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

# PFIC IMPACT: October Focus Group Discussion Summary

## Overview

We held our seventh PFIC IMPACT Focus Group meeting on October 22nd, 2024!

- Attendees: 12 patients & parents + 3 clinicians & researchers
- Our goal for the meeting was to understand what factors need to be considered at each stage when setting up a CER study to compare PFIC treatments.

## 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 October Focus Group, we did an exercise where we set up a mock CER study comparing nutrition interventions. We discussed which interventions to compare, identified meaningful outcomes and measures, and strategies for study design and enrollment. Members highlighted the importance of measuring itch, quality of life, palatability and acceptability when assessing nutrition interventions. We also explored ways to reduce participation barriers, like minimizing blood draws and offering child-friendly incentives.

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## Discussion Summary

In previous module surveys and focus groups, IMPACT members highlighted a range of treatments and outcomes they believe would be meaningful to compare in a PFIC CER study. In our most recent focus group, we explored how to design a mock study focused on comparing nutrition interventions, given that nutrition management was often cited by participants as a source of variability in care across the PFIC subtypes. During the discussion, we identified important aspects and potential challenges for each stage of the study design process:

**Choosing Comparators**

Focus Group members were asked about their experience managing nutrition with their care teams to reveal which interventions would be important to compare.

- **Aggressive vs. passive nutritional management.** Members shared varied experiences in managing malnutrition, ranging from gradual (e.g., oral formula) to more aggressive approaches like nasogastric (NG) tubes or Total Parenteral Nutrition (TPN). Comparing these approaches may help identify the best management strategies based on outcomes that matter most to patients.
- **Care with a Registered Dietitian vs. Without.** Some focus group members reported receiving support from a dietitian as part of their care and others not. Comparing outcomes between these two approaches could help highlight the impact of dietitian involvement on patient well-being and nutritional success.
- **High fat vs. low fat diet.** Some focus group members have observed gastrointestinal upset and increased itchiness after consuming high fat foods, showing that we also want to collect data on general nutrition (high fat vs low fat) if we decide to do a nutrition study after project IMPACT.

**Defining Outcomes & Measures**

*Outcomes* measure change that is observed because of a treatment or intervention. Outcomes are how we will measure differences between treatments in a CER study. Focus group members highlighted the following considerations for selecting outcomes in a PFIC CER study on nutrition:

- **It is important to measure multiple outcomes** because a single outcome does not always paint the full picture. For instance, weight gain may indicate improved nutritional health, yet vitamin levels could remain low.
- **Palatability and acceptability are important issues** facing patients in nutrition management that should be considered as potential study outcomes. For example, focus group members shared the discomfort and challenges their children experienced with a nasogastric (NG) tube, including repeatedly pulling it out and needing it reinserted.
- **Itch is a relevant outcome to include in a nutrition study.** Some focus group members reported experiencing an inverse relationship between their appetite and itch levels, highlighting the impact that increased itchiness can have on a patient's nutrition status.

Once we have determined what outcomes are important for comparing treatments in our study, we must decide how we will measure those outcomes. For example, if we choose weight gain as an outcome, we will need to decide: where will we measure the patient's weight– in clinic, or at home? How frequently?

Focus group members emphasized that patient preferences are crucial to consider when selecting outcome measures and cited frequent blood drawings as an example of a *less* acceptable measure. Outcome measures can also be influenced by study design.

**Choosing a Study Design**

To design a CER study, we will have to decide what research methods we will use. In the focus group, we discussed two main types of study design: observational studies and clinical trials. Observational studies* observe existing differences in real-world care and capture outcomes based on what patients are already experiencing.

*If we conduct an observational study, we will have to decide our outcome measures based on what data is already being collected through current standards of practice (i.e., how often patients are already going into the clinic and receiving certain tests).

Clinical trials are more structured: they assign specific treatments or interventions– like comparing NG tube versus no NG tube– and enroll patients into defined study groups based on their assigned treatment. Clinical trials provide more flexibility in choosing outcome measures but are often more costly than observational studies.

**Determining Enrollment Numbers**

To design our CER study, we will need to work with a statistician to determine the number of participants (i.e., sample size) we need to enroll to generate statistically meaningful results. One way we can determine the necessary sample size is to conduct a *pilot study*, which can help assess the magnitude of the difference between treatments we choose to compare, and guide whether a larger sample (e.g., 20 vs. 60 participants) is needed to identify a statistically significant effect.

A pilot study is a small, preliminary study conducted to test the feasibility, design, and methods of a larger research project. It helps researchers identify potential issues and refine their approach before committing to a full-scale (and far more costly!) study.

**Minimizing Barriers to Enrollment**

Focus group members discussed what would motivate them to participate in a CER study on nutrition, and highlighted the following key elements:

- **Meaningful results.** Focus group members shared that the most important factor is knowing the results could improve their or their child's life.
- **Reduced hospital visits.** Provide services at home or at local clinics.
- **Minimizing medical trauma.** Require as little follow-up as possible. Incorporate less invasive outcome measures, like painless blood draw devices.
- **Compassionate care from trial nurses.** Focus group members reported that small, personal acts of kindness from their trial nurse made a big difference.
- **Compensation** for adult patients and parents, also to recognize children for their participation. Compensation for kids can take other forms besides money, such as gift cards or zoo passes.
- **Toys** for kids, to create a more positive experience.
- **Therapy dogs** to help support patients through traumatic medical events.

## October Focus Group Wrap-Up

- We will continue to identify unanswered questions regarding PFIC treatments with the aim to conduct a first CER study after IMPACT finished in June 2025.
- During our next focus group, we will review data from the TEA to guide our discussion on which treatments and outcomes are relevant to compare.

*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…

- **Module 5 will go live in early December .**
  - Look out for emails from Melissa with an invitation!
  - If you haven't already, we encourage you to take Modules 1, 2, 3, & 4.
- **Our next focus group will take place on Wednesday, December 18th 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