



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

December Focus Group Discussion Summary

December 2023

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December Focus Group Discussion

Overview

We held our third IMPACT Focus Group meeting on December 20th, 2023!

- Attendees: 8 patients & parents + 5 clinicians & researchers
- This was a fully combined meeting that lasted 90 minutes.
- The goal of the meeting was to discuss IMPACT Module 2 survey responses about partnership and how to apply them in future patient-centered outcomes research projects.
- The [results from the Module 2 survey](#) and the feedback provided during the Focus Group discussion will be used to create a “checklist” that shows all important issues to keep in mind when setting up a research project.

Background

In IMPACT [Modules 1 & 2](#), we learned the fundamentals of Patient-Centered Outcomes Research, and the different ways patients can serve as partners on research teams.

“The term ‘research partner’, includes individuals who have experience living with, caring for, advocating for, and/or treating those with a condition. These individuals are included on research teams to share their knowledge and perspectives to ultimately enhance the relevance and use of the research.”

PCORI's Stakeholders

Patient Centered Outcomes Research Institute

The goal of Project IMPACT is to build a solid foundation for partnerships between patients, parents, clinicians, and researchers to jointly carry out future patient centered outcomes research in PFIC. The Module 2 survey and the December Focus Group had the same objective: understanding what the ingredients to an ideal partnership between PFIC patients and researchers are.

December Focus Group Key Takeaways

During the December Focus Group, we reflected on results from Module 2 as a starting point for discussion about what partnership could look like in future research projects. You can check out the [results of the Module 2 survey here](#).

We also identified some barriers to partnership in future research and potential solutions so that we can begin to find ways to address them together.



Main points concerning partnerships that were shared in the focus group:

1. Research teams should prioritize research questions that are directly meaningful to patients and should ensure effective communication of study information and results to partners and participants.
2. Patients and parents face barriers to participating in research such as long-distance travel, lack of research knowledge, and lengthy consent forms.
3. Clinicians and researchers encounter barriers to partnering in PFIC patient-centered outcomes research (PCOR) projects like insufficient funding and lack of PCOR awareness.

Discussion Summary

Research Partnership Ideals

- **Relevance.** It is important to consider questions that are directly meaningful to patients.
- **Transparency.** Patients participating in a study need clear and straightforward information about the names, roles, and funding sources of all members of a research team.
- **Communication.** Lab or study results need to be communicated, and participants need to have an opportunity to ask questions. For some, having the opportunity to discuss results face-to-face in-person can be very important.
- **Remuneration.** Financial compensation to patients for participation in research teams is critically important, as well as having the knowledge they're making a meaningful difference with their time and input.

Patients & Parents - Barriers to Partnership & Research Participation

- **Travel burden.** Some patients must travel significant distances to engage in research studies conducted at major centers, and this travel can occasionally lead to adverse psychological effects, particularly in children.
 - Suggested solution: Bring the trial to local communities (ex. train local providers to conduct services and/or research protocol locally) so the patient does not have to travel to participate or receive services.
- **Advocacy awareness.** When patients do not advocate or ask questions (e.g., because they are less assertive or have a language barrier) they can be left without information that should've been initially provided.
 - Suggested solution: Educate patients and parents early on about:
 - What types of questions can and cannot be answered in a research study and why.



- The role of the research coordinator, and why they may need to relay questions to the clinician.
- The different types and phases of clinical trials (ex. double blind), where the current study is and what to expect.

Different modalities of education should be developed to meet the different individual learning styles of patients and families (i.e. reading materials, videos, face-to-face conversation, webinar + Q&A).

- **Outcome metrics.** Inaccuracies in the tools/metrics employed to assess individual experiences with pruritus pose a concern when treatment decisions are based on data from these instruments.
 - 💡 Suggested solution: Develop tools/metrics for things like itch that more accurately capture individual patients' experiences. Find a balance between individual accuracy with quantifiability and scalability.
 - Consider using other research techniques like "free listing" or leverage AI to identify common themes in individual stories.
- **Consent Forms.** Consent forms can be excessively long and difficult to understand, proving overwhelming for patients already burdened by disease. Furthermore, clinicians have limited time during clinic visits to thoroughly discuss these forms with patients or their parents.
 - 💡 Suggested solution: Consent forms should be written in lay language so that they are easy to understand. Focus group members proposed:
 - Looking at the assent form when possible, since it is written for adolescents.
 - Turning the consent form into a visual or video, with the opportunity to ask the researcher questions after watching.
 - Encouraging patients to read over the paperwork and come up with a list of questions before their visit to speed things up.
 - Also, members expressed that it is better when a patient is approached by someone familiar from their care team to enroll in a research study before being transferred to a research coordinator.
- **Availability.** Not all patients are available or interested in participating on a research team. The time and energy it takes to participate can conflict with other life priorities not limited to managing the burdens of the disease.
- **Diversity.** It is difficult to capture the full range of voices and experiences in the patient community. Language barriers, historical mistrust of healthcare institutions, and economic barriers are just some of the factors that can influence the ability of PFIC patients to participate in research.



Clinicians/Researchers - Barriers to Partnership

- **Lack of funding.** A lack of funding can impact the level at which clinicians and researchers engage with patients. They often don't have enough time to field all of a patients' questions about the study and consent form.
- **Unclear value or output.** Some researchers might not see the value or output of Patient-Centered Outcomes Research clearly enough. Before they pursue this type of research project, researchers want to know whether it can result in a publication, or a valuable idea for other researchers to pursue in the future.
- **Research in small populations.** It is more complicated to understand how to do PCOR effectively in a smaller population like PFIC.
 - 💡 Suggested solution: We are hoping we can come up with a direction to move forward with through IMPACT. If we can figure out how to do it effectively, we can potentially bring this data to regulatory agencies to incorporate into trial design for future treatment studies.

December Focus Group Wrap-Up

- While focus group members expressed interest in participating in a research team (whether in designing a study, obtaining the funding, or conducting the study), it was acknowledged that there may also be many PFIC families and researchers that are not interested.
- Focus group members established that lowering the barriers to research participation for patients while increasing community awareness of patient-centered research will make it a more meaningful method of investigation.

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

- **Our next focus group will take place in February.**
 - Joint meeting with clinicians and researchers on weekday evening TBA.
- **Module 3 will go live in April.**
 - Look out for emails from Melissa with an invitation! If you haven't already, we encourage you to take [Modules 1 & 2](#).
- **Please continue to participate!**
 - Receive special awards if you complete every module and attend each bi-monthly focus group.

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



BARRIERS AND SOLUTIONS TO PARTNERSHIP IN PFIC PCOR PROJECTS

BARRIERS	DESCRIPTION	SOLUTIONS
Time Constraints & Burden of Disease	Patients & parents must schedule around work, hospital visits, and school; providers & researchers must balance high work demands.	Schedule project activities in alignment with availability; send materials in advance; keep activities short, efficient, and output-driven.
Logistics	Partners in research may navigate logistic challenges in order to participate (e.g., money, travel to research site).	Obtain sufficient project funding to provide compensation to participants and accommodate logistical support needs.
Knowledge	Clinicians and researchers may have a lack of experience with PCOR; patients, parents may lack knowledge of PCOR and the research process.	Provide laylanguage PCOR education through project IMPACT; provide opportunities for live Q&A; share results in laylanguage.
Patient confidence about contributions, project value	Patient partners are concerned whether their opinion and contributions will be valued, and if the project will truly help other patients.	Engage in active discussion with patient partners; include patients in project team; ensure study endpoints are relevant for patients.
Researcher confidence in value of PCOR	Many researchers are unfamiliar with PCOR, its value; want assurance it will result in a publication or idea to pursue in future research.	Continued clinician, researcher education on the importance of PCOR, including successful and transferable examples.
Cultural & Systemic	Historical mistrust of healthcare institutions, language, and systemic economic barriers impact diverse patient participation.	Engage targeted efforts to reach more of the PFIC community through inclusive PCOR awareness and education campaigns.
Child & Family Focused Research	Many PFIC patients are children, posing unique communication and scheduling challenges - but it is important to include their voices.	Provide child assent and parental consent forms; schedule activities around school hours; create material for childrens' education level.
Rare Disease (Small Population)	It is more complicated to understand how to do PCOR effectively in a smaller and variable population (with multiple subtypes) like PFIC.	We hope to come up with potential solutions through IMPACT. Resulting data could be meaningful for regulatory agencies to include in study design.