



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

2024 PFIC Family & Scientific Conference IMPACT Roundtable Discussion Summary

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IMPACT Roundtable Session

Overview

We held a two-hour session for project IMPACT during the PFIC Family & Scientific Conference on Friday April 26th, 2024!

- Attendees: 35 patients & parents + 35 clinicians & researchers
- The goals of the session were to:
 - Provide an update on IMPACT accomplishments.
 - Present the beta launch of the IMPACT Treatment Experience App (TEA).
 - Engage in discussion about knowledge gaps in PFIC treatment decision-making that could be addressed by future patient-centered outcomes research.
- The feedback provided during the roundtable session will be used to further enhance the Treatment Experience App, and to design future IMPACT activities.

Background

Launched in July 2023, Project IMPACT has now been in progress for nearly a year. The PFIC Family & Scientific conference in April 2024 served as a crucial platform to share updates and engage event attendees who may be encountering IMPACT for the first time. This conference also marked our first opportunity to have an IMPACT activity in-person! We held a dedicated 2-hour session on the first day of the conference that started with a presentation by the IMPACT project team members (you can [watch the recording here!](#)), followed by a facilitated roundtable discussion in the style of previous IMPACT focus groups.

Roundtable Session Key Takeaways

We reviewed project updates and presented the beta launch of the IMPACT TEA. Questions and feedback concerning the TEA were discussed with attendees:

1. The IMPACT TEA and the PFIC Network Patient Registry have different goals. The TEA has a single objective: collect and display treatment information. The registry collects all kinds of information from patients (including but not limited to diagnosis, symptoms, surgeries, medications, itch, quality of life, sleep, general health). Both the TEA and the registry are important for PFIC research.
2. If possible, reduce the age minimum for participation in the IMPACT TEA from 14 to 10 years old to include more patients.
3. Incorporating patient-identified outcomes is new in PFIC research. It would be helpful to find and draw upon learnings from other pediatric research projects that have engaged patients in the design and execution of research studies.



We also facilitated a discussion about treatment-decision making in PFIC, including current obstacles to reducing variability in care. Main themes that arose:

1. Clinical practice guidelines and patient and healthcare provider education might reduce variability in care that is related to lack of provider expertise.
2. Patients' personal circumstances and values inherently vary and are important factors to consider when choosing between treatments.
3. PFIC patients seek to learn from others' subjective experiences with treatment to inform their own treatment decisions. This might include outcomes not necessarily included in clinical practice such as considering the impact of a treatment on siblings and/or family life. Conducting systematic research that compares treatments based on patients' subjective experiences could provide valuable and accessible information for both patients and providers in making treatment decisions.

Roundtable Session Full Summary

Treatment Experience App Q&A

The IMPACT Project Team shared updates on project progress, and presented the beta version of the IMPACT Treatment Experience App (TEA). This was followed by a Q&A discussion that revealed the following key points:

1. The purpose of the TEA is not just to collect data, but to provide comprehensive clinical and patient-centered information about treatments to patients so they can make more informed treatment decisions with their providers.
2. The type of data being collected by the TEA is different from the PFIC Network Patient Registry.
 - The *TEA* asks patients to rate the importance of specific benefits and burdens of treatments they've experienced (whether receiving currently or >5 years ago). The goal of the TEA is to reveal what treatment outcomes matter most to PFIC patients, so that we could explore these outcomes in future research studies.
 - The *PFIC Network Patient Registry* is a comprehensive data collection that follows enrolled patients over time. Patients or their caregivers provide their diagnosis, symptoms, treatments, itch, quality of life, sleep, general health and financial burden associated with their PFIC disease, and give an update every six months. The registry can therefore track the disease in each participant over time with respect to a wide variety of outcomes.



Q&A participants shared following feedback:

- **Consider reducing age minimum required** for participation in the TEA survey. Clinicians expressed concern that the current age requirement of 14 years old will limit us in capturing the patient perspective.
- **Find examples of pediatric research projects** and explore what methods they used to identify outcomes meaningful to pediatric patients.
- **Patient-identified outcomes pose new challenges for researchers** as they can be harder to measure than traditional clinical outcomes (i.e. bilirubin levels) and require greater participation to capture. Examples of patient-identified outcomes may include quality of life, developmental delays, and days missed from school.

Facilitated Discussion

Following IMPACT project updates, we facilitated a discussion to explore knowledge gaps in treatment decision-making that could be addressed by future patient-centered outcomes research. To initiate the discussion, we posed two questions to participants:

1. What are the biggest obstacles to reducing variability in care?
2. In your experience with PFIC, have you been faced with a situation where you had to choose between two options, and if so, how did you decide?

Question 1 - Obstacles to Reducing Variability in Care

Clinicians who took part in the discussion pointed out that it's difficult to give consistent care for rare diseases like PFIC because of the differences in disease presentation that is associated with different genetic mutations. Since it is not yet well understood how exactly the PFIC diseases develop over time, or how well treatments work for each subtype, it's tough for doctors to give clear advice on treatments (especially when they have limited PFIC expertise). The current variability in care can make patients worry whether they're getting the right care, especially when they see that different hospitals have varying levels of knowledge concerning PFIC.

Solutions to reducing variability in care emerged from the discussion, including:

- **Patient education** through resources like the IMPACT Treatment Experience App (TEA), and lay language dissemination of clinical practice guidelines and study results. Accessible information about treatments enables patients to better advocate to their doctors and feel more informed in their care decisions.
- **Healthcare Provider education** on treatments and the latest research. Various potential approaches were highlighted, including:



- Sharing the IMPACT TEA with providers. The TEA includes clinical summaries of treatment information!
- Creating a resource/checklist for transition from pediatric to adult care.
- Partnering with professional societies like NASPGHAN, ESPGHAN, and AASLD to produce resources such as short videos.
- Adding questions about PFIC to pediatric hepatologist and gastroenterologist certificate renewal exams and including PFIC in the training requirements for adult hepatologists.
- Supporting existing education efforts by pharmaceutical companies.
- **Encouraging professional societies to develop clinical practice guidelines** for pediatric cholestasis and disseminate them to providers *and* patients.

Question 2 - Deciding Between Treatment Options

Patients and clinicians in the session reported treatment decisions between:

- Partial external biliary diversion versus IBAT inhibitor
- External biliary diversion versus internal biliary diversion
- IBAT + surgical biliary diversion versus liver transplant
- IBAT versus liver transplant

In most of the stories shared, the decision involved a discussion between the doctor and patient evaluating the pros and cons of each option. Some patients indicated that impact on quality of life and on family were important deciding factors.

Multiple session participants emphasized there are currently no “right or wrong” choices between treatment options in PFIC, only choices based on individual situations that ideally also consider patients’ personal values.

Ensuing conversation revealed that there are still concerning gaps in the treatment decision-making process:

- **Not all providers engage PFIC patients as partners in care decisions.** Partnering in care decisions involves having a conversation that does not just consider evidence-based clinical advice, but also talking through the potential outcomes of a treatment within the context of the patient’s personal situation and values. Some patients reported that they did not feel involved as a partner.
- **Lack of comprehensive knowledge regarding outcomes** of the disease of individual treatments. This leads to multiple challenges in decision making.
 - One parent hesitated in deciding on treatments due to uncertainty about the severity of their child's itching, struggling to gauge if it warranted taking risks with surgery compared to others' experiences.



- There has been a historical lack of accessible resources offering comprehensive information on patients' treatment experiences. Consequently, patients frequently turn to social media groups of other patients to seek advice on how treatments affect their families and their daily lives. This information, which patients deem crucial for decision-making, isn't readily available from doctors.
- **Treating PFIC is often reactive versus proactive.** Doctors find it hard to predict how quickly a patient's condition will worsen and when to change treatments. This is because the disease is not well understood, and there aren't clear clinical practice guidelines. So, decisions about treatment often happen quickly, with doctors and patients talking about the pros and cons in detail. That's why doctors say treating PFIC is more like an "art" than following a strict plan.

Participants recognized that the high level of variability in PFIC care is what prompted the PFIC Network to embark on its journey to patient-centered outcomes research. IMPACT is designed to support a shift in PFIC care from "art" to more clearly defined treatment strategies. A broader insight in patients' experiences with PFIC treatments will help design treatment research that is directly relevant to patients lives, and that will ultimately minimize uncertainty in care.

IMPACT Roundtable Session Wrap-Up

- The IMPACT Project Team, along with web developer Paolo Catalla, will work together to address TEA feedback from the roundtable session.
- Emergent themes and questions from the Roundtable session discussion will be used to design ongoing IMPACT project activities.

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

- **Our next IMPACT focus group will take place in June.**
 - Make sure you are subscribed to the PFIC Network mailing list to receive registration information!
- **Module 4 will go live on July 29th.**
 - If you haven't already, we encourage you to take [Modules 1, 2, & 3](#) before the next one releases.
- **Please continue to participate!**
 - Receive special awards if you complete every module and attend each focus group every other month.



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