Identifying research targets by **M**erging **Pa**tient And **C**linician **T**reatment information

PFIC IMPACT: April Focus Group Discussion Summary

Overview

We held our tenth and final PFIC IMPACT Focus Group meeting on April 22nd, 2025!

- Attendees: 10 patients & parents + 4 clinicians & researchers
- Our objectives for the meeting were to identify:
    - which stakeholders should be involved in future research teams, and
    - what type of support stakeholders will need to:
        - work together as equal partners, and
        - successfully execute all stages of a research project.

Background

Project IMPACT, inspired by discussions at PFIC Network's 2022 Conference, aims to prepare the PFIC community for patient-centered Comparative Effectiveness Research (CER). Patient-centered CER compares treatments based on patient-prioritized outcomes, such as itch reduction, family quality of life, and financial burden – and involves patients as active partners throughout the entire research process, from study design to implementation and dissemination. To lay the groundwork for these partnerships, we've spent the past two years gathering input from PFIC patients, parents, clinicians, and researchers to develop practical guidance for building effective and sustainable patient-researcher teams.

Discussion Summary

Key Stakeholder Partners for PFIC Research Teams

During the April focus group, participants discussed the importance of including a wide range of voices on the research team for a patient-centered CER project to ensure the full spectrum of experiences, expertise, and needs are represented throughout the research process. They recommended forming a consistent core team that includes:

- **Patients** to represent diverse lived experiences across ages, disease stages, and geographies, including adolescents, adults, and post-transplant individuals. Younger patients could be engaged through Child Life Specialist-led sessions or teen advisory boards.
- **Parents** to provide critical caregiver perspectives on the daily impacts of PFIC and experiences with clinical care.
- **Clinicians** from both specialty centers and general hospitals to offer insights into diagnosis, treatment practices, and care delivery challenges.

- **Researchers (clinical and lab)**: Clinical researchers have the experience to carry out studies with patient participants and can help ensure that research questions are clinically feasible and meaningful. Lab researchers have the experience to study biological phenomena in a controlled setting and can use patient-centered CER results to identify new biological questions or mechanisms to investigate through lab research.
- **Statistician** to ensure rigorous study design, data collection and analysis, and reporting to support credible, high-quality findings.

Participants agreed that maintaining a consistent core team - supported by rotating members based on project needs - can help balance continuity with the diverse expertise required across all stages of a research project. Participants recommended involving rotating members whose expertise is relevant to specific stages of the project, including:

- **Psychologist** to support patient-centered engagement and address emotional or behavioral aspects of research participation.
- **Child Life Specialist** to help capture pediatric patient voices through developmentally appropriate methods such as art, play, or storytelling.
- **Research Assistant** to assist with study coordination, participant communication, and research logistics as well as to support data collection.
- **Communications Specialist** to support dissemination of research findings through accessible formats, creative outreach, and broader public engagement beyond traditional academic channels.

Support for Research Teams

Participants identified several types of support needed to ensure that all stakeholders can work together as equal partners:

- **Flexible participation options**
  - offer multiple ways to engage - such as core team roles, short-term advisory roles, or rotating participation
  - allow input asynchronously and schedule meetings at times that accommodate multiple time zones
- **Clear expectations and structured tasks**
  - define roles, responsibilities, and timelines early
  - break tasks into manageable parts to support participation
- **Training on research processes and terms**
  - provide plain-language written guides
  - share brief (under 10 minute) explainer videos
  - offer 1:1 mentorship if possible

- **Opportunities to meet in person**
    - plan at least one in-person meeting early to build trust, strengthen communication, and support collaboration
- **Support for pediatric engagement**
    - given that a significant proportion of PFIC research participants will be children and adolescents, use pediatric-specific strategies to ensure meaningful input from younger patients, including:
        - child life specialist-led sessions
        - play-based methods
        - teen advisory boards
- **Compensation for time and effort**
    - offer financial compensation to recognize time contributions and reduce barriers to participation

Participants emphasized that building flexibility into the engagement structure is critical, as some studies may span several years, and participants' availability and capacity may change over time. To sustain engagement, participants recommended:

- **maintaining a consistent core group** of committed members to provide continuity across all research stages
- **creating flexible committees or advisory groups** comprising patients, families, clinicians, and other stakeholders to bring in new voices as needed based on the phase of the project
- **providing ongoing onboarding and clear communication** to support both core members and new contributors, making participation approachable at every stage
- **fostering connection and community:** feeling connected to others throughout the research project helps create a sense of support and belonging, which encourages ongoing engagement over time

Communications & Dissemination

Participants described a consistent experience of being asked to participate in research without receiving updates or results afterward unless they actively searched for them. This lack of follow-up was seen as a major gap in how research is communicated back to patients and their families. They expressed a strong desire for more accessible, timely, and understandable communication. Suggestions included:

- receiving study results in plain language with limited medical jargon
- using formats like visual abstracts, pictograms, or short explainer videos (ideally under 10 minutes, with chapters or segments)

- having a consistent point of contact from the research team to follow up with results and be available to answer any questions
- offering a centralized location - such as a section on the PFIC Network website - where updates and results could be posted in an easy-to-read format

While these suggestions were shared in the context of receiving research results and creating a study dissemination plan, participants also highlighted broader considerations for how PFIC Network communicates its research-related initiatives to the community. For example, when sharing updates about IMPACT or the Patient Registry, it may be beneficial to apply similar strategies - such as using concise, visually guided summaries and offering a centralized place for families to access information. These insights can also inform the development of training materials for future patient-researcher team members, helping ensure that content is accessible, engaging, and easy to understand.

Focus Group Wrap-Up & Next Steps

- The IMPACT project team will synthesize feedback from Module 6 and the April focus group to develop practical guidance for building effective multistakeholder research teams in future PFIC patient-centered CER projects.
- This guidance will be featured in the IMPACT Roadmap - the main resource that brings together all the key outcomes of the two-year project!

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

Make sure you're subscribed to PFIC Network's mailing list to receive alerts and access links for upcoming activities:

- **Be on the lookout for the draft IMPACT Roadmap!** We'll be sharing it soon via email and inviting your feedback to help shape the final version. 😊
- **We will conclude Project IMPACT with an in-person activity** during the Family Conference breakout on Friday, June 27, at the 2025 Cholestatic Liver Disease (CLD) Summit in Aurora, Colorado. We hope to see you there!

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