

# IMPACT

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

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# IMPACT Module 1

- Modules are educational and explain topics that are relevant for IMPACT.
- Module 1 explained what patient-centered outcomes research (PCOR) entails.
- Each module is followed by a survey.

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## August 2023

N = 37 participants completed the survey that followed Module 1

- 29 patients/parents
- 8 clinicians/researchers



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# IMPACT Module 1 Survey Results:

**Patients/Parents Have  
you or your child ever  
participated in research  
before?  
(n=29)**

23 out of 29 have participated in research

21 out of 29 have participated in PFIC research

# IMPACT Module 1 Results:

**If you or your child did participate in a research study, did you ever find out what the results of that study were? (n = 29)**

3 did receive the results

- 1 was sent the results from the research team
- 2 were sent the results after asking for them

17 did not receive the results

9 did not participate

# IMPACT Module 1 Results:

**Clinician/Researchers**  
**Have you ever  
participated in  
research before?**  
**(n=8, participants can  
check multiple boxes)**

1 has not participated in research before

6 have participated as investigators

1 has participated as study personnel

1 has as a study participant

# IMPACT Module 1 Results:

## Clinician/Researchers

**Have you ever participated in a PCOR study? (n = 8)**

5 have not

2 have participated as researchers

1 have participated as study personnel

# IMPACT

## Module 1

### Results:

**Patients & Clinicians**  
**What are the most important questions that we need to answer, or issues we need to address, to improve the lives of PFIC patients?**

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itch!

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understanding range of symptoms outside of itch

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access to drugs

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improving long term outcomes

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comparison of procedures and outcomes by subtype

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quality of life of patient and/or the family

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education around complexities of the disease

# IMPACT Module 1 Results:

## What IMPACT should do?

### Patient & Clinician Responses:

- reach as many families as possible
- make sure all stages of disease are included (just diagnosed, different stages of treatment, failed treatments)
- listen to needs of patients, ask patients about the most common burdens
- allow for patient input along the way
- focus on outcomes that are meaningful for patients







# IMPACT Module 1 Results: What IMPACT should *not* do

## Patient & Clinician Responses:

- overlook social and economic aspects of disease
- judge patients' questions
- assume to know what patients want/what the burdens of PFIC are

# IMPACT

## Module 1

### Results:

**Patients & Clinicians:**  
**What would research look like that is truly centered on patients and families?**

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transparent, open communication

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understand daily life of PFIC patients, not just science of disease

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improve quality of life

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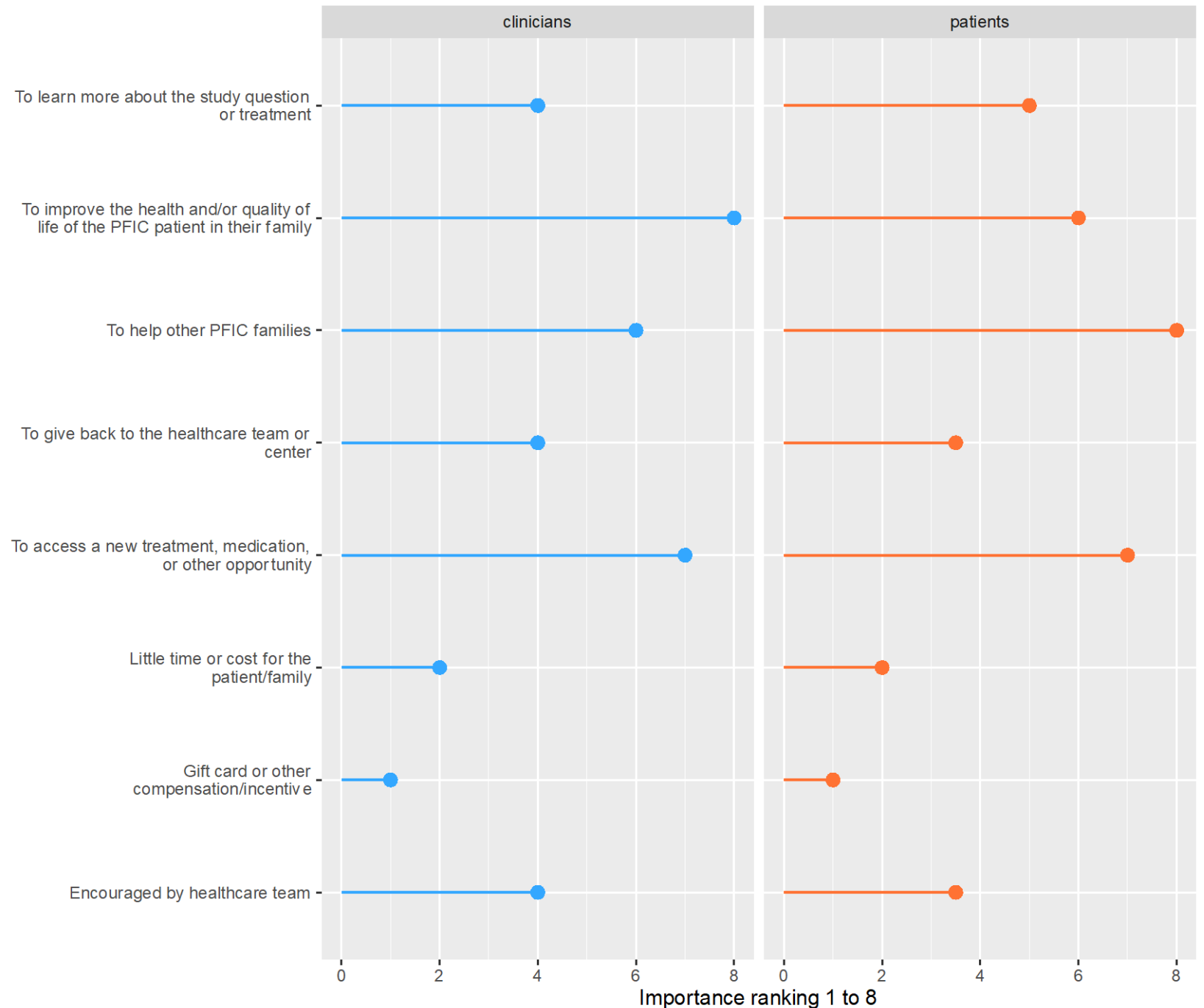
focus on actual issues patients have

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share results with patients

# IMPACT Module 1 Results:

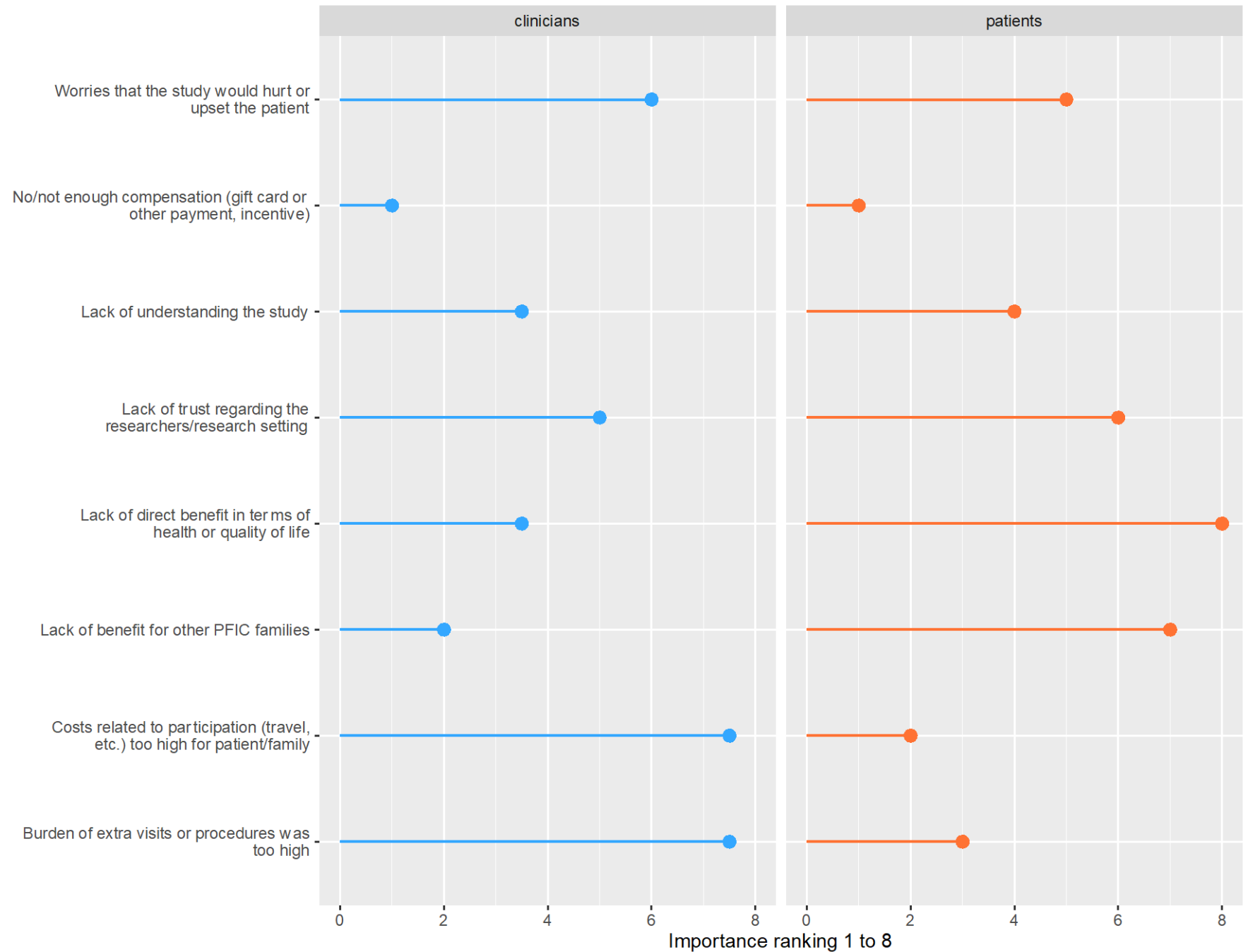
**Comparison of clinicians and patients concerning reasons to participate in research studies (i.e. what clinicians think patients' reasons are to participate, and what patients say their reasons are). Importance is measured as rankings on a scale of 1-8. Clinician and patient responses have a similar pattern.**



# IMPACT Module 1 Results:

**Comparison of clinicians and patients concerning reasons to NOT participate in research studies (i.e. what clinicians think patients' reasons are to NOT participate, and what patients say their reasons to not participate are).**

Importance is measured as rankings on a scale of 1-8.  
**The pattern of responses is not similar!**



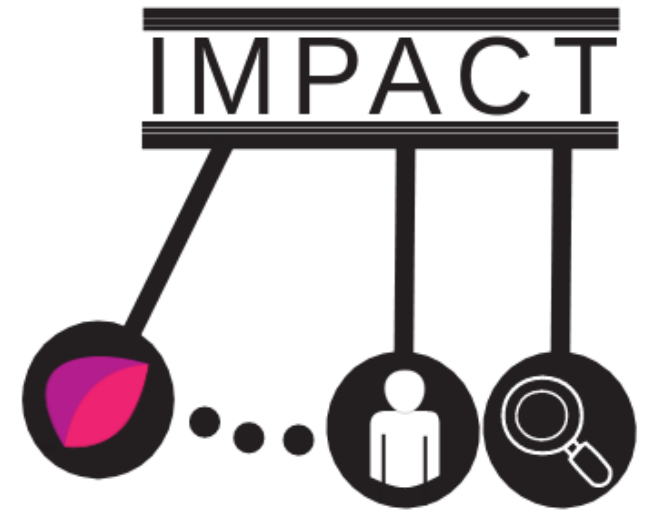
# IMPACT Focus Group Discussion Summary

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**August 2023**

Patient/Parent Focus Group = 7 participants

Clinician/Researcher Focus Group = 4 participants



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# What strategies can we use to maximize outreach to patients for project IMPACT activities?

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## Patient/Parent Focus Group:

- Offer more asynchronous ways to participate
  - Examples: self-guided modules, phone interviews, 1:1s, video voice calls like Marco Polo
- Outreach to patients through healthcare providers
- Make activities available in multiple languages if possible

## Clinician/Researcher Focus Group:

- Best if providers can send info directly to patients
  - Might need IRB approval from their centers



# Which PFIC treatments and interventions should be included on the IMPACT web app? Why?

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## Patient/Parent Focus Group:

- Nutrition (pre + post transplant)!!!
  - Dietary (calories, nutrition)
  - Medical (vitamins, supplements)
- Diversions (pre, post, with transplant)

## Clinician/Researcher Focus Group:

- Use the top 20 treatments, interventions, medications, vitamins reported by patients in the PFIC Network Patient Registry to eliminate bias





# What types of information do you think is important to know about a treatment/intervention when you are trying to choose between options?

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## Patient/Parent Focus Group:

- Side effects
  - E.g., side effects of not receiving a vitamin/supplement – such as optic nerve atrophy from vitamin E deficiency
  - Irreversible vs. reversible side effects
- How long it will postpone transplant (per subtype)
- Impact on quality of life, family
  - E.g., sleep, freedom to leave the home
- Cost/Inconvenience
- Other patients' experiences
- Delivery method
- Accessibility of medicine based on location (rural)



# If you had to evaluate two different treatment options, which factors would exercise the greatest influence on your decision?

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## Patient/Parent Focus Group:

- What's best for my child.
- If my child is old enough to be involved in the decision between treatments, then what they want to choose becomes an important factor.
- Quality of sleep.
- Reduction of itchiness vs. severity of side effects
- Factors can vary based on where you're at financially, or if your child is at the age to begin attending school.
- When choosing between transplant and an alternative treatment, one's decision can depend on the individual's unique threshold of what is too much to handle with the disease. For many, extreme lack of sleep can be the trigger - or reduced ability to parent, adapt, and cope.



# How should we organize the information for the treatments and interventions in the web app?

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## Clinician/Researcher Focus Group:

- Treatment info cannot be prescriptive
- Consider the impact of listing vs. not listing treatments that are not FDA approved
- Indications for a treatment vary on an individual basis; can depend on the goal
  - e.g. reduce pruritus or improve outcome of the disease
- Collect and summarize patients' feedback about treatments; systemic info is more useful than individual statements which can be misleading to other patients without full context