



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

February Focus Group Discussion Summary

February 2024

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February Focus Group Discussion

Overview

We held our fourth IMPACT Focus Group meeting on February 20th, 2024!

- Attendees: 13 patients & parents + 5 clinicians & researchers
- This was a fully combined meeting that lasted 90 minutes.
- The goals of the meeting were to:
 - Give a final review of the IMPACT web app and user recruitment strategy before it enters the development phase, and
 - Identify key themes and questions for the Conference Roundtable IMPACT Activity.
- The feedback provided during the Focus Group discussion will be incorporated into the final web app design and conference roundtable activity taking place on April 26, 2024.

Background

As part of project IMPACT, we are building a web application that aims to educate the community on variable clinical treatment options for PFIC and capture patients' experiences with those treatments. The web application is being designed with feedback from the IMPACT focus groups. We will use data gathered through the app to identify the outcomes most important to patients and families so we can design meaningful research studies together.

We will present the beta version of the application during the Day 1 roundtable at the [PFIC Family & Scientific Conference](#). The roundtable will also serve as our next IMPACT focus group, where we will have discussion about patient-centered outcomes research priorities in PFIC. All conference attendees are encouraged to participate.

February Focus Group Key Takeaways

During the first part of the February Focus Group, we looked at the full visual concept of the IMPACT web application. Main points of feedback concerning the concept that were shared in the focus group:

1. The design is very accessible and user-friendly.
2. Add more questions to the survey related to financial burden and accessibility.
3. Include additional medications, link to pfic.org mental health resources.



We also discussed the Conference Roundtable IMPACT activity and held preliminary discussion to identify key themes and questions. Main themes that arose during the preliminary roundtable discussion:

1. We need to minimize the travel burden on participants in clinical trial design.
2. Patient-reported research priorities include finding better measures for itch and integrating outcome measures to assess the financial impact of treatments.
3. How can we use patient-centered comparative effectiveness research to solve PFIC patients' decisional dilemmas.

Discussion Summary

Web App Visual Concept Feedback

Each treatment in the web app has its own page with a lay explanation and reasons it is prescribed. Patients' experienced benefits, burdens, and impacted areas of life are summarized underneath the clinical information. Each treatment page also provides the opportunity for patient/parent users to share their experiences via survey.

Respective to each treatment, the survey asks questions regarding:

- Reason(s) the treatment was chosen.
- The direct effect of the treatment (ex. itch reduction), as well as the effect of the treatment on patient and family quality of life, and sleep quality.
- The financial burden of the treatment, accessibility of treatment in terms of travel, and the quality of the treatment delivery method.
- The personal importance of each of the above outcomes and whether there were any other benefits or burdens the patient experienced.

Focus group members shared the following feedback:

- **Consider adding questions to the survey** for country location and type of health insurance to provide more context to financial burden and accessibility questions. Resulting data could then be used in country-specific advocacy. Also add questions about mental health.
- **Explore data dissemination strategies** for uncommon ("n of 1") clinical management approaches reported in the application survey so other healthcare providers can consider/be aware of them.
- **Augment and optimize** the treatment info and survey over time based on user feedback and survey responses.
- **Add treatments**, specifically colesevelam, and include more specific names for bile acid medications.
- **Link to pfic.org mental health resources** from the web application.



- **Motivate users to share their experiences** by getting clinicians to promote the application, running raffles, and clearly showing how their survey responses will improve the application and help the greater PFIC community.

Conference Roundtable IMPACT Activity

During the [PFIC Family & Scientific Conference](#), there will be (2) two-hour roundtable sessions, one on each day. The Day 1 Roundtable will be dedicated to project IMPACT, with the goal for attending patients for researchers to discuss what should be prioritized in PFIC patient reported outcomes research.

February Focus Group members had preliminary discussion regarding the following key question we will explore in the Day 1 Roundtable: What outputs or outcomes do patients want researchers to focus on?

Several key themes emerged from the focus group, including:

- **Lowering barriers to trial participation**, including travel burdens. Members reported having to travel several hours by car to participate, even to receive basic lab services that could be done locally. This burden could affect someone's choice to participate in a trial versus receiving another treatment.
- **Incorporating patient-reported itch measures**. Multiple members reported experiencing high levels of itchiness despite having healthy labs, which resulted in medical gaslighting. One member said this discrepancy between itch and labs almost served as a barrier for getting into a clinical trial. The group also emphasized how itch is even harder to measure in young children who cannot effectively express their discomfort.
- **Measuring financial burdens** of the disease and of specific treatments is important to incorporate in future PFIC research. Focus group members identified a variety of disease-related costs, including special clothing, increased use of air conditioning, creams and non-prescription medicines, and loss of income. Members noted that treatment-specific financial burdens, like caregiving time and transplant-related costs, would be important to explore.
- **Mental health is an important outcome measure to consider in research**. Focus group members identified many ways the disease and different treatments can impact mental health, such as anxiety about the long-term outcomes of a new treatment, impact on siblings, and impact on sleep. Focus group members also emphasized that the mental health toll of trial participation is commonly underestimated.



Focus group members also discussed the dilemmas patients are facing in their treatment decisions, and how we can use patient-centered outcomes research to alleviate them. Members raised questions like...

- Can we use patient-centered comparative effectiveness research to explore:
 - Whether it would be more beneficial to choose a diversion surgery versus an IBAT inhibitor or liver transplant?
 - If you had diversion surgery that's no longer working, whether it would make sense to still try an IBAT inhibitor?
 - What are the comparative benefits and risks of the two IBAT inhibitor medications use to treat PFIC?
- How big of a sample size do you need to conduct a patient-centered comparative effectiveness research study?
- How do you determine which PFIC subtype to investigate?

February Focus Group Wrap-Up

- The IMPACT Project Team will work together to incorporate feedback from the February Focus Group into the web application.
- Emergent themes and questions from the February Focus Group discussion will be used by facilitators Jim Squires and Alex Perez to help guide the conference roundtable activity.

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

- **Module 3 will go live on April 1st.**
 - Look out for emails from Melissa with an invitation! If you haven't already, we encourage you to take [Modules 1 & 2](#).
- **Our next focus group will take place as a Roundtable at the PFIC Family & Scientific Conference!**
 - We look forward to our first in-person IMPACT activity in Cincinnati this April! If you would like to participate (whether in-person or virtually), make sure you [register to attend the Conference!](#)
- **Please continue to participate!**
 - Receive special awards if you complete every module and attend each bi-monthly focus group.

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