



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

Module 3 Survey Results

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Module 3

Overview

- IMPACT Module 3 launched on April 1st, 2024.
- N=22 participants completed the survey that followed Module 3:
 - 17 patients/parents
 - 5 clinician/researchers
- Module 3 provided education about patient-centered comparative effectiveness research, and how to design research questions that lead to answers that matter to PFIC patients and families.
- Module 3 used materials published by PCORI, which you can check out here: <https://www.pcori.org/engagement/research-fundamentals>
- The education section was followed by a survey to assess PFIC patients and providers' priorities in treatment research. The results are summarized below.

Module 3 Survey Results Overview

The main points concerning PFIC treatment experiences and research priorities that were shared in the survey by module participants:

1. When considering treatments, patients prioritize understanding the benefits and risks, with emphasis on mitigating life-threatening complications. Patients consider itch relief and improving quality of life as key treatment outcomes.
2. Clinicians value open communication with patients and preventing liver disease progression as key care outcomes. Clinicians reported that treatment costs, lack of care standards, and variability in provider knowledge can impact care consistency. Proposed solutions to improve care consistency include provider education and clinical care guideline development.
3. High priority was given to focusing a comparative effectiveness study on itch, for instance to compare different surgeries (internal vs external diversion vs liver transplant) or different medications. Impact on quality of life was mentioned as an important outcome to include in such a comparison.

Module 3 Survey Results - Part 1

In the first part of the Module 3 survey, we asked questions about participants' treatment experiences. Patients/caretaker participants and clinician/researcher participants received slightly different surveys. Here is a summary of the patients/caretaker responses to each of the questions:



Q1: When you and your provider are considering a PFIC treatment - a new medication for example, or a surgery such as liver transplant - what are the most important things for you to know about the treatment?

- Benefits and risks (including potential harms)
- Shorten and long-term side effects
- Safety and efficacy data
- Impact on quality of life
- Invasiveness
- Cost
- Experiences of other patients/families

Q2: What are the most concerning risks/harms of the treatments that you or your child have received? (Please note that answers are NOT ordered.)

- Potential for life threatening complications
- Stomach pain, diarrhea, malnutrition
- Developmental delays
- Complications post-surgery
- Unknown long-term effects
- Medical trauma
- Elevated cancer risk

Q3: What are the most important outcomes of treatments that you or your child have received?

- Itch relief
- Improved quality of life
- Improved sleep quality
- Slow/halt disease progression
- Weight gain

Here is a summary of the clinician/researcher responses to each of the questions:

Q1: In your experience as a clinician or researcher, what are the most important outcomes that need to be achieved right after a PFIC diagnosis?

- Education and clear communication with the patient/family
- Genetic counseling
- Addressing vitamin and nutritional deficiencies
- Screening for advanced liver disease
- Managing pruritus



- Preventing liver failure and avoiding transplant
- Improving quality of life

Q2: What are the most concerning risks/harms of the treatments that you recommend or provide to PFIC patients directly after their diagnosis?

- Diarrhea
- Bad taste of medicine/supplements
- Vitamin deficiencies
- Treatment doesn't work, risking disease progression

Q3: What do you think are the main obstacles to reducing variability in care in PFIC?

- Cost and accessibility of treatments and surgeries
- Lack of standard care practices for PFIC
- Lack of designated PFIC "centers of excellence"
- Variability in provider knowledge of PFIC subtypes
- Lack of communication between providers at different centers

Q4: How do you think these obstacles can be overcome?

- Provider education on different PFIC disorders and specific issues
- Consensus and development of clinical guidelines by professional societies
- Develop realistic treatment guidance for under resourced care centers
- Patient education and advocacy

Module 3 Survey Results - Part 2

The second part of the Module 3 survey asked questions about patient-centered outcomes research priorities.

Background

Most PCOR studies are comparative effectiveness research (CER) studies - they compare the effect of 2 known treatments on patient-centered outcomes. The IMPACT team is exploring priority areas for CER studies in PFIC. In the Module 3 survey we asked participants to choose 2 out of 3 priority areas for research originally identified through IMPACT focus groups that they thought were most important. The three priority areas provided that were identified in the focus groups were:

1. Focusing on the impact of surgeries on patient and family quality of life
2. Comparing different itch treatments with respect to effectiveness and burden
3. Comparing the economic impact of different treatments.



See the table below for participant responses to priority areas in CER for PFIC:

Priority Area	Clinicians	Patients
Impact surgeries on QoL	0.67	0.83
Comparing itch treatments	1	0.87
Comparing econ impact	0.33	0.17

Note: Numbers are proportions (i.e., multiplied by 100 would be percentages)

In follow-up, we asked participants if there were any research priority areas not listed that would also be important to consider.

Patient/parent participants reported:

- Potential future therapies
- Efficacy of alternative/homeopathic itch treatments
- Comparing the effectiveness of IBATs by PFIC subtype
- Comparing treatments by impact on family
- Finding the mechanism for itch

Clinician/researcher participants reported:

- Potential future therapies
- Post-transplant outcomes and management approaches
- Comparing outcomes of external biliary diversion surgery to internal biliary diversion surgery
- Nutrition management

Because CER studies compare the effect of 2 known treatments on patient-centered outcomes, we also asked participants to identify which PFIC treatments they consider most feasible to compare:

Patient/parent participants reported:

- Odevixibat vs. Maralixibat
- Biliary diversion surgery vs. IBAT Inhibitor
- External biliary diversion vs. internal biliary diversion
- Biliary diversion vs. liver transplant
- Non-IBAT anti-pruritic medication vs. biliary diversion surgery or transplant
- Behavioral intervention for itch vs. no behavioral intervention

Clinician/researcher participants reported:

- Odevixibat vs. Maralixibat



- Biliary diversion surgery vs. IBAT Inhibitor
- Biliary diversion vs. liver transplant
- Various anti-pruritic medications (IBAT and non-IBAT)