necessity Guidelines:
Livmarli (maralixibat) solution

Effective: August 11, 2026

Prior Authorization Required	√	Type of Review – Care Management	
Not Covered		Type of Review – Clinical Review	√
Pharmacy (RX) or Medical (MED) Benefit	RX	Department to Review	RXUM
These pharmacy medical necessity guidelines apply to the following: ☒ Tufts Health RITogether – A Rhode Island Medicaid Plan		**Fax Numbers:** RXUM: 617.673.0939	

Note: This guideline does not apply to Medicare Members (includes dual eligible Members).

OVERVIEW

FOOD AND DRUG ADMINISTRATION-APPROVED INDICATIONS

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

Alagille Syndrome (ALGS)
- The treatment of cholestatic pruritus in patients with ALGS 3 months of age and older.

Progressive Familial Intrahepatic Cholestasis (PFIC)
- The treatment of cholestatic pruritus in patients 12 months of age and older with PFIC. 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.

COVERAGE GUIDELINES

The plan may authorize coverage of Livmarli solution for Members when **ALL** of the following criteria are met:

Alagille Syndrome

Initial Authorization Criteria

1. Documented diagnosis of Alagille syndrome confirmed by genetic testing demonstrating the presence of a jagged canonical notch ligand 1 (JAG1) or notch receptor 2 (NOTCH2) mutation

AND

2. Documentation of cholestasis as defined by at least **one (1)** of the following:
    a. Total serum bile acid greater than 3 times the upper limit of normal for age
    b. Conjugated bilirubin greater than 1 mg/dL
    c. Fat soluble vitamin deficiency that is otherwise unexplainable
    d. Gamma Glutamyl Transferase (GCT) greater than 3 times the upper limit for age
    e. Intractable pruritus explainable only by liver disease

AND

3. Prescribed by or in consultation with a hepatologist, gastroenterologist, or a provider who specializes in Alagille syndrome (ALGS)

AND

4. Patient is 3 months of age or older

AND

5. Documentation of moderate to severe pruritus

AND

6. Documentation of **one (1)** of the following:
    a. Inadequate response to at least two (2) other medications to treat pruritus (e.g., ursodeoxycholic acid, antihistamines, rifampin, naltrexone, bile acid sequestrants)
    b. Clinical inappropriateness for use of other medications to treat pruritus

Reauthorization Criteria

1. Documented diagnosis of Alagille syndrome confirmed by genetic testing demonstrating the presence of a jagged canonical notch ligand 1 (JAG1) or notch receptor 2 (NOTCH2) mutation

AND

2. Prescribed by or in consultation with a hepatologist, gastroenterologist, or a provider who specializes in Alagille syndrome (ALGS)

AND

3. Patient is 3 months of age or older

AND

4. Documentation of a positive clinical response as evidenced by one of the following:

a. Improvement in severity of pruritus
b. Reduction in serum bile acid from baseline

AND

5. Documentation the Member has not had a liver transplantation

Progressive Familial Intrahepatic Cholestasis

Initial Authorization Criteria

1. Documented diagnosis of progressive familial intrahepatic cholestasis

AND

2. Documentation of molecular genetic testing confirming both of the following:
   a. Diagnosis of progressive familial intrahepatic cholestasis
   b. No indication of progressive familial intrahepatic cholestasis type 2 with ABCB11 variants encoding for nonfunction or absence of bile salt export pump protein (BSEP-3)

AND

3. Prescribed by or in consultation with a hepatologist, gastroenterologist, or a provider who specializes in progressive familial intrahepatic cholestasis

AND

4. The patient is 12 months of age or older

AND

5. Documentation of moderate to severe pruritus

AND

6. Documentation of trial and failure with at least **one (1)** systemic medication considered standard of care for progressive familial intrahepatic cholestasis, unless contraindicated (e.g., antihistamines, cholestyramine, rifampicin, ursodiol)

AND

7. Documentation the patient has not had a liver transplant

Reauthorization Criteria

1. Documented diagnosis of progressive familial intrahepatic cholestasis

AND

2. Documentation of molecular genetic testing confirming both of the following:
   a. Diagnosis of progressive familial intrahepatic cholestasis
   b. No indication of progressive familial intrahepatic cholestasis type 2 with ABCB11 variants encoding for nonfunction or absence of bile salt export pump protein (BSEP-3)

AND

3. Prescribed by or in consultation with a hepatologist, gastroenterologist, or a provider who specializes in progressive familial intrahepatic cholestasis

AND

4. The patient is 12 months of age or older

AND

5. Documentation of a positive clinical response as evidenced by **one (1)** of the following:
   a. Improvement in severity of pruritus
   b. Reduction in serum bile acid from baseline

AND

6. Documentation the Member has not had a liver transplantation

LIMITATIONS

- Initial coverage of Livmarli (maralixibat) solution will be authorized for 6 months. Reauthorization of Livmarli (maralixibat) solution will be provided in 12-month intervals.
- Patients new to the plan stable on Livmarli (maralixibat) solution should be reviewed against Reauthorization Criteria.
- Livmarli (maralixibat) tablets are non-formulary

CODES

None

REFERENCES

1. Ayoub MB, et al. Alagille syndrome: diagnostic challenges and advances in management. Diagnostics (Basel). 2020;10(11):907.
2. Gonzales E, Sturm E, Stormon M et al. Durability of treatment effect with long-term maralixibat in children with Alagille syndrome: 4-year safety and efficacy results from the ICONIC study.

Hepatology. 2019b;70(6): 1477A-1501A. 70th Annual Meeting of the American Association for the Study of Liver Diseases abstract LO3.
3. Gonzales E, Sturm E, Stormon M et al. Phase 2 open-label study with a placebo-controlled drug withdrawal period of the apical sodium-dependent bile acid transporter inhibitor maralixibat in children with Alagille syndrome: 48-week interim efficacy analysis. *J Hepatol*. 2019a; 70(suppl 1):e119-e120. The International Liver Congress 2019 abstract PS-193.
4. Livmarli (maralixibat) [prescribing information]. Forest City, CA: Mirum Pharmaceuticals, Inc; April 2025.
5. Mitchell E, Gilbert M, Loomes KM. Alagille syndrome. *Clin Liver Dis*. 2018;22(4):625-641.
6. Schwartz R, Rehder K, Parsons DJ, Morrell DS. Intense pruritus and failure to thrive in Alagille syndrome. *J Am Acad Dermatol*. 2008;58(2 Suppl):S9-11.
7. Jacquemin E. Progressive familial intrahepatic cholestasis. Clin Res Hepatol Gastroenterol. 2012;36 Suppl 1:S26-S35.

APPROVAL HISTORY

September 13, 2022: Reviewed by Pharmacy & Therapeutics Committee.

- May 9, 2023: Expanded age requirements to 3 months of age or older (effective July 1, 2023)
- February 13, 2024: Added Documentation of one (1) of the following: Inadequate response to at least two (2) other medications to treat pruritus (e.g., ursodeoxycholic acid, antihistamines, rifampin, naltrexone, bile acid sequestrants) or Clinical inappropriateness for use of other medications to treat pruritus. Updated diagnosis requirements to include genetic testing confirming the diagnosis. Added the requirement of moderate to severe pruritus (effective May 1, 2024).
- October 8, 2024: Retire Medical Necessity Guideline. Coverage is moved to non-formulary status effective 1/1/25 (eff 12/31/2024)
- November 12, 2024: Un-retire Medical Necessity Guideline based on 2025 coverage (eff 1/1/25).
- February 11, 2025: Added coverage criteria for the supplemental indication in PFIC (eff 3/1/25).
- August 12, 2025: Updated Medical Necessity Guideline to clarify that coverage applies to Livmarli solution. Livmarli tablets are non-formulary (eff 9/1/25).
- August 11, 2026: No changes.

BACKGROUND, PRODUCT AND DISCLAIMER INFORMATION

Pharmacy Medical Necessity Guidelines have been developed for determining coverage for plan benefits and are published to provide a better understanding of the basis upon which coverage decisions are made. The plan makes coverage decisions on a case-by-case basis considering the individual member's health care needs. Pharmacy Medical Necessity Guidelines are developed for selected therapeutic classes or drugs found to be safe, but proven to be effective in a limited, defined population of patients or clinical circumstances. They include concise clinical coverage criteria based on current literature review, consultation with practicing physicians in the service area who are medical experts in the particular field, FDA and other government agency policies, and standards adopted by national accreditation organizations. The plan revises and updates Pharmacy Medical Necessity Guidelines annually, or more frequently if new evidence becomes available that suggests needed revisions.

For self-insured plans, coverage may vary depending on the terms of the benefit document. If a discrepancy exists between a Pharmacy Medical Necessity Guideline and a self-insured Member's benefit document, the provisions of the benefit document will govern.

Treating providers are solely responsible for the medical advice and treatment of members. The use of this policy is not a guarantee of payment or a final prediction of how specific claim(s) will be adjudicated. Claims payment is subject to member eligibility and benefits on the date of service, coordination of benefits, referral/authorization and utilization management guidelines when applicable, and adherence to plan policies and procedures and claims editing logic.

Provider Services