



REAL PATIENT STORIES

Explore the stories of real patients who were prescribed LIVMARLI to treat cholestatic pruritus in Alagille syndrome. See how LIVMARLI made a real difference. Mirum is thankful to the patients and their families for sharing their experiences.

Note: Individual results may vary. The profiles shown here reflect the experiences of actual patients treated with LIVMARLI. Not every patient responds to LIVMARLI; those who do respond may respond differently based on their unique characteristics.

INDICATION

LIVMARLI is indicated for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) 3 months of age and older.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

Liver Test Abnormalities: Patients enrolled in clinical trials had abnormal liver tests at baseline. In the main clinical trial, treatment-emergent elevations or worsening of liver tests (ALT, AST or T/DB) relative to baseline were observed. Obtain baseline liver tests and monitor during treatment. Dose reduction or treatment interruption may be considered if abnormalities occur in the absence of other causes. For persistent or recurrent liver test abnormalities, consider treatment discontinuation. Discontinue permanently if a patient progresses to portal hypertension or experiences a hepatic decompensation event.

Please see Important Safety Information throughout and full [Prescribing Information](#) for LIVMARLI.





3 YEARS OLD

Meet Sammy

Struggled with intermittent itching

Taking LIVMARLI since 2021

GENDER

Male

FAVORITE ACTIVITIES

Visiting parks, trails, and museums

FAVORITE TOYS

All things musical

**“LIVMARLI really helped with Sammy’s
cholestatic pruritus.”**

–Heidi, Sammy’s mom



IMPORTANT SAFETY INFORMATION (cont’d)

WARNINGS AND PRECAUTIONS

GI Adverse Reactions: Diarrhea, abdominal pain and vomiting were reported as the most common adverse reactions. If diarrhea, abdominal pain and/or vomiting occur and no other etiologies are found, consider reducing the dose or interrupting LIVMARLI. For diarrhea or vomiting, monitor for dehydration and treat promptly. Consider interrupting LIVMARLI dosing if a patient experiences persistent diarrhea or has diarrhea with accompanying signs and symptoms such as bloody stool, vomiting, dehydration requiring treatment, or fever. Restart LIVMARLI at 190 mcg/kg/day when diarrhea, abdominal pain or vomiting resolve, and increase the dose as tolerated. If they recur upon re-challenge, consider stopping LIVMARLI treatment.

Please see Important Safety Information throughout and full [Prescribing Information](#) for LIVMARLI.

 **Livmarli**[®]
(maralixibat) oral solution

3 YEARS OLD

Meet Sammy (cont'd)



Medical History

- **Diagnosis:** Diagnosed with Alagille syndrome at 2 months old. Confirmatory genetic testing revealed a mutation in *JAG1*
- **Surgeries:** Liver biopsy
- **Current medications:** Ursodiol, rifampin, and LIVMARLI
 - Note: Hydroxyzine is used sparingly for intermittent itchiness
- **Other symptoms related to Alagille syndrome:** Pulmonary artery stenosis, jaundice



Life Before LIVMARLI

- **Sammy's cholestatic pruritus before LIVMARLI:** Itching with minor abrasions
 - Sammy was unable to verbalize the itch, but he would squirm, scratch, and dig in his ears
- **How Sammy's itch was managed:** Took ursodiol, rifampin, and hydroxyzine, but continued to itch
- **How the itch impacted Sammy:**
 - Few scratches and abrasions
 - Fussiness and anxiousness
 - Always wore onesies to avoid new scratches
 - Bathed infrequently to avoid overly dry skin/aggravating the itch



Life With LIVMARLI

- **Sammy's cholestatic pruritus since starting LIVMARLI:** Rubbing or mild scratching when undistracted
- **When Sammy saw improvements in itch:** Within 4 weeks
- **Living with less itch:**
 - Significantly less scratching
 - No new scratches
 - Less cranky and irritable
 - Now able to wear T-shirts, shorts, and other clothes since itching is less of a concern
- **Side effects:** Loose stools, changes in liver tests
 - Loose stools resolved after 6 months
 - Levels from liver function tests went up but returned to patient's baseline within 2 months

Stories reported by caregiver.
Individual results may vary.

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(maralixibat) oral solution



11 YEARS OLD

Meet Noelle

Lived with a chronic itch despite SOC treatments

Taking LIVMARLI since 2021

GENDER

Female

FAVORITE HOBBIES

Playing outside, making videos

**FAVORITE SUBJECT
IN SCHOOL**

Recess

“She’s now able to stay outside and continue playing without tearing up her skin.”

–Kim, Noelle’s mom



SOC=standard of care.

IMPORTANT SAFETY INFORMATION (cont’d)

WARNINGS AND PRECAUTIONS

Fat-Soluble Vitamin Deficiency: ALGS patients can have fat-soluble vitamin (FSV) deficiency (vitamins A, D, E, and K) at baseline, and LIVMARLI may affect absorption of FSV. In the main clinical trial, treatment emergent FSV deficiency was reported in 3 (10%) patients during 48 weeks of treatment. Obtain baseline serum levels and monitor during treatment, along with any clinical manifestations. Supplement if deficiency is observed. Consider discontinuing LIVMARLI if FSV deficiency persists or worsens despite adequate FSV supplementation.

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 **Livmarli**[®]
(maralixibat) oral solution

11 YEARS OLD

Meet Noelle (cont'd)



Medical History

- **Diagnosis:** Diagnosed with Alagille syndrome at 2½ months old. Confirmatory genetic testing revealed a mutation in *JAG1*
- **Surgeries:** None
- **Current medications:** Ursodiol, rifampin, and LIVMARLI
- **Other symptoms related to Alagille syndrome:** Jaundice, high bilirubin, facial characteristics, Tetralogy of Fallot with pulmonary valve atresia and major aortopulmonary collateral arteries, coarctation of the aorta, and pulmonary artery stenosis



Life Before LIVMARLI

- **Noelle's cholestatic pruritus before LIVMARLI:** Active scratching with abrasions
- **How Noelle's itch was managed:** Took ursodiol, rifampin, and hydroxyzine, but continued to itch
- **How the itch impacted Noelle:**
 - Learned to live with the itch and managed it with various coping mechanisms
 - Used brushes to scratch her skin and frequently asked her mother to scratch her back
 - Significant sleep disturbances
 - Patient's mother noted that patient would scratch all night



Life With LIVMARLI

- **Noelle's cholestatic pruritus since starting LIVMARLI:** Rubbing or mild scratching when undistracted
- **When Noelle saw improvements in itch:** Within 1 week after starting LIVMARLI; improvements confirmed at 3-week follow-up appointment; itch gradually improved thereafter
- **Living with less itch:**
 - Less scratching and fewer abrasions
 - Fewer sleep disturbances
 - Less impacted by changes in temperature and weather

Stories reported by caregiver.
Individual results may vary.

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 **Livmarli**[®]
(maralixibat) oral solution



19 YEARS OLD

Meet Emma

Suffered from debilitating itch throughout her childhood

Taking LIVMARLI since 2016

GENDER

Female

FAVORITE HOBBIES

Drawing, playing the ukulele, and taking care of her cats

FAVORITE SUBJECTS IN SCHOOL

English language arts, choir, and Spanish



“LIVMARLI gave her a second chance—a chance to live with less itching.”

—Julee, Emma’s mom

IMPORTANT SAFETY INFORMATION (cont’d)

ADVERSE REACTIONS

The most common adverse reactions ($\geq 5\%$) are diarrhea, abdominal pain, vomiting, fat-soluble vitamin deficiency, liver test abnormalities, gastrointestinal bleeding and bone fractures.

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 **Livmarli**[®]
(maralixibat) oral solution

19 YEARS OLD

Meet Emma (cont'd)



Medical History

- **Diagnosis:** Diagnosed with Alagille syndrome by 3 months old. Confirmatory genetic testing revealed a mutation in *JAG1*
- **Surgeries:** Balloon angioplasty
- **Current medications:** Ursodiol, vitamin D, and LIVMARLI
- **Other symptoms related to Alagille syndrome:** Failure to thrive



Life Before LIVMARLI

- **Emma's cholestatic pruritus before LIVMARLI:** Constant active scratching leading to cutaneous mutilation, hemorrhage, and scarring
- **How Emma's itch was managed:**
 - Ursodiol: Taken since birth
 - Rifampin: Taken for 1 year before being discontinued due to ineffectiveness
 - Cholestyramine: Taken for <6 months before being discontinued due to ineffectiveness
- **How the itch impacted Emma:**
 - Moody and irritable
 - Significant sleep disturbances
 - Patient's mother noted that patient would often rip holes in her clothing and bite at her skin



Life With LIVMARLI

- **Emma's cholestatic pruritus since starting LIVMARLI:** Occasional rubbing or mild scratching when undistracted
- **When Emma saw improvements in itch:** Within 2 weeks; improvements confirmed at 3-week follow-up appointment
- **Living with less itch:**
 - Improved skin appearance
 - Fewer abrasions and no more bleeding
 - Less moody and irritable
 - Fewer sleep disturbances

Stories reported by caregiver.
Individual results may vary.

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YOUR PATIENTS' OUTCOMES
START WITH
YOUR TREATMENT DECISION

Cholestatic pruritus in Alagille syndrome is related to elevated serum bile acid (sBA) levels and is considered among the worst of any cholestatic liver disease.¹⁻⁴

LIVMARLI is an ileal bile acid transporter (IBAT) inhibitor that aims to reduce toxic bile acid buildup in the body and help treat cholestatic pruritus in patients with Alagille syndrome who are ≥ 3 months of age.⁵⁻⁷

See how LIVMARLI can help
your patients at LIVMARLIhcp.com

IMPORTANT SAFETY INFORMATION (cont'd)

DRUG INTERACTIONS

Administer bile acid binding resins at least 4 hours before or 4 hours after administration of LIVMARLI.

A decrease in the absorption of OATP2B1 substrates (eg, statins) due to OATP2B1 inhibition by LIVMARLI in the GI tract cannot be ruled out. Consider monitoring the drug effects of OATP2B1 substrates as needed.

References: 1. Krantz ID, Piccoli DA, Spinner NB. Alagille syndrome. *J Med Genet.* 1997;34(2):152-157. doi:10.1136/jmg.34.2.152 2. Jesina D. Alagille syndrome: an overview. *Neonatal Netw.* 2017;36(6):343-347. doi:10.1891/0730-0832.36.6.343 3. Hartley JL, Gissen P, Kelly DA. Alagille syndrome and other hereditary causes of cholestasis. *Clin Liver Dis.* 2013;17(2):279-300. doi:10.1016/j.cld.2012.12.004 4. Kamath BM, Stein P, Houwen RHJ, Verkade HJ. Potential of ileal bile acid transporter inhibition as a therapeutic target in Alagille syndrome and progressive familial intrahepatic cholestasis. *Liver Int.* 2020;40(8):1812-1822. doi:10.1111/liv.14553 5. LIVMARLI® (maralixibat) oral solution. Prescribing Information. Mirum Pharmaceuticals, Inc. 6. Martin P, Apostol G, Smith W, Jennings L, Vig P. Dose-dependent fecal bile acid excretion with apical sodium-dependent bile acid transporter inhibitors maralixibat and volixibat in a dose-ranging phase 1 study in overweight and obese adults. Poster presented at: American Association for the Study of Liver Diseases: The Liver Meeting; November 8-12, 2019; Boston, MA. 7. Gonzales E, Hardikar W, Stormon M, et al. Efficacy and safety of maralixibat treatment in patients with Alagille syndrome and cholestatic pruritus (ICONIC): a randomised phase 2 study. *Lancet.* 2021;398(10311):1581-1592. doi:10.1016/S0140-6736(21)01256-3

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