



Patient Engagement in Developing Core Outcome Sets

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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)¹
- 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):

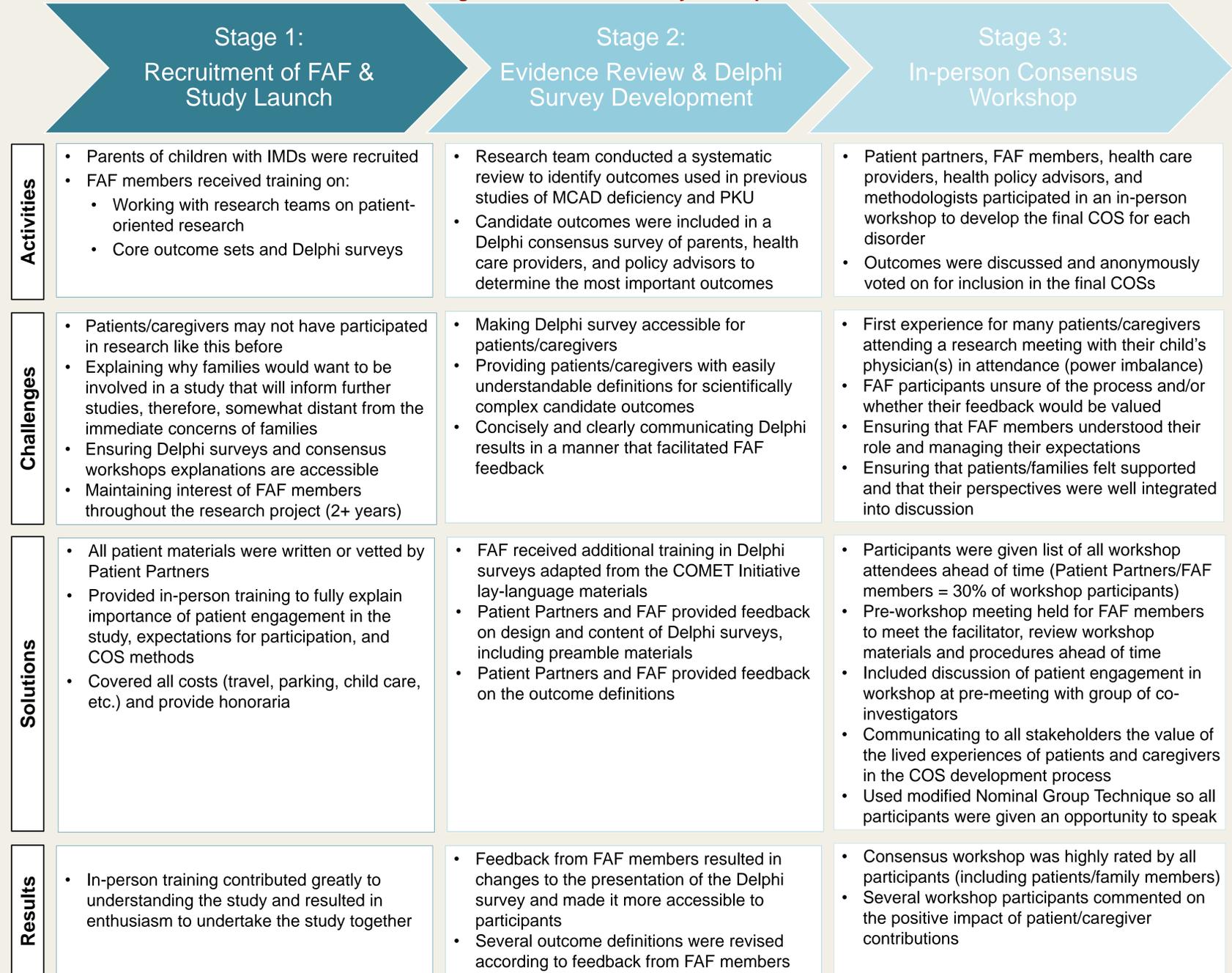
- Seven parents of children diagnosed with IMDs in Canada were recruited to participate by the clinician investigators and/or Patient Partners through their professional networks
- **Role:**
 - Provided feedback to the study team throughout the project, specifically in developing the Delphi surveys
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

References:

1. Li G, et al. Registry-based randomized controlled trials- what are the advantages, challenges, and areas for future research? *J Clin Epidemiol.* 2016;80:16-24.
2. Young B and Bagley H. Including patients in core outcome set development: issues to consider based on three workshops with around 100 international delegates. *Research Involvement and Engagement.* 2016; 2:25
3. Potter BK, et al. Establishing core outcome sets for phenylketonuria (PKU) and medium-chain Acyl-CoA dehydrogenase (MCAD) deficiency in children: Study protocol for systematic reviews and Delphi surveys. *Trials.* 2017;18(603).

Patient Engagement Strategy

Stages of Patient and Family Participation



Example Materials for Patient Engagement

Seasonal newsletters written by Patient Partners



WHY A FAMILY ADVISORY FORUM?

- To provide feedback from patient and family experts to the full study team
- To assure inclusion of the perspectives of patients and families across all areas of the project
- To ensure that the final outcomes we develop are considered important and meaningful to patients and families

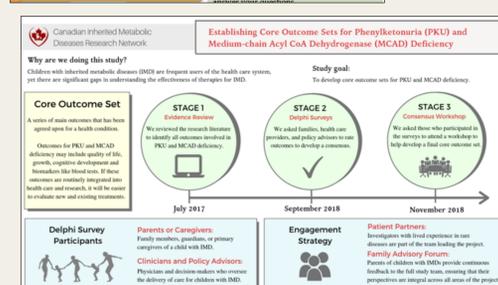
Training Materials

WHAT WILL I BE EXPECTED TO DO?

- Receive training in working together with research teams on patient-oriented research
- Receive training to learn about core outcome sets and Delphi surveys
- Participate in the design of meaningful and user-friendly surveys
- Participate in the selection of patient/family-reported measures of outcomes

Life happens - let us know how we can support your continued involvement

Study overview infographic



Consensus Workshop Experience

- FAF members who participated in the consensus workshop (n=5) reported:
 - pre-meeting was helpful (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)

"The whole day was fantastic. For myself, I especially appreciated an opportunity to be in a room with so many physicians and researchers who specialize in metabolic disorders - a first for me - and to hear their perspectives and experiences."
- FAF Member

"The only thing may have been to have more time to get familiar with all the info. We were given everything prior but personally I wish I had taken it upon myself to have gone over and had some pre-discussion by phone so that I felt better equipped."
- FAF Member

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