



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


PFIC IMPACT: Module 5 Results Summary

On December 3rd, 2024, we launched IMPACT Module 5. A total of 36 participants - 31 patients and parents, alongside 5 clinicians and researchers - completed the module and accompanying survey! The intention of Module 5 was to expand the information collected in the Treatment Experience App ([TEA](#)) and obtain **exhaustive lists** of:

- (1) treatment-related questions
- (2) treatment outcomes that matter most to the PFIC community.

Thank you to everyone who participated in Module 5. Your answers in this module were essential as they will shape the development of PFIC Network's patient-centered research agenda!

Treatment-related Questions

TREATMENT/INTERVENTION TYPE	QUESTIONS
 <p>Medications</p>	<ul style="list-style-type: none"> • <u>IBAT-related medications</u> <ul style="list-style-type: none"> ○ How do the different non-IBAT medications compare in reducing itch? ○ How does combining IBAT inhibitors with other medications compare to using IBAT inhibitors alone for reducing itch, gastrointestinal symptoms, and improving quality of life? ○ What are the short and long-term side effects of IBAT inhibitors versus diversion surgery? • <u>Non-IBAT medications</u> <ul style="list-style-type: none"> ○ How do alternative therapies like cold baths or massage impact itching and quality of life? ○ What is the best medication to reduce PFIC-related fatigue? ○ What is the best way to manage the psychological effects of PFIC on patients and families during any stage of the disease? ○ How does the mental health of PFIC patients receiving psychological intervention compare with those who receive none? • <u>Liver transplant & post-transplant medications</u> <ul style="list-style-type: none"> ○ Which is more effective at reducing post-transplant pruritus: IBAT inhibitors, non-IBAT medications, or other treatments? ○ What is the comparative effectiveness of antidiarrheals, IBAT inhibitors, and diversion surgery in reducing post-transplant diarrhea? ○ How do the short and long-term side effects of post-transplant medications compare to pre-transplant medications?




Surgeries

- Biliary diversion
 - How does the likelihood of benefit from internal versus external diversion vary among different PFIC types?
 - What will work better to relieve itch — internal or external biliary diversion?
 - What ostomy supplies have worked best with an external biliary diversion?
- Surgeries and IBATs
 - Which delays the need for transplant longer: diversion surgery or IBAT inhibitors?
 - How does the financial burden of surgical diversion or transplant compare to the financial burden of being prescribed an IBAT inhibitor long-term?
 - How and when do you most effectively reverse an external or internal diversion to solely using IBAT inhibitor?
- Liver transplant
 - What are the best criteria to determine the necessity and urgency of liver transplant?
 - When is the optimal time to pursue liver transplantation?
 - How does a live donor liver transplant compare to a deceased donor liver transplant when it comes to care, longevity, and quality of life after transplant?
 - How do growth and quality of life compare pre- and post- transplant?
- Surgeries - general
 - How do different surgeries (external or internal diversion, and liver transplant) compare in improving the quality of life for PFIC patients and their families?



Nutrition

- Vitamins & Supplements
 - What types of OTC supplements best support growth and development?
 - How do aggressive nutritional treatments (such as NG tubes or TPN) compare to more passive approaches (like oral formulas) in terms of tolerability, as well as their impact on weight, growth, and quality of life?
 - How does regular versus infrequent monitoring of vitamin levels impact health and quality of life outcomes related to vitamin deficiencies?
- Diet
 - Which is better: formula or blended food?
 - How does an MCT-enhanced diet impact growth versus a non- MCT enhanced diet?
 - How does a diet high in processed foods, sugars, fats, and starches compare with one containing fewer of these elements in terms of its impact on pruritus, gastrointestinal symptoms, quality of life, and liver health?

	<ul style="list-style-type: none"> • <u>Post-transplant</u> <ul style="list-style-type: none"> ○ How do antidiarrheal medications (like loperamide) compare to biliary diversion surgery in reducing diarrhea after liver transplant?
 <p>Diagnosis, delivery of care, & other support</p>	<ul style="list-style-type: none"> • <u>Diagnosis</u> <ul style="list-style-type: none"> ○ What is the best approach to reduce the time to diagnosis for PFIC? ○ How does one measure of itch compare to another (e.g. inpatient surveys, patient-reported outcomes, monitoring with a daily app, wearables)? • <u>Delivery of care</u> <ul style="list-style-type: none"> ○ What is the comparative effectiveness of pain management intervention (e.g., EMLA cream or gas protocol for needle insertion) versus no pain management intervention in improving patients' mental health? ○ What is the comparative effectiveness of having a dietician in the care team versus no dietician in improving nutrition and quality of life outcomes for PFIC patients? • <u>Other support</u> <ul style="list-style-type: none"> ○ What is the best way to make the process of insurance approvals for medications easier and faster? ○ What is the best way to educate my providers and specialists about PFIC? ○ What is the best way to educate my child's school personnel about PFIC? ○ What is the best way to improve the transition from pediatric to adult care? ○ What is the best supplemental insurance to help cover costs associated with PFIC?

Treatment Outcomes

OUTCOME TYPE	OUTCOMES
<p>Patient-Reported</p>	<ul style="list-style-type: none"> • <u>Medications-specific</u> <ul style="list-style-type: none"> ○ Itch relief, skin health, sleep quality, reduction of pain, reduction of fatigue, reduction of diarrhea, reduced frequency and severity of episodes • <u>Surgeries-specific</u> <ul style="list-style-type: none"> ○ Itch relief, reduced abdominal pain/discomfort, improved social acceptance (e.g., concerning ostomy bags, growth delays), reduction of diarrhea, improved toilet training • <u>Vitamins/Nutrition-specific</u> <ul style="list-style-type: none"> ○ Improved palatability (e.g. treatment taste, ease of consuming quantity) • <u>Cross-intervention</u>

	<ul style="list-style-type: none"> ○ Improved patient quality of life, improved quality of life for caregivers/family/siblings, improved mental health (reduced anxiety/depression/irritability), improved focus and school performance, improved ability to enjoy major life events, play and swim ● <u>Care Delivery & Support-specific</u> <ul style="list-style-type: none"> ○ Reduced number of workdays missed for care, lowered financial burden of the disease and treatments, impact of delayed insurance approvals on quality of life, impact of switching insurance companies on treatment access, improved ease of acquiring compounded medications, trust in provider/healthcare system, improved testing for cancers
Clinical	<ul style="list-style-type: none"> ● <u>Medications-specific</u> <ul style="list-style-type: none"> ○ Slowed/halted disease progression, native liver survival ● <u>Surgeries-specific</u> <ul style="list-style-type: none"> ○ Native liver survival, reduced post-transplant complications ● <u>Vitamins/Nutrition-specific</u> <ul style="list-style-type: none"> ○ Improved vitamin levels, weight gain, improved growth (height), improved strength, reduction/prevention of hearing loss

Preferences regarding study design

In earlier IMPACT modules and focus groups, we talked about how the relevance of treatments and outcomes to investigate in a PC-CER study can vary depending on factors like PFIC subtype, transplant status, or other individual circumstances. We also explored study ideas that could apply to (almost) everyone, such as comparing itch treatments or nutrition interventions. Module 5 asked patients and providers whether they would prefer a first research study that addresses questions for the whole PFIC community, or one focused on a specific subtype, post-transplant care, or age group:

- **14** participants responded that a first study should be relevant for all PFIC patients.
- **10** participants responded that a first study can focus on a specific group of patients (e.g., subtype, or age, or pre or post LT)
- **13** participants indicated they have no preference

Strategies to reduce the burden of research participation

In IMPACT focus groups, we brainstormed a list of ways to design a PC-CER study that would encourage enough patients and families to participate. To expand this list, Module 5 first asked patients and providers what would motivate them and reduce the burden to participate in a study comparing PFIC treatments. Participants responded:

- ✓ A strong recommendation from a trusted physician
- ✓ Services offered at home or through local clinics

- ✓ Minimal number of hospital visits required
- ✓ Minimal medical trauma anticipated
- ✓ Limited time required away from family
- ✓ Mental health support
- ✓ Financial compensation
- ✓ A dedicated case manager/care coordinator
- ✓ A communication specialist trained to answer questions for the trial
- ✓ Frequent updates on study progress, changes, and results
- ✓ A transparent plan for dissemination of study results to improve care
- ✓ Setting clear expectations
- ✓ Shorter visits
- ✓ No blood samples in addition to routine draws
- ✓ Toys or presents for child participants
- ✓ Flexible timing of visits
- ✓ Scheduling visits during breaks from school to minimize disruption
- ✓ Avoiding invasive procedures (e.g. by using surveys)
- ✓ Personalized reminders
- ✓ Warm, welcoming, and patient research staff
- ✓ Support for smaller centers to enroll study participants

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