Patient Engagement in Developing Core Outcome Sets

Nicole Pallone¹ and Maureen Smith² in collaboration with the Canadian Inherited Metabolic Diseases Research Network
1 Canadian PKU and Allied Disorders, Sparwood, BC; 2 Canadian Organization for Rare Disorders, Ottawa, ON

Background & Objectives

- There are important evidence gaps related to establishing the effectiveness of therapies for rare inherited metabolic diseases (IMD) in children
- An important design element in the development of robust evaluative studies is high-quality standardized collection of outcomes that are meaningful to patients and families, health care providers, and health systems, i.e., a core outcome set (COS)³
- There are no COSs for studies evaluating care for pediatric IMD
- Including patients and families in this research leads to final outcomes that are considered relevant and meaningful to patients and families
- While patient engagement is considered important for COS development, there is a lack of guidance available for how to best engage with patients in this type of research²

Study Objective:

- To develop a COS for each of two pediatric IMD: phenylketonuria (PKU) and medium-chain acyl-CoA dehydrogenase (MCAD) deficiency that can be incorporated into evaluative studies
- A key element of this study (presented here) was to include the patient perspective so that future PKU and MCAD deficiency research would include outcomes that are meaningful to patients and their families
- The study protocol has been published³

Patient Engagement: Who?

Patient Partners (co-investigators on the study):

- Nicole Pallone: Board member of Canadian PKU and Allied Disorders, mother of a child with PKU, experience in sharing the patient perspective with medical professionals and government policy-makers
- Maureen Smith: Board member of the Canadian Organization for Rare Disorders, patient with a rare pediatric disease, expertise in collaborating with multi-stakeholders on advisory committees

Role:

- Involved from protocol development stage
- Contributed expertise to identify challenges to incorporating patient perspectives and designed strategies to address those challenges
- Led the patient engagement activities, including newsletters, training, and communication

Family Advisory Forum (FAF):

- Seven parents of children diagnosed with IMDs in Canada were recruited to participate by the clinician investigators and/or Patient Partners through their professional networks
- Provided feedback to the study team throughout the project, specifically in developing the Delphi surveys
- Participated in the in-person consensus workshop

Patient Engagement Strategy

Stage 1: Recruitment of FAF & Study Launch

- Parents of children with IMDs were recruited
- FAF members received training on:
  - Working with research teams on patient-oriented research
  - Core outcome sets and Delphi surveys

Stage 2: Evidence Review & Delphi Survey Development

- Research team conducted a systematic review to identify outcomes used in previous studies of MCAD deficiency and PKU
- Candidate outcomes were included in a Delphi consensus survey of parents, health care providers, and policy advisors to determine the most important outcomes
- Making Delphi survey accessible for patients/caregivers
- Providing patients/caregivers with easily understandable definitions for scientifically complex candidate outcomes
- Concisely and clearly communicating Delphi results in a manner that facilitated FAF feedback

Stage 3: In-person Consensus Workshop

- Patient partners, FAF members, health care providers, health policy advisors, and methodologists participated in an in-person workshop to develop the final COS for each disorder
- Outcomes were discussed and anonymously voted on for inclusion in the final COSs
- First experience for many patients/caregivers attending a research meeting with their child’s physician(s) in attendance (power imbalance)
- FAF participants unsure of the process and/or whether their feedback would be valued
- Ensuring that FAF members understood their role and managing their expectations
- Ensuring that patient/families felt supported and that their perspectives were well integrated into discussion

Stages of Patient and Family Participation

Activities

- Patients/caregivers may not have participated in research like this before
- Explaining why families would want to be involved in a study that will inform further studies, therefore, somewhat distant from the immediate concerns of families
- Ensuring Delphi surveys and consensus workshops explanations are accessible
- Maintaining interest of FAF members throughout the research project (2+ years)

Challenges

- All patient materials were written or vetted by Patient Partners
- Provided in-person training to fully explain importance of patient engagement in the study, expectations for participation, and COS methods
- Covered all costs (travel, parking, child care, etc.) and provide honoraria

Solutions

- Feedback from FAF members resulted in changes to the presentation of the Delphi survey and made it more accessible to participants
- Several outcome definitions were revised according to feedback from FAF members
- FAF received additional training in Delphi surveys adapted from the COMET Initiative lay-language materials
- Patient Partners and FAF provided feedback on design and content of Delphi surveys, including preamble materials
- Patient Partners and FAF provided feedback on the outcome definitions

Results

- In-person training contributed greatly to understanding the study and resulted in enthusiasm to undertake the study together
- Review of materials sent ahead of the meeting to ensure participants (including patients/family members) understood definitions and requirements

Example Materials for Patient Engagement

Seasonal newsletters written by Patient Partners

- Communicating regularly to parents and Patient Partners: feedback, updates on Delphi surveys, and child-related content

Consensus Workshop Experience

- FAF members who participated in the consensus workshop (n=5) reported:
  - (100% agree/strongly agree)
  - being able to express their views freely (100% agree/strongly agree)
  - input was considered during the discussion (100% agree/strongly agree)

Conclusions

- A tailored approach to patient engagement guided by patient partners has been feasible and valuable
- Many unique challenges to meaningful engagement by patients and caregivers in COS development require special consideration
- Our approach to patient engagement in developing COSs can be applied to other rare disease contexts, allowing the patient perspective to influence the direction of future research projects

References:


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