



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

PFIC IMPACT: June Focus Group Discussion Summary

Overview

We held our fifth PFIC IMPACT Focus Group meeting on June 20th, 2024!

- Attendees: 10 patients & parents + 3 clinicians & researchers
- The goals of the meeting were to:
 - Learn more about comparative effectiveness research (CER).
 - Start to think about what CER projects in PFIC should look like.

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 June Focus Group, it became clear that setting up a CER study in PFIC is not easy! Treatment choices can vary a lot across different diagnoses ("every patient is unique"). The first step is to find a consensus on which treatments to compare and the second step is to decide which patient-centered outcomes are relevant for the chosen treatments to include in the CER study. Moving forward, we aim to prioritize a list of PFIC CER research questions by July 2025 via the PFIC IMPACT Treatment Experience App and the upcoming focus group meetings.

Discussion Summary

Our main goal for the June Focus Group was to better understand how CER could work for PFIC: what treatments can we compare, and outcomes should we consider when comparing the treatments? We learned that we should look at these two questions together; for instance, financial burden can be a very important outcome but not so much when comparing two treatments that have a similar price tag.

We also reviewed examples of treatments and outcomes compared in CER studies for rare diseases [Myasthenia Gravis](#) and [Eosinophilic Esophagitis](#), and continued previous discussions on what treatments and outcomes would be applicable in a PFIC study. June focus group members identified the following considerations in evaluating which outcomes and treatments to investigate for PFIC:



- **Patient-centered outcomes can be measured!** Itch, sleep, quality of life, and mental health were identified in previous focus groups as important outcomes. These outcomes can be measured reliably using validated questionnaires such as the PROMIS scales that we use in the patient registry.
- **Outcomes must be measurable in a reasonable timeframe.** For example, native liver survival is a crucial clinical outcome but can take years to measure.
- **Treatments and outcomes may vary between diagnoses & transplant status.** We must decide as a community whether to prioritize studies focused on a specific PFIC diagnosis such as FIC1 deficiency (formerly known as PFIC 1) or choose treatments and outcomes relevant across all subtypes and stages, such as nutrition, access to care, and psychosocial support.
- **Not all treatments are feasible to compare.** For example, participants in past modules and focus groups have proposed studying the differences between the two available IBAT inhibitors (i.e., maralixibat and odevixibat). Both treatments are increasingly common in PFIC care. However, conducting a comparative study is challenging because the clinical trials for these two medications have shown very similar success rates. We would therefore need very large participation numbers to detect meaningful differences—a difficulty exacerbated by PFIC's rare and limited population.
- **Variability in care approaches can potentially reveal which treatments to compare.** Focus group members discussed ways PFIC patients' experiences with care have varied between centers, including:
 - Time to diagnosis
 - Nutrition monitoring intervals and interventions
 - Quality of care at local providers vs. large specialty centers or telehealth
 - Transition from pediatric to adult care

June Focus Group Wrap-Up

- Over the next year, we will continue compiling a list of which treatments should be compared, and what outcomes are most important to look at.
- Our goal is to winnow down that list to 1-2 priorities by July 2025, recognizing we may end up still having some learning and consensus-building to do!
- **Help us decide!** Please visit the [PFIC IMPACT Treatment Experience App \(TEA\)](#) and share your treatment experiences in the TEA survey. The results from the survey will help us to better understand which treatments and outcomes are most relevant and as representative as possible of our community's lived experiences.





- The IMPACT Project Team will summarize and share back results from the IMPACT TEA survey in future focus groups to reveal meaningful CER targets.

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

- **Module 4 will go live on Monday, July 29th.**
 - Look out for emails from Melissa with an invitation!
 - If you haven't already, we encourage you to take [Modules 1, 2, and 3](#).
- **Our next focus group will take place on Tuesday, August 20th 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](#).

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