



Helping You and Your Doctor Make Decisions Together

A Guide for Caregivers and Patients With Alagille Syndrome (ALGS)

You've done your homework, monitored Alagille syndrome symptoms, and you still have questions about the road ahead. If you haven't already done so, now is the time to check in with your doctor, have an open conversation, and get their expert opinion.

You know yourself or your child better than anyone, which means the most important source of information for your doctor is *you*. What you share can help your doctor learn how Alagille syndrome affects daily life and decide what steps to take next, such as more monitoring or even a change to the current treatment plan. This guide can help you decide what information to share and what to ask at your next appointment.

The Itch

You or your child may feel an intense itch—also known as cholestatic pruritus. It is caused by a buildup of bile in the liver and blood. This unbearable itch can be the most frustrating symptom of Alagille syndrome. It can lead to scratches and scars. Many people also have difficulty sleeping because of the overwhelming desire to scratch.

If you feel that current treatment isn't working and/or your or your child's quality of life is affected, be honest with your doctor about your concerns.

QUESTIONS FOR YOU TO HELP ASSESS HOW ITCHY YOU OR YOUR CHILD ARE

How itchy are you or your child throughout the day, from the early morning until bedtime?

Keep a daily log of itch severity and any potential triggers for the itch, both for your own symptom tracking and to better explain to your doctor what you or your child are experiencing.

How often does feeling itchy result in skin damage (ie, red marks, scratches, open wounds, and/or scars)?

Tell your doctor if this happens under certain circumstances or times of the day.

Where on the body are you or your child itchy?

Note the specific areas of the body that are scratched the most.

How often does feeling itchy impact sleep?

Think about whether it's difficult to fall asleep, to stay asleep, or both.

How often does feeling itchy impact daily life?

Take note of whether you or your child have an inability to concentrate, feel tired in school, or struggle with participating in sports and/or playing, or other ordinary daily activities.

How often does itching negatively affect your or your child's mood?

Perhaps you're noticing low self-esteem, irritability, and/or fussiness.

How much relief do you or your child get from current treatments?

Share if you've seen a lot of relief, some relief, or no relief at all.

QUESTIONS FOR YOUR DOCTOR

- What causes the intense itch?
- Are there potential triggers that make the itch worse?
- Are there any tests that might help further evaluate or monitor this symptom?



Poor Growth

Many infants and children with Alagille syndrome suffer from poor growth, which is often called a failure to thrive. This means their growth is delayed or much less than that of other children of the same age and gender.

When thinking about development, be sure to bring up key points to your doctor.

Lately, how has your or your child's growth and development been?

It's important for your doctor to know not only if symptoms have worsened, but also if symptoms haven't gotten any better.

Are you concerned that you or your child may not be getting enough nutrients?

Reflect on the current diet, including any fat-soluble vitamins being taken, such as vitamins A, D, E, and K. Your doctor or dietitian can then make recommendations on how to ensure you or your child are getting the nutrients needed.

Are you or your child increasingly irritable and tired?

Malnutrition can impact emotional and social behavior.

QUESTIONS FOR YOUR DOCTOR

- My child doesn't want to eat. How can we make sure he or she is properly nourished?
- Are there any tests that might help to further evaluate or monitor this?
- What are realistic expectations for growth and weight?

How is your current feeding schedule working for you or your child?

Note any excess stress or sleep disturbances you or your child have experienced due to the frequency of your current feeding schedule.

Have there been any fractured or broken bones?

Be sure to mention these injuries.

How much improvement do you or your child get from current treatments?

Share if you've seen drastic improvement, slight improvement, or no improvement at all.





Painful, Yellowish Bumps

Painful, yellowish bumps, also known as xanthomas, are common in patients with Alagille syndrome. For children, the presence of these bumps can often lead to difficulties with walking and writing, and restrictions in physical activities. They can also cause feelings of low self-esteem.

If you or your child are struggling with these bumps, consider the following before your next doctor visit.

Recently, have these bumps improved, stayed the same, or gotten worse?

Consider if they have grown in size or if they have increased in number. Also consider if they have kept you or your child from everyday activities or from reaching key developmental milestones.

Describe how this symptom makes you or your child feel.

Think about the symptom's impact on daily life.

How much relief do you or your child get from current treatments?

Share if you've seen a lot of relief, some relief, or no relief at all.

QUESTIONS FOR YOUR DOCTOR

- What is likely causing these bumps?
- Are these bumps temporary or long-lasting?
- What are the treatment options for these bumps?



Yellowing of the Skin and/or Eyes

Yellowing of the skin and whites of the eyes, also known as jaundice, is common in patients with Alagille syndrome. Jaundice can cause people with Alagille syndrome to feel tired and cranky, and they may not want to eat.

If you've noticed yellowing of the skin and eyes, consider the following.

Recently, has the yellowing of the skin or eyes gotten worse, stayed the same, or improved?

Examine the face, chest, stomach, arms, and legs regularly.

How much improvement do you or your child get from current treatments?

Share if you've seen drastic improvement, slight improvement, or no improvement at all.

QUESTIONS FOR YOUR DOCTOR

- Is jaundice harmful?
- How can I tell if the jaundice is getting worse?
- Are there any tests that might help further evaluate or monitor this symptom?

Moving Forward With Confidence

Monitoring symptoms, answering questions, handling doctor's visits—you've taken an active role in your or your child's Alagille syndrome story. Now, with all of this information in mind and your family's best interests at heart, you can speak more openly with your doctor about Alagille syndrome and its impact on your lives.

Use the space below to take notes during your next appointment with your doctor.

Resources for You and Your Family

Visit **LifeWithALGS.com** for more information.



© 2021 – Mirum Pharmaceuticals, Inc.
All rights reserved. US-2100087 08/2021