Rise above cholestatic pruritus (itch) to

LIVMARLI is an **FDA-approved medicine** for cholestatic pruritus 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:

• Liver injury. Changes in certain liver tests are common in patients but may worsen during treatment with LIVMARLI.

Please see page 2 for more information on liver tests.





A Medicine for Cholestatic Pruritus (Itch) in Alagille Syndrome

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 26 of this brochure for ways to talk to your child about LIVMARLI.

I'M FINLEY

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

These changes may be a sign of liver injury and can be serious. Your health care provider should do blood tests and physical exams 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
- your skin or the white part of your eye turns yellow
- dark or brown urine
- pain on the right side of your stomach (abdomen)
- fullness, bloating, or fluid in your stomach area (ascites)
- loss of appetite
- bleeding or bruising more easily than normal, including vomiting blood

I'M NOELLE

Please see Important Safety Information throughout and full <u>Patient Information</u>.



'M EMMA

Table of Contents

WHAT CAN YOU FIND IN THIS BROCHURE?

- The liver and cholestatic pruritus (itch)
 - What are the effects of bile acid buildup?
- Cholestatic pruritus (itch) is 6 more than just scratching
- Cholestatic pruritus (itch) awareness and normalization
- Impact of cholestatic pruritus (itch)
- The need for effective treatment

Discover LIVMARLI

- How does LIVMARLI work to reduce cholestatic pruritus in the body?
 - Early improvements in cholestatic pruritus (itch)
- Lasting results with LIVMARLI
- Long-term impact of 15 cholestatic pruritus (itch) improvements
 - How cholestatic pruritus was assessed

123 How is LIVMARLI taken?

What are the possible 19 side effects of LIVMARLI?



Mirum Access Plus

What other resources are available?



26

Talking to your child about I IVMARI I

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 and stomach pain during treatment. Diarrhea can also cause the loss of too much body fluid (severe dehydration). Your health care provider may advise you to monitor for new or worsening stomach problems, including stomach pain, diarrhea, blood in your stool, or vomiting

Tell your health care provider right away if you have any new or worsening signs or symptoms of stomach and intestinal problems, including:

- diarrhea
- more frequent bowel movements than usual
- stools that are black, tarry, or sticky, or have blood or mucous
- vomiting
- urinating less often than usual
- dizziness
- headache



- severe stomach-area pain or tenderness



Understanding the Disease

THE LIVER AND CHOLESTATIC PRURITUS (ITCH)

How does Alagille syndrome affect the liver and cause cholestatic pruritus?



People with Alagille syndrome have too few bile ducts, which **prevents bile from** flowing out of the liver.

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



2

95% of bile acids made by the liver are **recycled in the intestine through the ileal bile acid transporter (IBAT)**.



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.

Cholestatic pruritus 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 (cholestatic pruritus) and has long-term impacts (liver problems).

CHOLESTATIC PRURITUS (ITCH) IS MORE THAN JUST SCRATCHING

Cholestatic pruritus is often a very challenging symptom for people with Alagille syndrome.

Beyond the scratching, it can be difficult to navigate all aspects of cholestatic pruritus without the right support. Signs and symptoms related to cholestatic pruritus can look different during the day and/or night, vary among patients, or even change with age.

Recognize the signs of cholestatic pruritus at every age



INFANTS

Cholestatic pruritus in Alagille syndrome is **frequently first seen in infancy or early childhood**. Early signs of cholestatic pruritus may often look like hunger or tired cues. Infants may express cholestatic pruritus with:



Sleep disturbances



Irritability



Wiggling



Rubbing the eyes



Digging at the ears

Identifying the Signs (cont'd)

CHOLESTATIC PRURITUS (ITCH) IS MORE THAN JUST SCRATCHING





Sleep disturbances



Normalizing the itch



Irritability



Physical discomfort

Skin damage



Unable to focus



Rubbing of the feet



Burning, tingling, or prickling sensation

7

Identifying the Signs (cont'd)

CHOLESTATIC PRURITUS (ITCH) AND NORMALIZATION

Cholestatic pruritus 101: Did you know?

Normalization and minimization

Symptoms of cholestatic pruritus may start to seem normal or become routine over time. This can make cholestatic pruritus easier to downplay or minimize.

Flare-ups

Cholestatic pruritus may only happen occasionally or may get worse during certain times of year. Some people refer to **times when cholestatic pruritus is worse as a "flare-up."**

Easy to mistake

Cholestatic pruritus can be **mistaken for** other common causes of itch, such as:



Change of seasons



Reactions to possible irritants, like laundry soap



Skin conditions that may cause itch



Identifying the Signs (cont'd)

IMPACT OF CHOLESTATIC PRURITUS (ITCH)





53% of people with Alagille syndrome **report difficulty falling asleep** due to cholestatic pruritus, while **59% report difficulties staying asleep**.

These **sleep disturbances** due to cholestatic pruritus **are associated with fatigue**.

All levels of cholestatic pruritus deserve attention

Share your experience with cholestatic pruritus with your doctor and let them know about any changes in symptoms.

Check out LIVMARLI.com/between-the-fines/ to hear real people open up about their lives with cholestatic pruritus.

TELL THE FULL STORY OF CHOLESTATIC PRURITUS

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?

Treating an Underlying Cause of Cholestatic Pruritus (Itch)

THE NEED FOR EFFECTIVE TREATMENT



Cholestatic pruritus impacts more people than you may think

Cholestatic pruritus 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 cholestatic pruritus

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



Learn about a treatment option to help relieve cholestatic pruritus.

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 (vitamin 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, and may monitor for bone fractures and bleeding, which are common side effects





DISCOVER LIVMARLI

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

Early Improvements With Long-Term Impact

More than 80% of patients experienced less cholestatic pruritus 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 cholestatic pruritus– that is, more than a 1-point reduction in Itch Reported Outcome (Observer) (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 15 and 16).^{†‡}

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

⁺Persistent cholestatic pruritus was a common reason for liver transplant in many patients with Alagille syndrome.

Helping Patients for Years



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

Over the last 6+ years, LIVMARLI has been used by 850+ patients with Alagille syndrome to battle bile acid buildup.^{||}

Includes clinical studies, early access program, and commercial treatment.

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.^{1#}

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

*ItchRO(Obs) is a tool designed for caregivers to measure cholestatic pruritus severity over time.

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

*Data from 1/1/2022 to 12/31/2023. *Patients enrolled in the Mirum Patient Assistance Program are included in those paying \$10 or less per fill.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

Tell your health care provider about all medicines that you take, as LIVMARLI may interact with other medicines. 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.





Rise Above Cholestatic Pruritus (Itch)

HOW DOES LIVMARLI WORK TO REDUCE CHOLESTATIC PRURITUS IN THE BODY?



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



LIVMARLI blocks bile acids from going back into the liver and increases the amount of bile acids removed from the body in feces.



In the clinical study for LIVMARLI, reductions in bile acid buildup were associated with decreases in intensity of cholestatic pruritus.

The way LIVMARLI improves cholestatic pruritus is not completely known. It may involve inhibition of the IBAT, which interrupts bile acid recycling to the liver and decreases serum bile acids.





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 cholestatic pruritus intensity

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

Your health care provider may change your dose, or temporarily or permanently stop treatment if you have certain side effects.





Rapid Relief That Lasts

EARLY IMPROVEMENTS IN CHOLESTATIC PRURITUS (ITCH)

The goal of LIVMARLI is to provide fast and lasting relief from cholestatic pruritus.

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



Early Improvements

For many patients taking LIVMARLI, relief from cholestatic pruritus 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 cholestatic pruritus than they felt at the start of treatment.

Improvements in Fatigue

An analysis of people taking LIVMARLI in a clinical study showed that those who responded to treatment experienced improvements in levels of fatigue during the first year of treatment.*

*Treatment response defined as an improvement in

Over the last 6+ years, LIVMARLI has been used by

+ patients

with Alagille syndrome to battle bile acid buildup.[†]

[†]Includes clinical studies, early access program, and commercial treatment.



Itch Reported Outcome (ItchRO) score of 1 or more.

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.





LASTING RESULTS WITH LIVMARLI



Lasting Results

For those who stayed on LIVMARLI in the long-term part of the study, relief from cholestatic pruritus was maintained for nearly 4 years.

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



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

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

These are not all of the possible side effects of LIVMARLI. For more information, ask your health care provider or pharmacist. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.





Cholestatic Pruritus (Itch) Improvements

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 cholestatic pruritus using the **Itch Reported Outcome (ItchRO) tool**. They were assessed to determine the relationship between their cholestatic pruritus intensity score and transplant-free survival.

Patients included in the long-term studies:

- Had moderate-to-severe cholestatic pruritus
- Were considered to be benefiting from LIVMARLI
- Stayed on treatment for at least 48 weeks
- Had lab results at 48 weeks

.



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 cholestatic pruritus were also more likely to experience transplant-free survival.

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

LIVMARLI can cause serious side effects, including:

- Liver injury. Changes in certain liver tests are common in patients 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 and physical exams 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
 - your skin or the white part of your eye turns yellow
 - dark or brown urine
 - pain on the right side of your stomach (abdomen)
- fullness, bloating, or fluid in
- your stomach area (ascites)
- loss of appetite
- bleeding or bruising more easily than normal, including vomiting blood





LONG-TERM IMPACT TO THE LIVER

Persistent cholestatic pruritus was a common reason for liver transplant in many patients with Alagille syndrome.

For those who achieved more than a 1-point reduction in Itch Reported Outcome (Observer) (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), 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.

Patients with >1 point reduction



Patients with ≤ 1 point reduction

57% TRANSPLANT FREE

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?



Learn more about ltchRO scores on the next page!

LIVMARLI can cause serious side effects, including:

• Liver injury. Changes in certain liver tests are common in patients 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 and physical exams 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
- your skin or the white part of your eye turns yellow
- dark or brown urine
- pain on the right side of your stomach (abdomen)
- fullness, bloating, or fluid in your stomach area (ascites)
- loss of appetite
- bleeding or bruising more easily than normal, including vomiting blood





Itch Reported Outcome (ItchRO) Tool

HOW CHOLESTATIC PRURITUS (ITCH) WAS ASSESSED

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



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 and stomach pain during treatment. Diarrhea can also cause the loss of too much body fluid (severe dehydration). Your health care provider may advise you to monitor for new or worsening stomach problems, including stomach pain, diarrhea, blood in your stool, or vomiting

Tell your health care provider right away if you have any new or worsening signs or symptoms of stomach and intestinal problems, including:

- diarrhea
- more frequent bowel movements than usual
- stools that are black, tarry, or sticky, or have blood or mucous
- vomiting
- urinating less often than usual
- dizziness
- headache



- severe stomach-area pain or tenderness



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.

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 (vitamin 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, and may monitor for bone fractures and bleeding, which are common side effects

Tell your health care provider about all medicines that you take, as LIVMARLI may interact with other medicines. 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.

Your health care provider may change your dose, or temporarily or permanently stop treatment if you have certain side effects.





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:

- Diarrhea
- Abdominal pain
- 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
- Other common side effects reported during treatment with LIVMARLI were liver test abnormalities 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.







TIPS FOR CERTAIN SIDE EFFECTS

\checkmark	_	
\checkmark	_	
1	_	
1	 _	
V	_	

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





TIPS FOR CERTAIN SIDE EFFECTS



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.

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. Consider storing your bottle of LIVMARLI in the refrigerator





Getting Started Is Easy

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.

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.



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

•••

Get started by texting MIRUM to 47936 or calling us at 1-855-MRM-4YOU (1-855-676-4968)

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.

*Data from 1/1/2022 to 12/31/2023. *Patients enrolled in the Mirum Patient Assistance Program are included in those paying \$10 or less per fill.

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 <u>Alagille.org</u>



Real Patient Stories



4 Years Old Real Person Taking LIVMARLI

Story reported by caregiver. Individual results may vary.

About Sammy

- Diagnosed with Alagille syndrome: 2 months old
- Level of cholestatic pruritus (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 cholestatic pruritus

Experience With LIVMARLI

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

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.

These are not all of the possible side effects of LIVMARLI. For more information, ask your health care provider or pharmacist. Call your doctor for medical advice about side effects. You may report side effects to the FDA at 1-800-FDA-1088.



١.



Real Patient Stories (cont'd)

MEET NOELLE!

12 Years Old Real Person Taking LIVMARLI

Story reported by caregiver. Individual results may vary.

About Noelle

- **Diagnosed with Alagille syndrome:** 2 1/2 months old
- Level of cholestatic pruritus (itch): Active scratching with red marks
- Impact of cholestatic pruritus on daily life:
 - Learned to live with cholestatic pruritus and used coping strategies
 - Used brushes to scratch her skin and often asked her mom to scratch her back
 - Significant sleep disturbances
 - Noelle's mom said Noelle would scratch all night

Experience With LIVMARLI

- Level of cholestatic pruritus: Occasional rubbing or mild scratching when undistracted
- When Noelle saw improvements in cholestatic pruritus: Within 1 week after starting LIVMARLI; improvements confirmed at 3-week follow-up appointment; itch continued to improve over time
- Living with less cholestatic pruritus:
 - Less scratching and fewer red marks
 - Fewer sleep disturbances
- Less impacted by changes in temperature and weather

IMPORTANT SAFETY INFORMATION (cont'd)

What are the possible side effects of LIVMARLI?

LIVMARLI can cause serious side effects, including:

- Liver injury. Changes in certain liver tests are common in patients 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 and physical exams 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
- your skin or the white part of your eye turns yellow
- dark or brown urine
- pain on the right side of your stomach (abdomen)
- fullness, bloating, or fluid in your stomach area (ascites)
- loss of appetite
- bleeding or bruising more easily than normal, including vomiting blood



Info for Kids

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 cholestatic pruritus (itch)?

It can be tricky learning how to talk about cholestatic pruritus. 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?

Cholestatic pruritus 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.

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 and stomach pain during treatment. Diarrhea can also cause the loss of too much body fluid (severe dehydration). Your health care provider may advise you to monitor for new or worsening stomach problems, including stomach pain, diarrhea, blood in your stool, or vomiting





TALKING TO YOUR CHILD ABOUT LIVMARLI



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 (itch) in Alagille syndrome who have bile acid buildup and feel itchy. After taking LIVMARLI, many kids **had less cholestatic pruritus 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 diarrhea or a bellyache 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.**



What are the possible side effects of LIVMARLI?

Tell your health care provider right away if you have any new or worsening signs or symptoms of stomach and intestinal problems, including:

- diarrhea
- more frequent bowel movements than usual
- stools that are black, tarry, or sticky, or have blood or mucous
 severe stomach-area pain or tenderness
- vomiting
- urinating less often than usual
- dizziness
- headache





Rise above cholestatic pruritus (itch) to



EARLY IMPROVEMENTS WITH LONG-TERM IMPACT

More than 80% of patients experienced less cholestatic pruritus 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 cholestatic pruritus—that is, more than a 1-point reduction in Itch Reported Outcome (Observer) (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 15 and 16).^{†‡}

[†]The impact of LIVMARLI medicine on transplant-free survival has not been established. No information about liver fibrosis (scarring) was collected. [‡]Persistent cholestatic pruritus was a common reason for liver transplant in many patients with Alagille syndrome.



HELPING PATIENTS FOR YEARS

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

Over the last 6+ years, LIVMARLI has been used by 850+ patients with Alagille syndrome to battle bile acid buildup.^{||}

Includes clinical studies, early access program, and commercial treatment.



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.**^{1#} Mirum Access Plus offers **resources, education, and services** to help you start and stay on LIVMARLI.

To learn more about LIVMARLI, talk to your doctor or visit <u>LIVMARLI.com</u>

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** (vitamin 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, and may monitor for bone fractures and bleeding, which are common side effects

Please see Important Safety Information throughout and full Patient Information.

*ItchRO(Obs) is a tool designed for caregivers to measure cholestatic pruritus severity over time. [§]From extended observation of patients taking LIVMARLI after completing a placebo-controlled study. [§]Data from 1/1/2022 to 12/31/2023.

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





