



Identifying research targets by **M**erging **P**atient And **C**linician **T**reatment information

PFIC IMPACT: August Focus Group Discussion Summary

Overview

We held our sixth PFIC IMPACT Focus Group meeting on August 20th, 2024!

- Attendees: 11 patients & parents + 6 clinicians & researchers
- Our main goal was to reveal knowledge gaps and variability in care that could be addressed by future research.

Background

Project IMPACT, inspired by discussions at PFIC Network's 2022 Conference, aims to make treatment decisions easier and more informed. This can be done through patient-centered Comparative Effectiveness Research (CER). CER studies compare two or more treatments based on outcomes important to patients, like itch reduction, family quality of life, and/or financial burden.

Key Takeaways

During the August Focus Group, we talked about current knowledge gaps regarding disease progression and factors involved in the causation of itch and other symptoms. We also discussed ways patients' care varies between treatment centers, including in the combination of antipruritic medications and nutritional approaches prescribed. We considered which challenges and symptoms are most important to focus on solving, including growth delays, quality of life, and diarrhea and itch post-transplant.

Discussion Summary

Knowledge Gaps

August Focus Group participants were asked to discuss the following prompt:

What questions about PFIC disease or treatments remain unanswered for you?

The ensuing discussion yielded many important questions that we've organized into five key themes below:

1. Triggers

- What causes the itch?
- Why would itch return several years after a successful diversion surgery?
- Is there a relationship between hormones/puberty and itchiness, or episodes of transplant rejection?
- Are there certain triggers for BRIC episodes to avoid (ex. vaccines)?



2. Pre-transplant prognosis

- Should PFIC be considered a family of diseases? Which genotypes?
- Does BRIC eventually progress to PFIC?
- Why do some PFIC patients have tumors in the liver at a young age?
- Why do some patients respond to IBATs or diversion surgery, and some don't? What are the environmental or genetic factors?
- Which IBAT is more effective, and has the least long-term side effects?
- How long will my native liver survive/what is the timing of transplant?

3. Post-transplant prognosis

- Why do some transplant recipients develop fatty liver in their grafts, and what we can do about it?
- How long will my transplanted liver last? Will I need another one?

4. Nutrition

- How do I know if my child with PFIC is experiencing growth delays?
- Are growth delays entirely nutritional, or are they a symptom of PFIC disease in a way that we don't understand?
- What is optimal nutritional and GI symptom management for PFIC? For FIC 1 deficiency post-transplant?
- What are the best foods to maximize nutrition absorption for PFIC kids?

5. Extrahepatic manifestations

- How is PFIC impacting biological systems outside the liver?
- How frequently should we check vitamin levels to avoid side effects of vitamin deficiencies?
- Do certain forms of PFIC contribute to hearing loss (vs. medication)?
- What causes diarrhea post-transplant, and why do certain genotypes have worse diarrhea post-transplant than others?
- What is the best way to treat post-transplant diarrhea (e.g. loperamide, maralixabat, PIBD, PEBD, etc.)?

Variability

We also explored two sources of variability in care revealed in previous focus groups - medications and nutrition interventions. Regarding *medications*, we learned:

- Some patients receive treatments in addition to IBATs, including antipruritic medications and vitamin supplements, while others do not.
- The number and type of antipruritic medications received in combination with IBATs also varies, but there is no clear explanation covering the different cases.



We discussed the difference in *nutritional care* between infants and teens with seemingly more attention given to infants, and the need for nutrition interventions to better balance optimizing growth and quality of life at all developmental stages.

Priority Areas

We closed the discussion with one final question for the group: *Which symptom or challenge is most important to you when it comes to thinking about research priorities in PFIC?* Several key topics emerged:

- Improving quality of life
- Growth delays
- Post-transplant itch and diarrhea
- Reducing unknowns!
 - Speed of disease progression
 - What factors influence the symptoms and severity of disease
 - How certain interventions will impact disease progression
 - Negative side effects of medications, pre or post-transplant

August Focus Group Wrap-Up

- We will continue to compile a list of priorities based on the treatments and outcomes identified in our discussions.
- Our ultimate goal is to transform this list of priorities into research questions that can be addressed in future research projects.



If you haven't already, please visit the [PFIC IMPACT Treatment Experience App \(TEA\)](#) and share your treatment experiences in the TEA survey. The treatment experiences you enter into the TEA are super important to get a better picture of what is most important to research!

Don't miss it! Next IMPACT activities...

- **Our next focus group will take place on Tuesday, October 22 at 6pm EST.**
 - It will be hosted as a virtual Zoom meeting and will last 90 minutes. Participants will receive a \$90 gift card as thanks for their time and input.
 - We hope to see you there! If you would like to participate, [register here](#).
- **Module 5 will go live on Monday, November 25th.**
 - Look out for emails from Melissa with an invitation!
 - If you haven't already, we encourage you to take [Modules 1, 2, 3, & 4](#).

Please contact us anytime with feedback, questions, or concerns: melissa@pfic.org