

Rise above cholestatic pruritus (itch) to...

LIVMARLI UP

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

Please see page 2 for more information on liver tests.

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

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

DISCOVER LIVMARLI

Finding out that you or your child has Alagille syndrome can be hard. Finding ways to manage the condition can be even harder.

This brochure will explain an FDA-approved medication that targets one of the underlying causes of liver-related problems in Alagille syndrome.



If you care for a young child with Alagille syndrome, check out page 28 of this brochure for ways to talk with your child about LIVMARLI.

I'M FINLEY



I'M NOELLE



I'M EMMA



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. 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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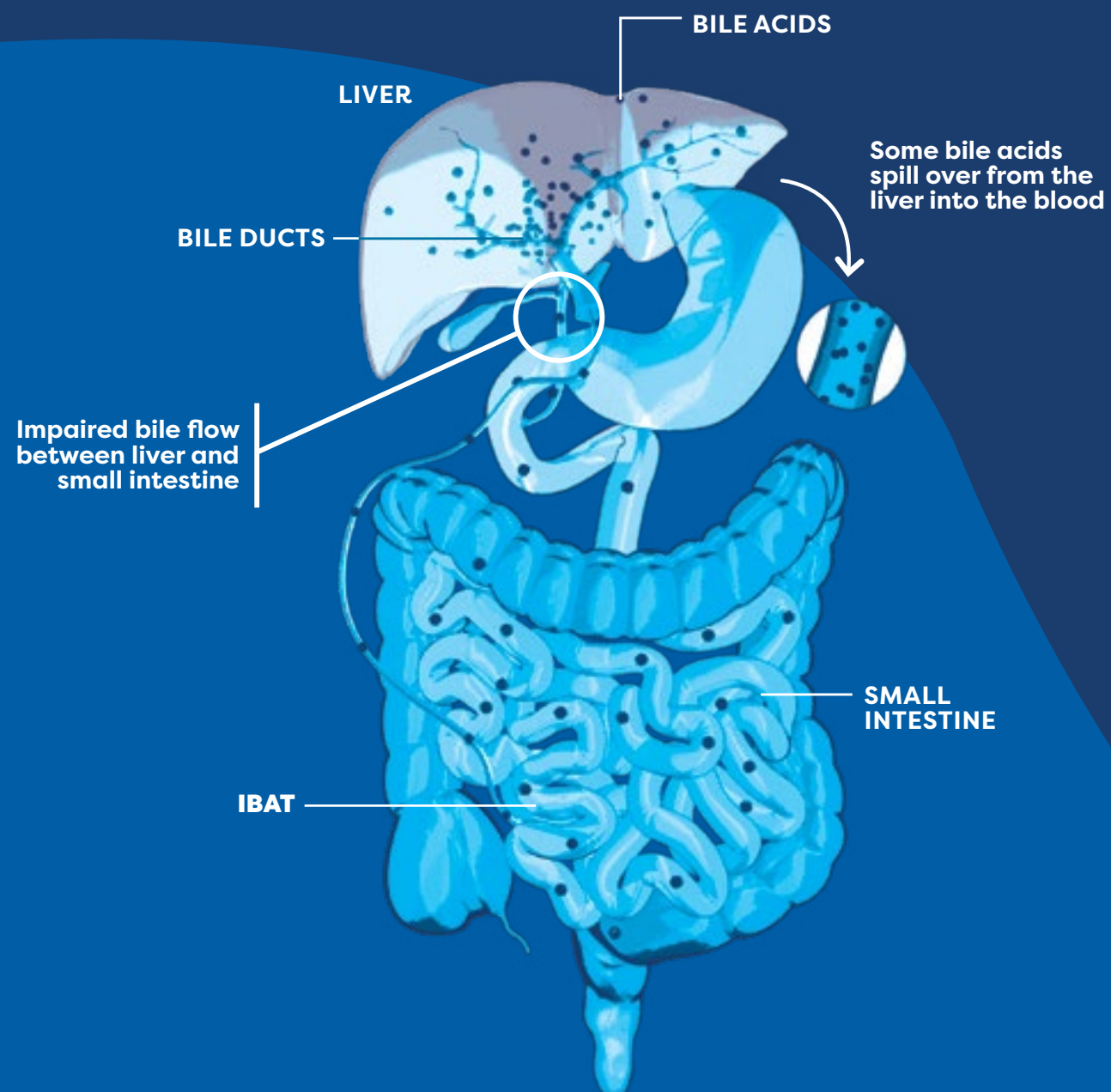
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Understanding the Disease

HOW DOES ALAGILLE SYNDROME AFFECT THE LIVER?

Alagille syndrome is a rare disease that can affect several parts of the body, including the liver.

Liver problems occur because a network of tubes in the liver, called bile ducts, are smaller than normal or malformed. This prevents bile—a fluid that helps with digestion of fats—from flowing out of the liver. Bile acids, which are a part of bile, then **build up in the liver and the blood**.



Immediate and Long-Term Impact

WHAT ARE THE EFFECTS OF BILE ACID BUILDUP?

When bile acids build up in the liver, they 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. **The itch affects up to 88% of people with Alagille syndrome and is considered the most burdensome symptom.**

Over time, the buildup of bile acids in the liver can lead to long-term problems, including:



Bile acid buildup causes immediate problems (the itch) and has long-term impacts (liver problems).

Identifying the Signs

CHOLESTATIC PRURITUS (ITCH) IS MORE THAN JUST SCRATCHING

Itch is often a very challenging symptom for people with Alagille syndrome. Beyond the scratching, 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/or night, vary among patients, or even change with age.

Recognize the signs of itch at every age



INFANTS

The itch in Alagille syndrome is **frequently first seen in infancy or early childhood**. Early signs of itching may often look like hunger or tired cues. Infants may express the itch with:



Sleep disturbances



Irritability



Wiggling



Rubbing the eyes



Digging at the ears



CHILDREN

Over time, **children may start to show their discomfort in different ways**. They may experience various symptoms because of the itch, including:



Sleep disturbances



Irritability



Skin damage



Rubbing of the feet



Active scratching



Fidgeting



Unable to focus



TEENAGERS AND YOUNG ADULTS

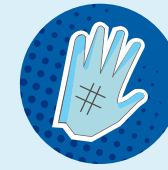
Teenagers and young adults are navigating **changes and emotions**. Those who struggle with the itch may deal with:



Sleep disturbances



Irritability



Skin damage



Rubbing of the feet



Normalizing the itch



Physical discomfort



Unable to focus

TELL THE FULL STORY OF THE ITCH

Consider the following questions and be sure to share your answers with your doctor:

- Do you notice wiggling, rubbing, and/or scratching?
- Have you noticed any changes in mood or irritability?
- Have there been problems with falling asleep or staying asleep?
- Does feeling itchy impact daily life (for example: unable to focus in school, can't participate in activities, etc)?
- Has there been frequent scratching or biting of the skin? Has it caused damage to the skin?
- Have you noticed digging in the ears, rubbing of the feet, or wiggling on the carpet?

THE NEED FOR EFFECTIVE TREATMENT



Cholestatic pruritus impacts more people than you may think

The itch affects up to 88% of people with Alagille syndrome and is among the worst of any cholestatic liver disease.



In the past, there were limited options to treat the itch

In the past, the only choices for people to manage the itch in Alagille syndrome were medicines that are not made specifically for Alagille syndrome, surgery, and/or liver transplant.



Now there is a treatment option to help relieve the 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.

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

INTRODUCING LIVMARLI

LIVMARLI is the only FDA-approved treatment for the itch in patients with Alagille syndrome who are 3 months of age and older. LIVMARLI helps lower bile acid buildup and relieve the itch.

Early Improvements With Long-Term Impact



More than 80% of patients experienced less itchiness than they felt at the start with LIVMARLI. Improvements were seen as early as the very first assessment (Week 3).

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 (please see important study design information on pages 14 and 15).†

†The impact of LIVMARLI medicine on transplant-free survival has not been established. No information about liver fibrosis (scarring) was collected.

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 600+ patients to battle bile acid buildup.§

Getting Started and Finding Support Is Easy



Approximately 94% of patients are approved for LIVMARLI by their insurance and 98% of patients pay \$10 or less per fill.¶

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

*Itch Reported Outcome for Observers (ItchRO[Obs]) is a tool designed for caregivers to measure itch severity over time.

‡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.

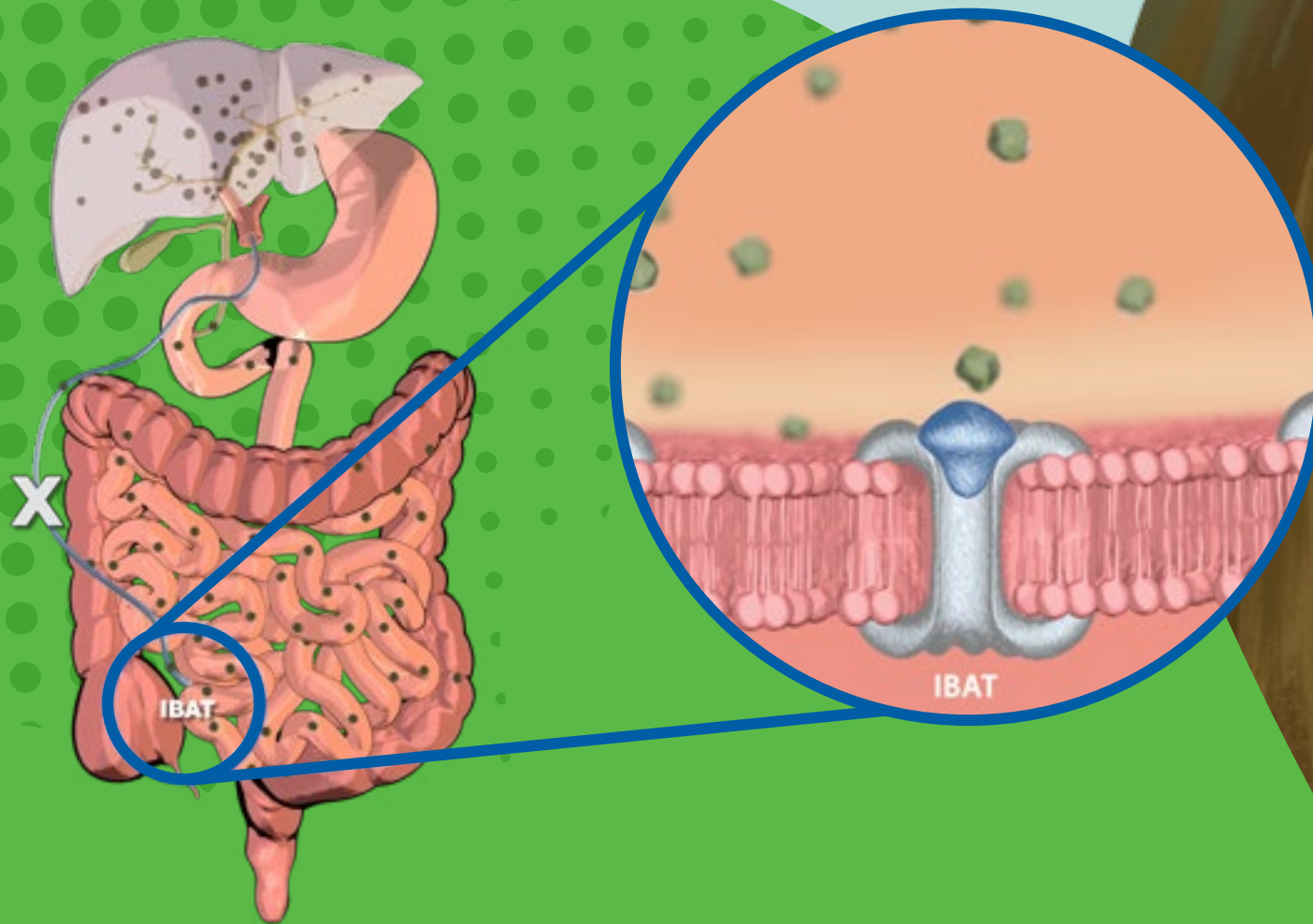
¶Data from 1/1/2022 to 8/15/2023.

†Patients enrolled in the Mirum Patient Assistance Program are included in those paying \$10 or less per fill.

HOW DOES LIVMARLI WORK?

LIVMARLI targets and inhibits 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 from the body in feces



80%

During the first year of treatment, **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, **reductions in bile acid buildup were associated with decreases in itch intensity**

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.

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

Rapid Relief That Lasts

EARLY IMPROVEMENTS IN CHOLESTATIC PRURITUS (ITCH)

The goal of LIVMARLI is to provide fast and lasting itch relief.

Patients were treated with LIVMARLI in a 4-year clinical study. During that time, many patients taking LIVMARLI experienced significantly less itchiness.



Early Improvements

For many patients taking LIVMARLI, itch relief was seen **at the very first assessment** (Week 3) and continued through the first year.



Relief With LIVMARLI

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.

600+

Over the last 6+ years, LIVMARLI has been used by 600+ patients to battle bile acid buildup.*

*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.

More Days Without Itch

LASTING RESULTS WITH LIVMARLI



Lasting Results

For those 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.



Once-daily LIVMARLI offers meaningful, long-lasting improvements in itchiness in Alagille syndrome.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

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

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

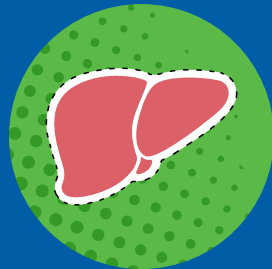
LONG-TERM IMPACT TO THE LIVER



Some patients from the clinical 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.

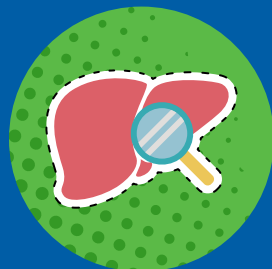
Patients kept a log of their itch using the **Itch Reported Outcome (ItchRO) tool**. They were assessed to determine the relationship between their itch intensity score and transplant-free survival.

Patients were included if they had moderate-to-severe itch, were considered to be benefitting from LIVMARLI, stayed on treatment for at least 48 weeks, and had lab results at 48 weeks. All patients took LIVMARLI.



Transplant Free

Transplant free means patients were 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.



Long-Term Impact

Patients who experienced a significant improvement in their itch were also more likely to experience transplant-free survival.

IMPORTANT SAFETY INFORMATION (cont'd)

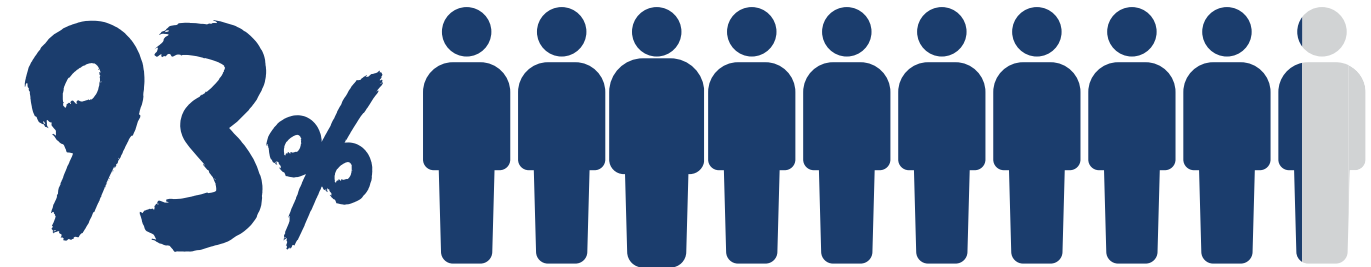
What are the possible side effects of LIVMARLI?

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 Important Safety Information throughout and full Patient Information for LIVMARLI.

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.***



were alive and did not have a transplant

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

*The impact of LIVMARLI medicine on transplant-free survival has not been established. No information about liver fibrosis (scarring) was collected.



Learn more about ItchRO scores on the next page!

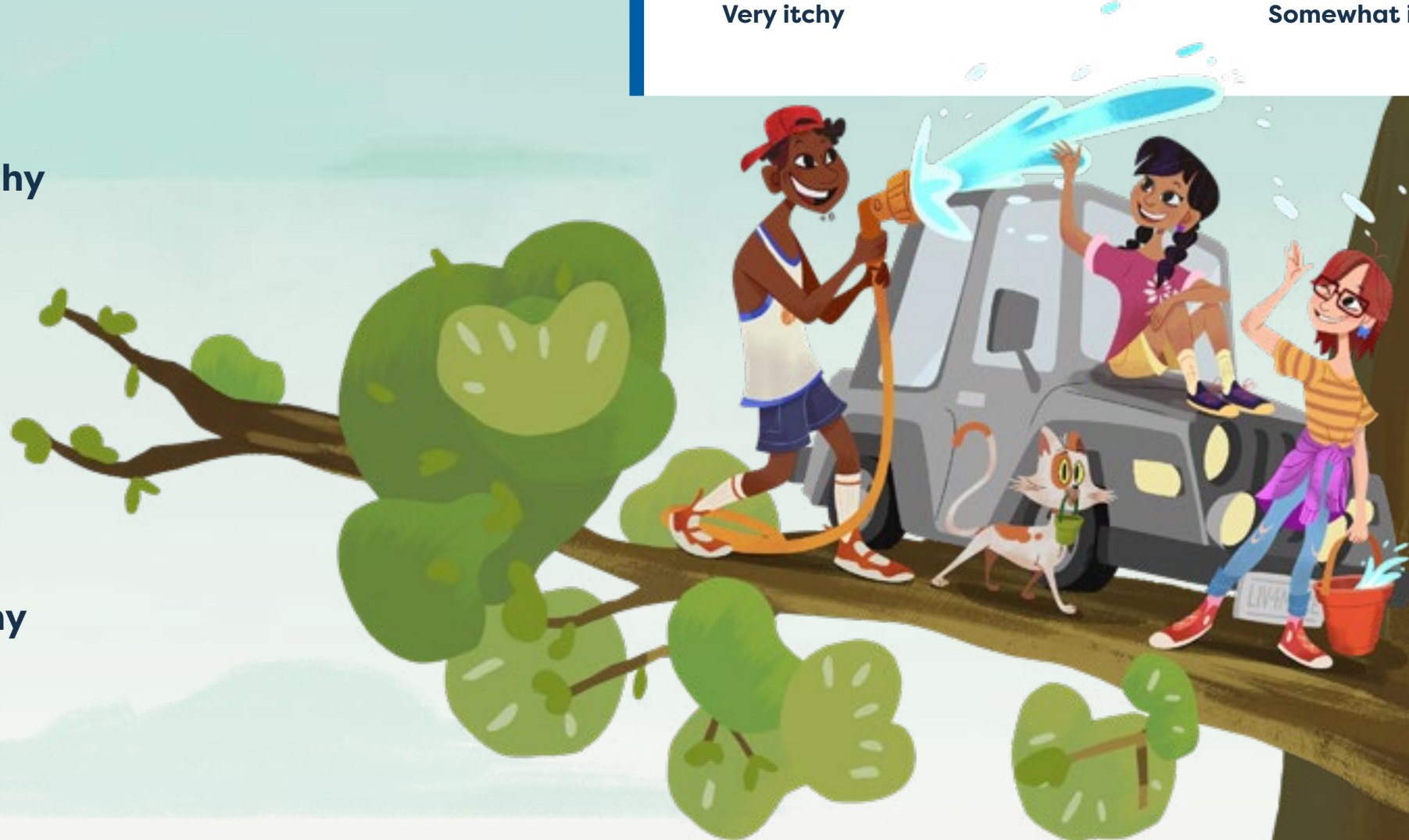
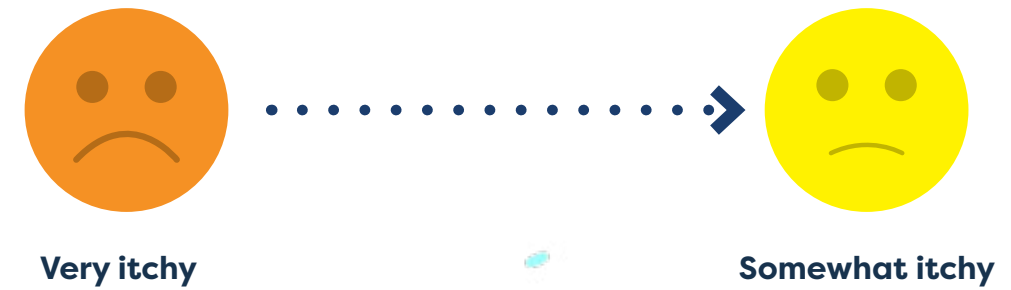
HOW THE ITCH WAS ASSESSED

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

-  **0 Not itchy at all**
-  **1 A little bit itchy**
-  **2 Somewhat itchy**
-  **3 Very itchy**
-  **4 Extremely itchy**

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:



One Dose, Once per Day

HOW IS LIVMARLI TAKEN?

Once you and your doctor have decided that once-daily LIVMARLI is the right treatment, **here's how and when you can take your liquid medicine:**



How is LIVMARLI taken?

LIVMARLI is a **liquid medicine taken by mouth** directly from a measuring device that's delivered with your medicine.



When is LIVMARLI taken?

LIVMARLI is taken **1 time each day**, 30 minutes before a meal in the morning.



How much LIVMARLI is taken?

Most patients take 1 mL or less—**about a quarter of a teaspoon!***

*Dosing is based on weight.



Always Safety First!

WHAT ARE THE POSSIBLE SIDE EFFECTS OF LIVMARLI?

LIVMARLI may cause side effects. Talk to your or your child's 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
- 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

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.

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

TIPS FOR CERTAIN SIDE EFFECTS



What steps can be taken if side effects occur?

As always, if you're experiencing side effects when taking LIVMARLI, or if your symptoms worsen, contact your doctor or health care team. You may want to share whether you were experiencing these symptoms before starting treatment, and if so, make note of how they've changed now that you're taking LIVMARLI.

If you do experience side effects with LIVMARLI, your doctor may suggest taking these steps:

Nausea, vomiting, loose stools, or diarrhea



DO drink plenty of clear liquids, such as water, to prevent dehydration



DO eat foods that are gentle on your stomach. Many people find that clear broth and/or toast help with nausea or vomiting, while dairy and/or concentrated sweets may worsen gastrointestinal symptoms

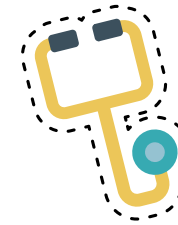


DO NOT exercise immediately after eating

Abdominal pain

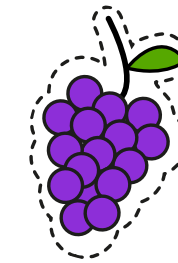


DO talk to your doctor for medical advice about abdominal pain



Should treatment with LIVMARLI stop if side effects occur?

Your doctor might choose to pause treatment or reduce your LIVMARLI dose if you are experiencing certain side effects. If treatment is paused, your doctor may recommend restarting LIVMARLI at a lower dose and slowly increasing it until you can tolerate it. You should never adjust your dose without speaking to a health care provider.



What does LIVMARLI taste like?

LIVMARLI is a grape-flavored liquid medicine.

What if someone doesn't like taking liquid medicines?

Here are a couple of tips:

- **Take the right aim.** Use the measuring device that comes with LIVMARLI to squirt the medicine into the inside of the cheek for minimal contact with taste buds
- **Add a flavorful twist.** Suck on fruit, such as an orange or lemon, before or after taking LIVMARLI
- **Cool it.** Store your bottle of LIVMARLI in the refrigerator

Mirum Access Plus



Approximately **94%** of patients are approved for LIVMARLI by their insurance and **98%** of patients pay \$10 or less per fill.*†

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

Once your doctor has sent your prescription to Mirum Access Plus, the dedicated team of experts will help you understand the process of getting your medication.

Within Your Reach



Mirum Access Plus experts will work with you and your health care team to **figure out insurance coverage.**



Mirum Access Plus experts will contact you with **refill reminders** and assist you with any steps needed before shipping the refill.



Mirum Access Plus experts will **conveniently ship your medication straight to your doorstep overnight** when coverage is approved.

There's More to Mirum Access Plus

When you sign up, Mirum Access Plus offers an enhanced, personalized experience that's designed to help you start and stay on therapy with education and resources— at no cost to you.



Simply text **ENROLL** to **833-MAP-4YOU** (833-627-4968)

Do you have questions about Mirum Access Plus?
Visit LIVMARLI.com/mirum-access-plus-map/ or contact us at 1-855-MRM-4YOU (1-855-676-4968).
We are available Monday to Friday, from 8:00 AM to 8:00 PM ET.

What other resources are available?

If you're looking to connect with people who understand what you're going through, we've got you covered. There are support groups available to listen, share information, and help guide you through the challenges of living with Alagille syndrome.



The Alagille Syndrome Alliance (ALGSA)

ALGSA is a nonprofit organization with the sole purpose of making living a life with Alagille syndrome easier. They support the Alagille syndrome community by promoting unity and advocating for a cure to inspire, empower, and enrich the lives of people affected by Alagille syndrome. You're not alone in this journey.

Join the ALGSA and benefit from:

- Resources and free materials
- Updates on research and clinical studies
- Connections with families
- Phone support with people who understand

Learn more about the ALGSA at Alagille.org

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



*Data from 1/1/2022 to 8/15/2023.

†Patients enrolled in the Mirum Patient Assistance Program are included in those paying \$10 or less per fill.

MEET SAMMY!

3 Years Old
Taking LIVMARLI Since 2021

Story reported by caregiver.
Individual results may vary.

About Sammy

- **Age at diagnosis:** 2 months old
- **Level of itch:** Itching with minor red marks
- **Impact of cholestatic pruritus on daily life:**
 - Few scratches and red marks
 - Fussiness and anxiousness
 - Always wore onesies to avoid new scratches
 - Infrequently bathed to avoid overly dry skin/ aggravating the itch

Experience With LIVMARLI

- **Level of itch:** Rubbing or mild scratching when undistracted
- **When Sammy saw improvements in itch:** Within 4 weeks
- **Living with less itch:**
 - Significantly less scratching
 - Less fussy and less irritable
- **Side effects:** Loose stools, changes in liver tests

MEET EMMA!

19 Years Old
Taking LIVMARLI Since 2016

Story reported by caregiver.
Individual results may vary.

About Emma

- **Age at diagnosis:** By 3 months old
- **Level of itch:** Constant active scratching leading to red marks, bleeding, and scarring
- **Impact of cholestatic pruritus on daily life:**
 - Moody and irritable
 - Significant sleep disturbances
 - Emma's mom said she 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 disturbances



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.

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

LIVMARLI PEER PROGRAM

Are you ready to connect with another person who understands the ins and outs of living with cholestatic pruritus in Alagille syndrome? The LIVMARLI Peer Program is for patients and their caregivers who are looking to connect with others who share their experiences.

The LIVMARLI Peer Program

Chat one-on-one with a trained LIVMARLI peer—a real person with experience navigating life with cholestatic pruritus in Alagille syndrome.

Ready to learn more about joining the LIVMARLI Peer Program? Explore our growing community at LIVMARLIPeerProgram.com



MEET YOUR LIVMARLI PEERS!



Michelle

Michelle is the mother of 9-year-old Téa, who has cholestatic pruritus in Alagille syndrome. She saw the difference LIVMARLI made for her daughter's constant itching and wants to share her story with others.



HI, I'M TÉA



Terisa

Terisa has 3 sons; her middle child, Tyler, has cholestatic pruritus in Alagille syndrome. After 10 years of persistent itching, Tyler has experienced less itch with LIVMARLI. Terisa is happy to listen to others' stories and share her experience with LIVMARLI.



HI, I'M TYLER



Dani and Jay

Dani and Jay are the parents of 2 boys, and their 2-year-old son, Finley, has cholestatic pruritus in Alagille syndrome. They are here to talk to other caregivers about why LIVMARLI was right for their child.



HI, I'M FINLEY

TALKING TO YOUR CHILD ABOUT LIVMARLI

If you care for a young child with Alagille syndrome, they may have questions. Here are some suggestions for how to talk to your child about LIVMARLI.



What makes my liver different?

When you have Alagille syndrome, **your liver needs extra help** to make sure bile doesn't get stuck as it moves out of your liver.



How do I talk about my itch?

It can be tricky learning how to talk about the itch. Think about any times or moments when you feel uncomfortable, itchy, or wiggly. Be sure to share this information with your doctor.



Why might LIVMARLI be right for me?

Your itch can be hard to manage, but **LIVMARLI was made to help you feel less itchy.** Talk to your doctor to see if LIVMARLI is right for you.



What is LIVMARLI?

LIVMARLI is a medicine that goes into your body, finds what's making you feel itchy, and fights to help you feel less itchy.



How has LIVMARLI helped kids like me?

LIVMARLI has helped other kids with cholestatic pruritus in Alagille syndrome who have bile acid buildup and feel itchy. After taking LIVMARLI, many kids were **less itchy overall, with some days where they didn't feel itchy at all.**



How do I take LIVMARLI?

It's easy to take LIVMARLI! LIVMARLI is a grape-flavored medicine that's squirted into your mouth. Every morning, you'll take it right when you wake up.

After you take it, you need to wait 30 minutes before you can eat. You can use that time to play a game, draw a picture, or get ready to start the day.



How will LIVMARLI make me feel?

LIVMARLI can help you feel less itchy. Some kids may get a bellyache or feel like they're going to be sick after starting LIVMARLI, but it should go away in a few days. Talk to an adult who can let your doctor know how you're feeling.

Your doctor is here to help you.



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. 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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Rise above cholestatic pruritus (itch) to...

LIV IT UP



EARLY IMPROVEMENTS WITH LONG-TERM IMPACT

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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 600+ patients to battle bile acid buildup.§



GETTING STARTED AND FINDING SUPPORT IS EASY

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

IMPORTANT SAFETY INFORMATION (cont'd)

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