



Talking with  
your Doctor



**PFIC VOICES**<sup>®</sup>

Progressive Familial  
Intrahepatic Cholestasis

*Created in partnership with PFIC Network*





## About this Guide

This guide was created with input from families living with PFIC. It may help you prepare for your or a loved one's medical appointments and create a plan.

Many physicians do not have experience treating PFIC. Hepatologists (liver doctors) and gastroenterologists (doctors who specialize in digestive illness), may be your best sources of information. Learning about PFIC — and knowing which questions to ask — can help you advocate for yourself or your child.

# Talking to Your Doctor About PFIC

Progressive familial intrahepatic cholestasis (PFIC) is a life-threatening, rare, genetic, pediatric liver disease that can take a devastating toll on affected children, their parents and their entire families.

The first signs of PFIC may include any of the following:

- unusual bruising or swelling
- jaundice
- enlarged liver or spleen
- unrelenting, insatiable itching, or pruritus

Three main genes are involved in the spectrum of PFIC, each of which is necessary for bile acid transport out of the liver. As the result of a defect in one of the genes, bile acids accumulate in the liver and bloodstream, causing progressive symptoms within and, sometimes, outside the liver. Even the same type of PFIC can affect different people in different ways.

Though PFIC is rare, there are three gene mutations that are most commonly seen, which are: ATP8B1, ABCB11, and ABCB4. However new types of PFIC are still being discovered, and doctors are still learning how the types of PFIC can affect people in different ways. For instance, an individual may have one of the above-mentioned gene mutations, wherein symptoms may “flare” for a period of time before subsiding. This intermittent form of PFIC is often referred to as Benign Recurrent Intrahepatic Cholestasis or BRIC.

# Easy Ways to Prepare for a Doctor Visit

Planning ahead for your doctor's visit can help you get the most out of each appointment. Here are steps you can take to prepare.



## **Write down your questions in advance.**

During the course of a normal day, you may think of questions to ask your doctor. It can be easy to forget what you wanted to ask on the day of your actual appointment. Keeping an ongoing list of questions can help you remember important topics.



## **Bring a notebook and pen.**

Your healthcare provider may cover so many topics that it is hard to remember everything. Taking notes can help ensure that you have a record of what you talked about. You can also ask the doctor or nurse to write down important points or print out a copy of the visit's notes.



## **Keep track of medications and dosages.**

Tell your doctor about any medication or topical treatment being taken. Be sure to track dosages, how helpful the medications have been and side effects experienced.



## **Keep a journal and bring it to your appointment.**

A written record can help you share symptoms and concerns. For instance, if you or your child often lose sleep because of severe itching, the days and nights may start to blur together. Track sleeping patterns to discuss possible solutions with your doctor. Writing down what you or your child eats can help uncover any links between diet and symptoms.



### **Bring activities.**

If you are taking a younger child to the doctor, a toy or game can help pass the time when you are waiting for the appointment to start.



### **Explain to your child what to expect.**

Doctor's appointments can be a scary experience for young children. Help them prepare for the appointment by sharing what you know in a way that they may be able to understand.

## During Your Appointment



### **Ask your most important questions first.**

If you run out of time, you'll have the answers you need most.



### **Ask more questions.**

If you don't understand something your doctor says, ask for a better explanation. Try to make sure that you leave the appointment feeling comfortable that all your questions have been answered. There is never a wrong question. Ask all questions that come to mind, even if you feel like you might know the answer.



### **Bring a companion.**

Sometimes it can be helpful to have someone with you to take notes and help you remember everything you wanted to talk about. If you have a young child, a companion can sit with your child while you're talking with the doctor so you can have a focused conversation.

# Questions to Ask Your Provider

PFIC is a progressive condition, which means that it may change over time. Answers to questions you have asked before can change from one appointment to the next. Keeping a log of questions to ask at every appointment may be helpful for tracking disease changes or progression.

## About PFIC Symptoms and Management

- What can I expect from PFIC? What risks and symptoms should I know about?
- What are the options for medication and how can they help? What side effects can I expect from the medications?
- Other than medication, what can I do to reduce symptoms?
- How will I know if and when my child needs a transplant or other surgical procedure?
- Is biliary diversion a good option for my child?
- Should my child get a genetic test for PFIC?
- Are there any resources that you can recommend that provide helpful information about PFIC and its symptoms?



- Is my child receiving adequate nutrition? What are signs or symptoms that we should be aware of to monitor vitamins?

## About Your Appointments

- How often should I expect appointments? What does each appointment involve?
- What tests should I prepare for? How long will they take?
- When will I have my child's results?
- How can I keep track of my child's test results?
- If I have a concern about my child's symptoms, who should I contact?

## Other Healthcare Needs

- Can you help me find a support group?
- Can you help me contact a social worker who can help me understand what types of financial and other support are available to me?
- Who do I go to if I have questions about insurance?
- Is all testing, including a genetic test covered by insurance?
- How can living with PFIC affect my child's mental health? Do you have a psychologist you recommend if we need one?



## Remember

Your doctor is a member of your healthcare team. Open communication will help build trust so you can achieve the best outcomes for your family.

**This brochure is published by PFIC Voices (an initiative of Albireo Pharma) in partnership with the PFIC Network, a parent- and patient-led advocacy organization.**

### **About Albireo**

Albireo is committed to supporting people with PFIC, their families, and healthcare providers with resources to better understand and manage this devastating disease. The company is focused on the development of novel bile acid modulators to treat rare pediatric liver diseases, such as PFIC.

**For more information and resources about Albireo's work with the PFIC Community, visit [www.PFICvoices.com](http://www.PFICvoices.com).**

