

A guide for people with Alagille syndrome
aged 12 years old and up

For people with Alagille syndrome...

BATTLE BILE ACID BUILDUP*

Take down cholestatic pruritus.

LIVMARLI is an FDA-approved medicine for cholestatic pruritus (itch)
in patients with Alagille syndrome who are 3 months of age and older.

*LIVMARLI targets and temporarily blocks the ileal bile acid transporter (IBAT). In doing so, LIVMARLI lowers the amount of bile acids in the body because more bile acids are removed in feces. The way LIVMARLI improves cholestatic pruritus is not completely known.

IMPORTANT SAFETY INFORMATION

What are the possible side effects of LIVMARLI?

LIVMARLI can cause serious side effects, including:

Changes in liver tests. Changes in certain liver tests are common in patients with Alagille syndrome but may worsen during treatment with LIVMARLI.

See page 2 for more information on liver tests.

Please see Important Safety Information throughout
and full Patient Information.

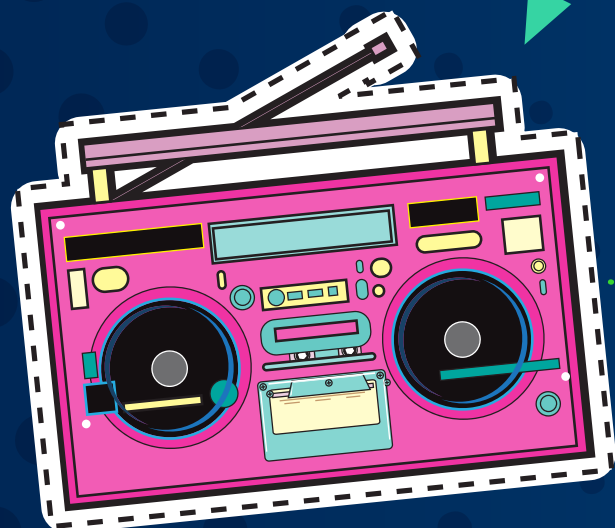
 **Livmarli**[®]
(maralixibat) oral solution

A Guide to Life With



TAKING CHARGE OF MY TREATMENT!

As you get older, you may start to take more responsibility over your health. Read on to learn about a medicine that may help you treat the itch in Alagille syndrome. Plus, look for the “TAKING CHARGE OF MY TREATMENT” sections throughout this brochure for steps that you can take today.



IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

Changes in liver tests may be a sign of liver injury and can be serious. Your health care provider should do blood tests before starting and during treatment to check your liver function.

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Please see Important Safety Information throughout and full [Patient Information](#).

Alagille Syndrome Treatment Option

HOW CAN I BATTLE BILE ACID BUILDUP* AND TREAT THE ITCH?

You may have tried other medicines or home remedies to reduce the itching, but sometimes it's necessary to try something made specifically for the itchiness in Alagille syndrome.

That's where LIVMARLI comes in.

LIVMARLI targets and inhibits something in your body called the ileal bile acid transporter (IBAT). In doing so, LIVMARLI lowers the amount of bile acids in the body.



LIVMARLI is a medicine that's **made to treat cholestatic pruritus in Alagille syndrome.**

*LIVMARLI targets and temporarily blocks the ileal bile acid transporter (IBAT). In doing so, LIVMARLI lowers the amount of bile acids in the body because more bile acids are removed in feces. The way LIVMARLI improves cholestatic pruritus is not completely known.

IMPORTANT SAFETY INFORMATION (cont'd)

Tell your health care provider right away if you get any signs or symptoms of liver problems, including:

- nausea or vomiting
- skin or the white part of the eye turns yellow
- dark or brown urine
- pain on the right side of the stomach (abdomen)
- loss of appetite

An Itch You Can't Scratch

WHAT DOES ITCH LOOK LIKE FOR ME?

You're probably very familiar with **cholestatic pruritus (itch) that's common with Alagille syndrome.** This itch leads some people to scratch so hard and so often that it causes red marks, bleeding, and scarring.



Itch is different for everyone. That's why the impact of the itch can reach beyond scratching and often lead to:

- Wiggling, rubbing, and/or fidgeting
- Irritability
- Sleep troubles
- Physical discomfort

This challenging symptom happens because people with Alagille syndrome experience a buildup of bile acids in the liver and blood. This buildup occurs because bile—a fluid that helps with the digestion of fats—can't properly drain out of the liver.

Is the itch disrupting your day-to-day life?

If your itch is impacting your sleep, or your school, work, and/or social life, your doctor needs to know.

- Take note of how itchy you're feeling every day. You can do this in a journal or by downloading the Itch✓ app to your phone



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TAKING CHARGE OF MY TREATMENT



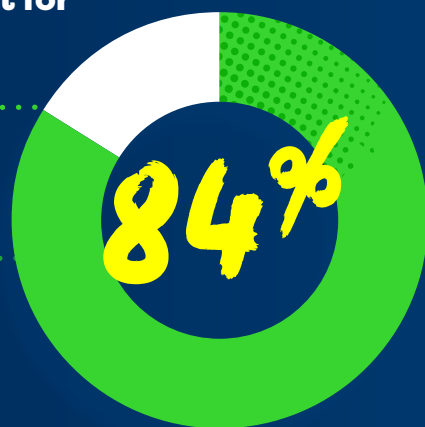
Please see Important Safety Information throughout and full Patient Information.

Results With LIVMARLI

HOW HAS LIVMARLI HELPED PEOPLE LIKE ME WITH ALAGILLE SYNDROME?

LIVMARLI can help people like you feel less itchy. People with Alagille syndrome were treated with LIVMARLI in a **2-part clinical study**. The first part lasted 1 year. During the first year, all participants were given LIVMARLI, except for during a 4-week period in which half of the participants were given LIVMARLI and the other half were not. In the second part, all participants received LIVMARLI and had the option to **stay on treatment for up to 4 years total**.

During the first year of taking LIVMARLI, **84%** of people with Alagille syndrome **experienced less itchiness** than they felt at the start of treatment.



Throughout the first year of the clinical study, for people taking LIVMARLI, **the number of days with minimal to no itch continued to increase**.

WHEN WILL LIVMARLI START WORKING?

For many people taking LIVMARLI, **itch relief was seen at the very first assessment**.* These itch improvements continued for 3 to 4 months.

*Itch relief was first assessed at Week 3.

For people who stayed on LIVMARLI in the long-term part of the study, **itch relief was maintained for nearly 4 years**.

During the long-term study, patients experienced **little to no itch for the vast majority (95%) of days**.

IMPORTANT SAFETY INFORMATION (cont'd)

Stomach and intestinal (gastrointestinal) problems. LIVMARLI can cause stomach and intestinal problems, including diarrhea, stomach pain, and vomiting during treatment. Tell your health care provider right away if you have any of these symptoms more often or more severely than normal for you.

Getting Started With LIVMARLI

HOW DO I TAKE LIVMARLI?

When do I take LIVMARLI?

- **Once daily**
- 30 minutes before a meal in the morning

How do I take LIVMARLI?

- By mouth, directly from a measuring device that comes with your medicine

What if I don't like taking liquid medicines?

You may want to try the following tips:

- **Squirt the medicine** into the inside of the cheek to avoid most taste buds
- **Suck on some fruit** (for example, an orange or lemon) before or after taking LIVMARLI
- **Store your medicine** in the refrigerator for a chilled taste

Where should I keep LIVMARLI?

- Store unopened LIVMARLI at room temperature between 68 °F and 77 °F (20 °C and 25 °C)
- Throw away any remaining LIVMARLI 100 days after first opening the bottle
- After opening the LIVMARLI bottle, store below 86 °F (30 °C)—once opened, the bottle can be refrigerated
- Keep LIVMARLI and all medicines out of the reach of children

What if I forget to take LIVMARLI?

- If you miss a dose within 12 hours of the time you normally take it, take LIVMARLI as soon as possible, and then take your next dose at the usual time
- If it's been more than 12 hours, skip the dose and continue your original dosing schedule the next day

Please see **Important Safety Information** throughout and full **Patient Information**.

Possible Side Effects

WHAT ELSE DO I NEED TO KNOW ABOUT LIVMARLI?

It's possible to have side effects while taking LIVMARLI. Talk to your doctor about what to expect and how to manage any side effects.

Common Side Effects

The most common side effects with LIVMARLI include:

- Changes in liver tests
- Stomach and intestinal (gastrointestinal) problems, including diarrhea, stomach pain, and vomiting
- A condition called fat-soluble vitamin (FSV) deficiency, caused by low levels of certain vitamins (vitamins A, D, E, and K) stored in body fat. Your doctor should do blood tests before and during treatment to check for levels of these vitamins in your body
- Gastrointestinal bleeding
- Bone fractures

Your doctor may change your dose or temporarily or permanently stop treatment with LIVMARLI if you have certain side effects. These are not all the possible side effects of LIVMARLI. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.

TAKING CHARGE OF MY TREATMENT!

I think I might be having a side effect. Now what?

Contact your doctor or health care team for medical advice about any side effects you're having.

Talk to your health care team before taking any other medication.

If you have loose stools or diarrhea, drink plenty of water and other clear liquids to help prevent dehydration.



Please see Important Safety Information throughout and full Patient Information.

Mirum Access Plus

WHAT SUPPORT IS AVAILABLE?

When starting a new medication, it's hard to know what to expect. If you're feeling overwhelmed, Mirum Access Plus can point you in the right direction.

Mirum Access Plus is a support program that makes it easy for you to start and stay on LIVMARLI.

- ✓ **96% of patients are approved for LIVMARLI by their insurance*** and **pay \$10 or less** per fill.†
- ✓ **Cost support options are available** if there is a disruption in insurance coverage.‡
- ✓ Mirum Access Plus **Navigators are here to support you** and answer your questions—whenever and wherever you need us.

TAKING CHARGE OF MY TREATMENT!

There's More to Mirum Access Plus

Mirum Access Plus is available to help you not only get started with LIVMARLI, but to provide support along the way. Your Navigator will be your teammate throughout treatment. All of our Navigators have experience providing health education and supporting people with rare diseases like you.

Visit LIVMARLI.com/mirum-access-plus-map/ to learn what services and support are available to you.

Talk to your parent(s) or guardian(s) about enrolling in Mirum Access Plus. Text ENROLL to **833-MAP-4YOU (833-627-4968)** to sign up.

*Data from 1/1/2022 to 11/30/2022.

†Exceptions apply for some patients, including those with TRICARE insurance.

‡Subject to program terms and conditions.

Real Patient, Real Story

MEET EMMA

About Emma

- **Current age:** 18 years old
- **Age at diagnosis:** By 3 months old
- **Level of itch:** Constant active scratching leading to red marks, bleeding, and scarring
- **Other symptoms related to Alagille syndrome:** Delays in growth
- **Impact of cholestatic pruritus on daily life:**
 - Moody and irritable
 - Significant sleep troubles
 - Emma’s mother said that Emma would often rip holes in her clothing and bite at her skin

Experience With LIVMARLI

- **Level of itch:** 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:**
 - Fewer red marks and no more bleeding
 - Less moody and less irritable
 - Fewer sleep troubles

IMPORTANT SAFETY INFORMATION (cont’d)

A condition called **Fat Soluble Vitamin (FSV) Deficiency** caused by low levels of certain vitamins (vitamins A, D, E, and K) stored in body fat. FSV deficiency is common in patients with Alagille syndrome but may worsen during treatment. Your health care provider should do blood tests before starting and during treatment.

Other common side effects reported during treatment were gastrointestinal bleeding and bone fractures.

Tell your health care provider about all medicines that you take. LIVMARLI may affect the way some other medicines work, and some other medicines may affect the way LIVMARLI works. If you take a medicine that lowers cholesterol by binding bile acids, such as cholestyramine, colestipol, or colestipol, take it at least 4 hours before or 4 hours after you take LIVMARLI.



EMMA
18 Years Old
Taking LIVMARLI since 2016

Story reported by caregiver.
Individual results may vary.

Important Safety Information

INDICATION

LIVMARLI is a prescription medicine used to treat cholestatic pruritus (itch) in patients with Alagille syndrome 3 months of age and older. It is not known if LIVMARLI is safe and effective in children under 3 months of age or in adults 65 years and older.

IMPORTANT SAFETY INFORMATION

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LIVMARLI is taken by mouth, 1 time each day, 30 minutes before a meal in the morning. Be sure to use the provided oral dosing dispenser to accurately measure the dose of medicine.

These are not all of the possible side effects of LIVMARLI. Call your health care provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please see full Patient Information.

For people with Alagille syndrome...

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Take down cholestatic pruritus.

To learn more about LIVMARLI, talk to your doctor and visit www.LIVMARLI.com.



Early Itch Improvements With Lasting Results

For many patients, itch relief was **seen at the very first assessment**.[†]

In the clinical study, patients who stayed on LIVMARLI long term (up to 4 years) saw **lasting itch relief**.



Helping Patients for Years

Backed by more than 5 years of data,[‡] LIVMARLI has a well-known safety record in patients with the itch in Alagille syndrome.

Over the last 6+ years, LIVMARLI has been used by **500+ patients to battle bile acid buildup**.[§]



Getting Started and Finding Support Is Easy

96% of patients are approved for LIVMARLI by their insurance^{||} and **pay \$10 or less** per fill.^{††}

Mirum Access Plus offers **resources, education, and services** to help you start and stay on LIVMARLI.

*LIVMARLI is an ileal bile acid transporter (IBAT) inhibitor that interrupts bile acids from going back into the liver. In doing so, LIVMARLI lowers the amount of bile acids in the body because more bile acids are removed in feces. The way LIVMARLI improves cholestatic pruritus is not completely known.

[†]Itch relief was first assessed at Week 3.

[‡]From extended observation of patients taking LIVMARLI after completing a placebo-controlled study.

[§]Number includes all patients with cholestatic pruritus in Alagille syndrome treated with LIVMARLI in clinical studies, as well as those treated in the United States commercially and through the expanded access program.

^{||}Data from 1/1/2022 to 11/30/2022.

^{††}Exceptions apply for some patients, including those with TRICARE insurance.

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