For people with cholestatic pruritus (itch) in Alagille syndrome...

The First and Only Treatment to Provide Early Improvements With Long-Term Impact

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

Visit **LIVMARLI.com** to learn more.

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. These changes 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.

Please see page 6 for more information on liver tests.

Please see Important Safety Information throughout and full Patient Information for LIVMARLI.

Livmarli®
(maralixibat) oral solution

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Alagille syndrome can AFFECT THE LIVER

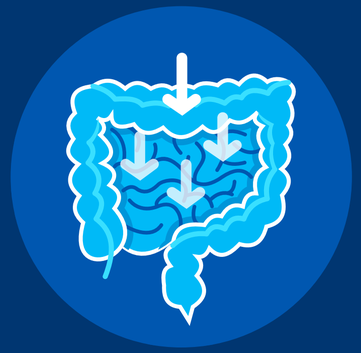
Alagille syndrome can affect several parts of the body, including the liver. In the liver, there is a network of tubes called **bile ducts.** For people with Alagille syndrome, these tubes are smaller than normal or malformed. This **prevents bile from flowing out of the liver.**

BILE 101

Bile is a fluid that is created in the liver and then released into the intestines.

Bile has many purposes, including:

Aiding in the digestion of fats



Helping with the absorption of fat and certain types of vitamins (vitamins A, D, E, and K)

When bile is not able to flow out of the liver, **bile acids**—a part of bile—**build up in the liver and the blood.**

It all starts with THE ITCH

When bile acids build up in the liver and consequently spill over into the bloodstream, this can cause **an increase in bile acids throughout the entire body**. These high levels of bile acids are **an underlying cause of cholestatic pruritus (itch)** in Alagille syndrome.

A COMMON AND CHALLENGING SYMPTOM

The itch affects **up to 88%** of people with Alagille syndrome. It is considered the most burdensome symptom.

The itch can affect a person's emotional, mental, and physical well-being. Signs and symptoms related to the itch can look different during the day and night, vary among patients, or even change with age.

- **INFANTS**
- **CHILDREN**
- **TEENAGERS AND YOUNG ADULTS**

INFANTS

Early signs of itching may often look like hunger or tired cues, such as wiggling, rubbing of the eyes and ears, and/or irritability.

No matter the age, the itch is often a very challenging symptom for people with Alagille syndrome.

It all starts with THE ITCH

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CHILDREN

They may experience various symptoms because of the itch, including active scratching, red marks and scarring, sleep troubles, and/or difficulty focusing.

No matter the age, the itch is often a very challenging symptom for people with Alagille syndrome.

It all starts with THE ITCH

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- **INFANTS**
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TEENAGERS AND YOUNG ADULTS

Those who struggle with the itch may deal with physical discomfort, mental and emotional health effects, scarring, and/or difficulty sleeping.

No matter the age, the itch is often a very challenging symptom for people with Alagille syndrome.

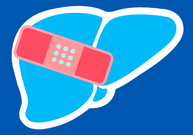
The scratching IS A SIGNAL

Bile acid buildup causes immediate problems that can be seen and felt, like the itch, but it also causes problems beneath the skin's surface.

Over time, the buildup of bile acids in the liver can lead to...

Inflammation

Damage to the liver that worsens over time



Scar tissue in the liver (known as fibrosis)

In the past, there were limited treatment options for cholestatic pruritus (itch) in Alagille syndrome. As a result, burdensome symptoms like the itch and the damage caused by bile acid buildup often led to **surgery or liver transplant** being used as treatment.

In Alagille syndrome, the unmanageable itch was a reason for **49%** to **82%** of liver transplants.

For people with Alagille syndrome, there's a need for effective treatment options that lower bile acid buildup, rapidly relieve the itch, and improve long-term liver outcomes.

With LIVMARLI, you can...

BATTLE BILE ACID BUILDUP.* Take down cholestatic pruritus.

What is LIVMARLI?

How does LIVMARLI work?

What does this mean for patients like me?

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

*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.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

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

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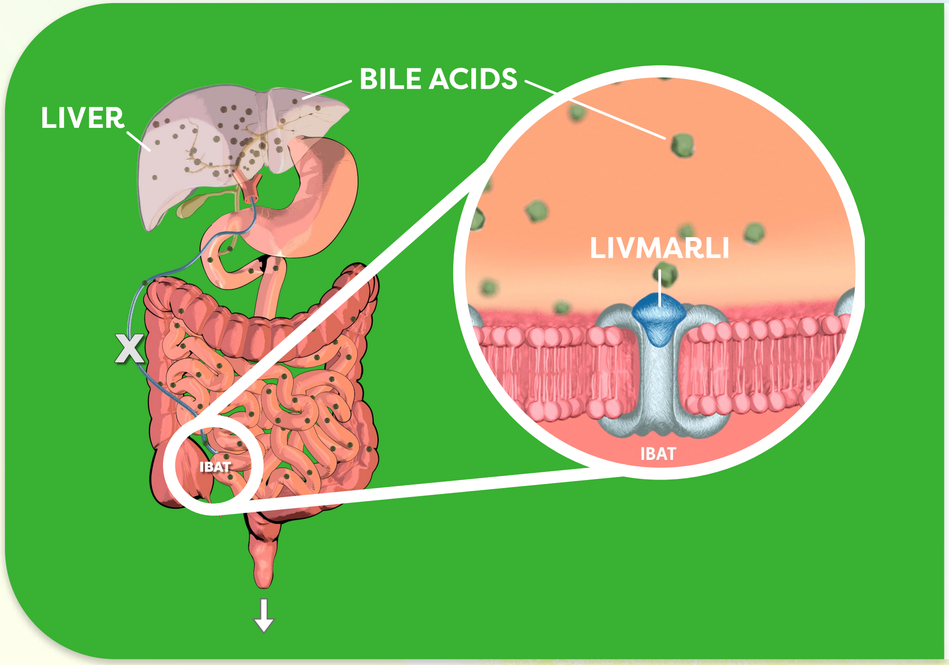

LIVMARLI **targets and temporarily blocks** something in the body called the ileal bile acid transporter (IBAT). In doing so, LIVMARLI **lowers bile acids in the body** (as measured by levels in the blood) by:

- Interrupting bile acids from going back into the liver
- Increasing the amount of bile acids that are removed in feces

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BATTLE BILE ACID BUILDUP.* Take down cholestatic pruritus.

- What is LIVMARLI?
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BYE, BYE, BILE ACID BUILDUP

During the first year of treatment with LIVMARLI, **more than 80%** of people experienced a **decrease in bile acid buildup** with LIVMARLI compared with when they started.

In the clinical study for LIVMARLI, **a decrease in bile acid buildup was associated with a decrease in itch intensity,** which means that these patients experienced less itch.

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Assessing improvements in cholestatic pruritus (itch)

HOW THE ITCH WAS MEASURED

What was the clinical study for LIVMARLI?

How did you monitor a patient's itch?

What was considered to be an improvement in itch?

Patients were treated with LIVMARLI in a **4-year clinical study**. The purpose of the study was to find out if LIVMARLI helped reduce a patient's level of itch.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

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.

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Assessing improvements in cholestatic pruritus (itch)

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Itch Reported Outcome (ItchRO) Tool

In the clinical study, patients kept an ongoing log of their itch using the Itch Reported Outcome (ItchRO) tool. **This tool was used to measure itch severity over time based on a 5-point scale:**

0 Not itchy at all

IMPORTANT SAFETY INFORMATION (cont'd)

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1 **A little bit itchy**

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4 **Extremely itchy**

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Assessing improvements in cholestatic pruritus (itch)

HOW THE ITCH WAS MEASURED

What was the clinical study for LIVMARLI?

How did you monitor a patient's itch?

What was considered to be an improvement in itch?

Changes in ItchRO score by 1 point or more represent noticeable differences in the itch; for example, a change from 3 to 2 means that a patient went from feeling very itchy to feeling somewhat itchy.

Example:

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

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Early improvements in cholestatic pruritus (itch)

THE RESULTS FROM THE STUDY

LIVMARLI was shown to provide fast and lasting itch relief.

RAPID RESULTS

For many patients taking LIVMARLI, itch relief was seen **at the very first assessment.*** Full improvement in the itch was felt by 3 to 4 months on LIVMARLI, and this relief was maintained through the first year.

SIGNIFICANT RELIEF

During the first year of treatment with once-daily LIVMARLI, **more than 80% of patients experienced less itchiness** than they felt at the start of treatment. This means that patients had **an improvement in their Itch Reported Outcome (ItchRO) score of 1 or more.**

*Itch relief was first assessed at Week 3.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

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.

Assessing the long-term impact to the liver

HOW THE LONG-TERM IMPACT WAS MEASURED

How was the impact on the liver studied?

What do you mean by "transplant-free survival"?

How did the studies work?

There were **3 clinical studies** conducted for patients with Alagille syndrome. Some patients from those studies chose to **stay on LIVMARLI long term**. These patients were observed to see if there were any factors that could predict transplant-free survival.

IMPORTANT SAFETY INFORMATION (cont'd)

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, colesevelam, or colestipol, take it at least 4 hours before or 4 hours after you take LIVMARLI.

Assessing the long-term impact to the liver

HOW THE LONG-TERM IMPACT WAS MEASURED

How was the impact on the liver studied?

What do you mean by "transplant-free survival"?

How did the studies work?

Transplant free means patients were still alive and had not undergone a liver transplant. Transplant-free survival refers to **the percentage of patients who are alive and have not gotten a liver transplant** at a certain point in time.

IMPORTANT SAFETY INFORMATION (cont'd)

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HOW THE LONG-TERM IMPACT WAS MEASURED

How was the impact on the liver studied?

What do you mean by "transplant-free survival"?

How did the studies work?

In the clinical studies, patients kept an ongoing log of their itch using the **Itch Reported Outcome (ItchRO) tool**. They were then assessed to **determine the relationship between their itch intensity score and transplant-free survival**.

IMPORTANT SAFETY INFORMATION (cont'd)

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Long-term impact to the liver

THE RESULTS FROM THE LONG-TERM STUDIES

Patients who experienced a significant improvement in their itch were also more likely to experience transplant-free survival, meaning that they were alive and did not have a liver transplant.

For those who achieved more than a 1-point reduction in ItchRO(Obs), **93% were alive and did not have a transplant** 6 years after starting LIVMARLI.

- For those who did not achieve more than a 1-point reduction in ItchRO(Obs), only 57% remained alive and did not have a transplant 6 years after starting LIVMARLI

ITCHRO +

IMPORTANT SAFETY INFORMATION (cont'd)

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.

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Livmarli® (maralixibat) oral solution

Long-term impact to the liver

THE RESULTS FROM THE LONG-TERM STUDIES

ITCHRO

What Does All This Information Mean?

There were 3 clinical studies conducted for patients with Alagille syndrome. Some patients from those studies chose to stay on LIVMARLI long term. During that time, patients used the Itch Reported Outcome (ItchRO) tool to measure itch severity over time based on a 5-point scale:

So, for those who had more than a 1-point improvement in their itch—for example, going from “very itchy” to “a little bit itchy”—93% were alive with their liver after 6 years of taking LIVMARLI. For those who had a 1-point improvement in their itch or less—for example, going from “very itchy” to “somewhat itchy”—57% were alive with their liver after taking LIVMARLI for 6 years.

IMPORTANT SAFETY INFORMATION (cont’d)

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Always Safety First

Well-Known Safety Record

LIVMARLI may cause side effects.

The most common side effects with LIVMARLI include:

- Changes in liver tests
- Stomach and intestinal (gastrointestinal) problems
- 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
- Other common side effects reported during treatment with LIVMARLI were gastrointestinal bleeding and bone fractures

Talk to your or your child's doctor about what to expect and how to manage any side effects. 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. You may report side effects to the FDA at 1-800-FDA-1088.

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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

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. These changes 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. 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

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.

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For people with cholestatic pruritus (itch) in Alagille syndrome...

The First and Only Treatment to Provide Early Improvements With Long-Term Impact

RAPID RESULTS THAT CONTINUED OVER TIME

More than 80% of patients experienced less itchiness than they felt at the start with LIVMARLI, with improvements seen as early as the very first assessment* and continued through the first year.

*Itch relief was first assessed at Week 3.

LONG-TERM IMPACT TO THE LIVER

For patients who experienced a significant improvement in their itch (that is, more than a 1-point reduction in ItchRO[Obs]), **93% were alive and did not have a liver transplant** 6 years after starting LIVMARLI.

For those who had a 1-point reduction or less in ItchRO(Obs), **57% remained alive and did not have a transplant** 6 years after starting LIVMARLI.

IMPORTANT SAFETY INFORMATION (cont'd)

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Talk to your doctor about LIVMARLI and visit **LIVMARLI.com** to see patient stories.

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