

No Choice of Outcomes About us Without us!



Bridget Young

**Bridget Young, Heather Bagley, John Turner,
Maureen Smith, Tess Harris & Paula
Williamson**

Aims

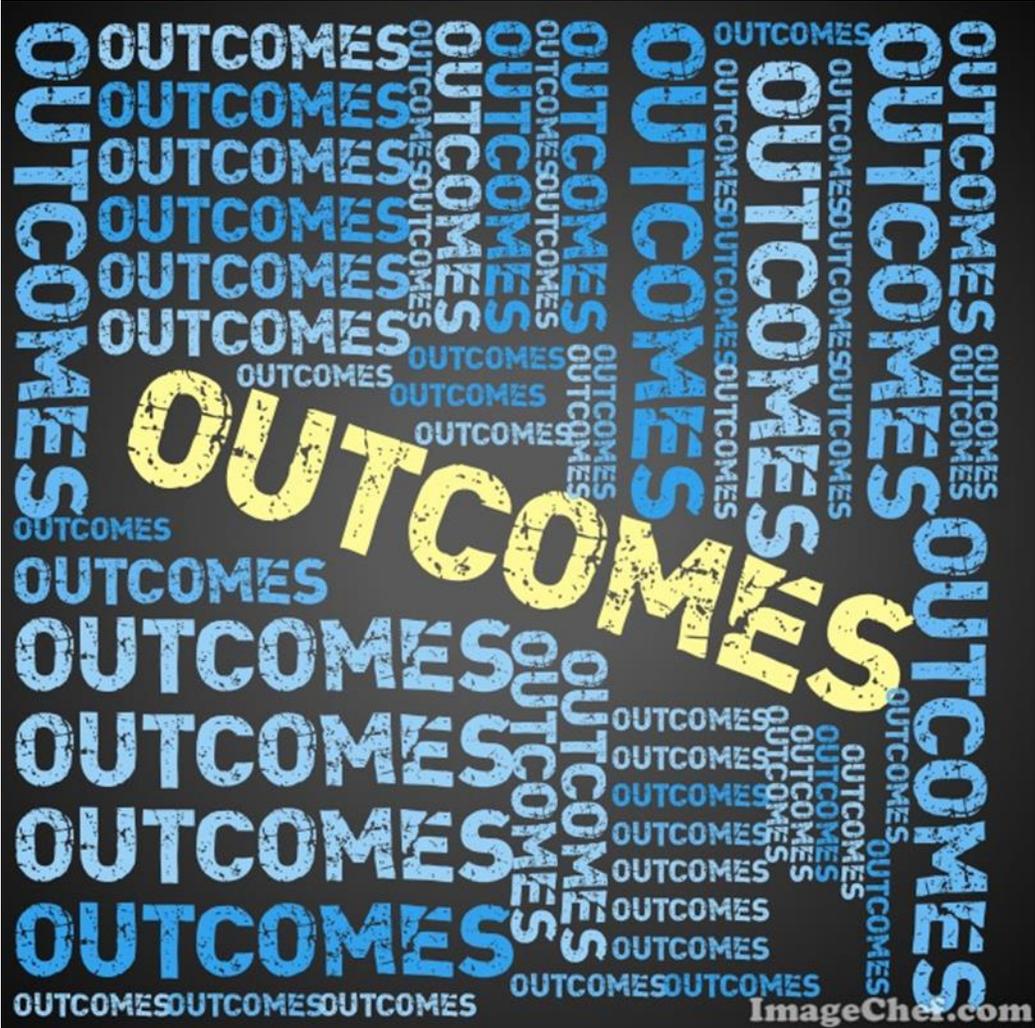
- What are the problems with outcomes in research?
- What are core outcome sets (COS) and how can these help?
- How are COS developed & what are the challenges?
- How are patients inputting into COS development.
- What is COMET and how are they helping?
- How have patient organisations helped with COS development?
- How can COMET / PoPPIE work with patient organisations?

The problems with outcomes in research & core outcome set development



Heather Bagley

Problem 1- Health care research is untidy



Problem 2 - Selective reporting of outcomes



- Studies reporting positive or significant results are more likely to be published
- Outcomes that are statistically significant are more likely to be fully reported

(Dwan et al, PLoS ONE 2008)

Problem 3 – Outcomes of relevance to patients?

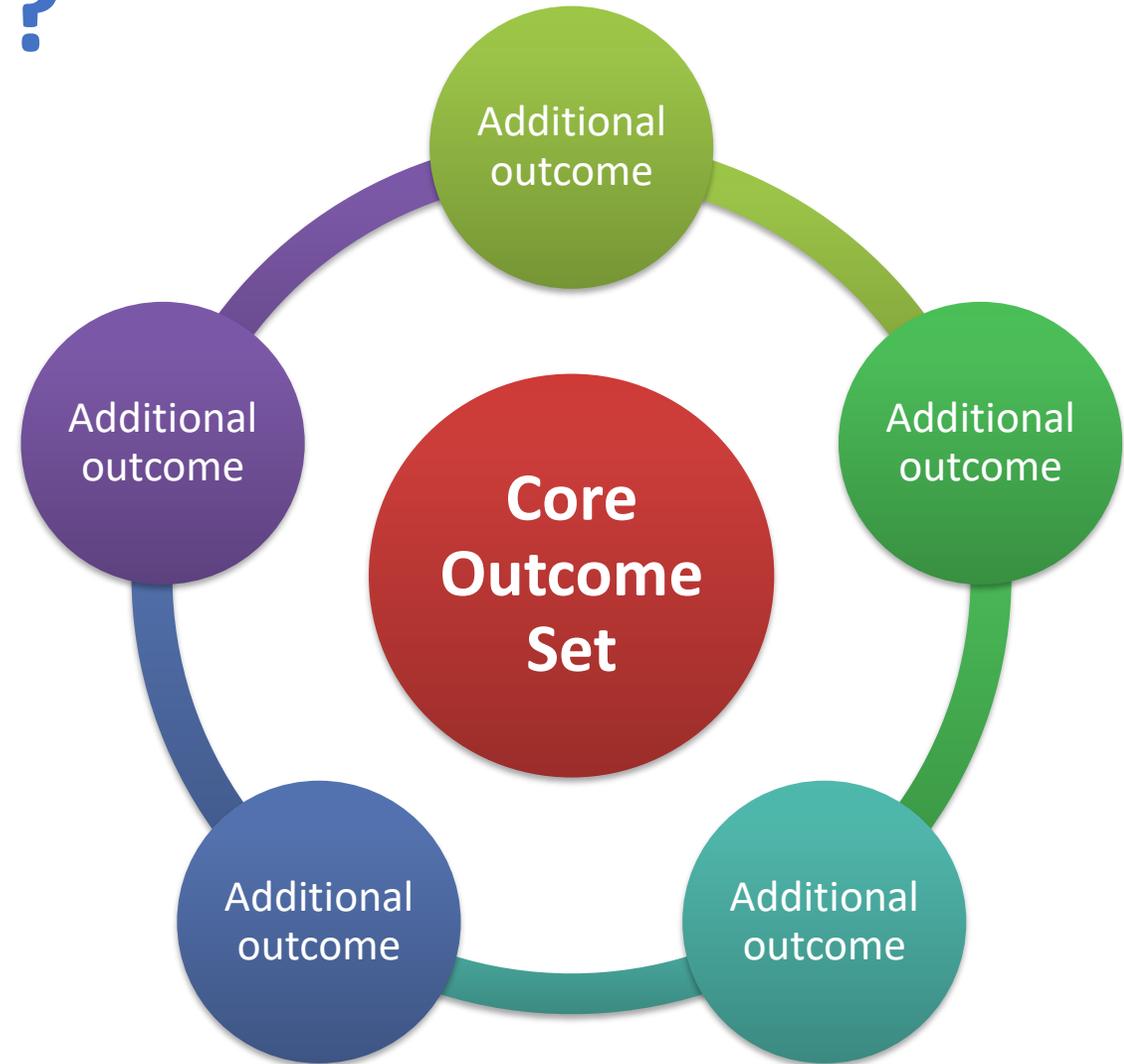


“Outcomes need to be relevant to patients, practitioners and policy-makers if the findings of the research are to influence practice and future research” Kirkham et al, 2013

What's the solution?

Core Outcome Set

An agreed standardised set of outcomes that should be measured and reported, as a minimum, in all clinical trials in specific areas of health or health care



How are core outcomes agreed upon?

- Interviews
- Systematic reviews
- Delphi study
- Consensus meeting

Having a voice in Core Outcome Set (COS) development



Maureen Smith



John Turner

How can patients contribute?

Patient Involvement

- Public research partners

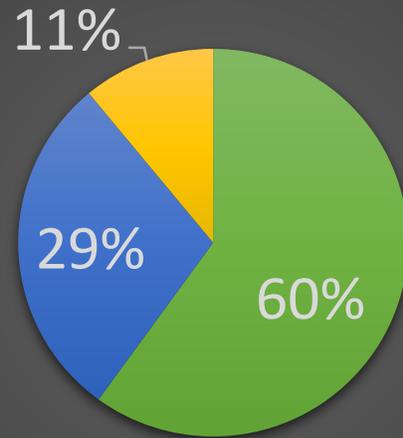
Patient participation

- COS study participants



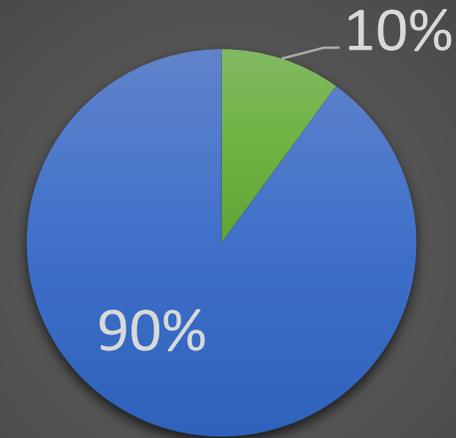
Patient/public input into COS development

Published (n=307)



■ No input ■ Input ■ No details given

Ongoing COS (n=223)



■ No input ■ Input

Gargon E, Gorst SL, Harman NL, Smith V, Matvienko-Sikar K, Williamson P. (2018) Choosing important health outcomes for comparative effectiveness research: 4th annual update to a systematic review of core outcome sets for research. *PLOS ONE*, 13(12).

What impact does the patient perspective have?

- **OMERACT Rheumatoid arthritis:**
Fatigue
- **IMPACT study (Chronic pain)**
fatigue, sleep, home and family care, social and recreational activities, interpersonal relationships, and sexual activities



What it was like for me!

- How I heard about the study
- Why I decided to take part
- How it worked



Selecting Core Outcomes for Randomised Effectiveness trials In Type 2 diabetes.

What it meant for me

- Empowered – became part of the solution
- Inclusive
- Respect
- Mutual valuing of opinions

Join the COS revolution!



Patient Engagement in Developing Core Outcome Sets

Nicole Pallone and Maureen Smith

in collaboration with the Canadian Inherited
Metabolic Diseases Research Network



International PPI Network Webinar – February 27, 2020

Funded by:
#151614



Study Objectives



- Purpose: to facilitate research that will tell us which treatments are most effective for children with inherited metabolic diseases
 - Initial focus is on phenylketonuria (PKU) and Medium-chain acyl-CoA dehydrogenase (MCAD) deficiency
- We will do this by establishing agreed core outcome sets for these conditions
- If these outcomes are routinely integrated into health care and research, it will be easier to evaluate existing and new treatments

Patient Engagement Strategy

Patient Partners (co-investigators on the study): Nicole Pallone and Maureen Smith

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 networks

Role:

- Provided feedback to the study team throughout the project, specifically in developing the Delphi surveys
- Participated in the in-person consensus workshop

Challenges

- ◆ Communicating importance of core outcome sets
- ◆ 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

Solutions

- ◆ 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
- ◆ 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

Challenges

- ◆ 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 patients/families felt supported and that their perspectives were well integrated into discussion

Solutions

- ◆ Participants were given list of all workshop attendees ahead of time (Patient Partners/FAF members = 30% of workshop participants)
- ◆ Pre-workshop material circulated to FAF to explain all details of the consensus workshop
- ◆ Pre-meeting held for FAF members to meet the facilitator, review workshop materials and procedures ahead of time
- ◆ At the consensus workshop, communicating to all stakeholders the value of the lived experiences of patients and caregivers in developing COS and how they had participated in the study
- ◆ Used modified Nominal Group Technique so all participants were given an opportunity to speak

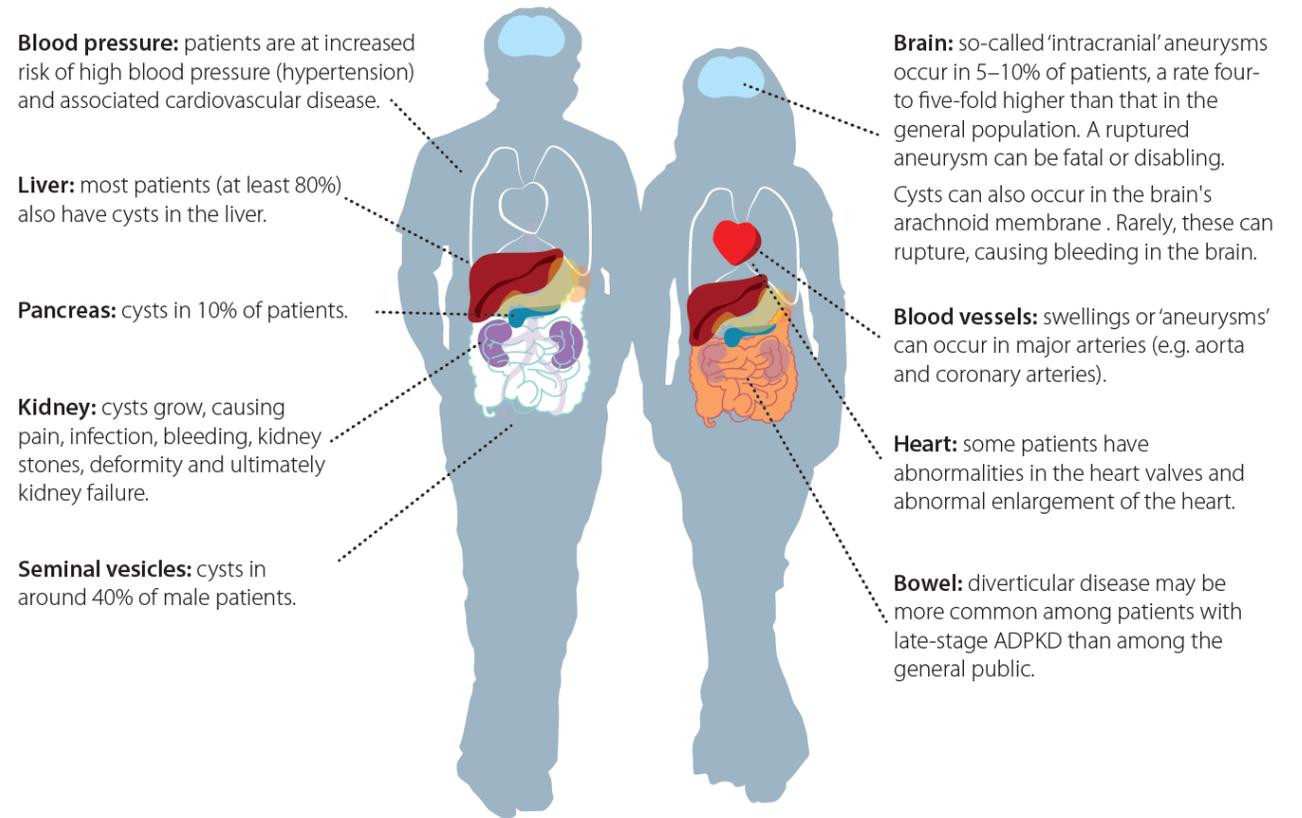
What can patient organisations do to support core outcome set development?



Tess Harris, Chief Executive,
Polycystic Kidney Disease Charity

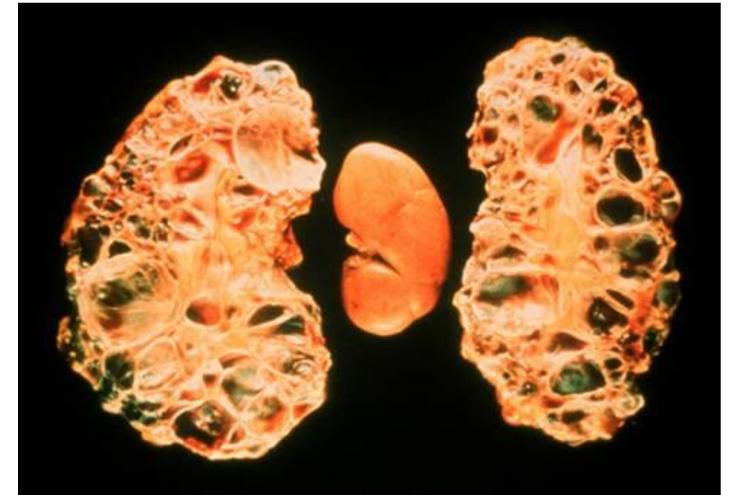
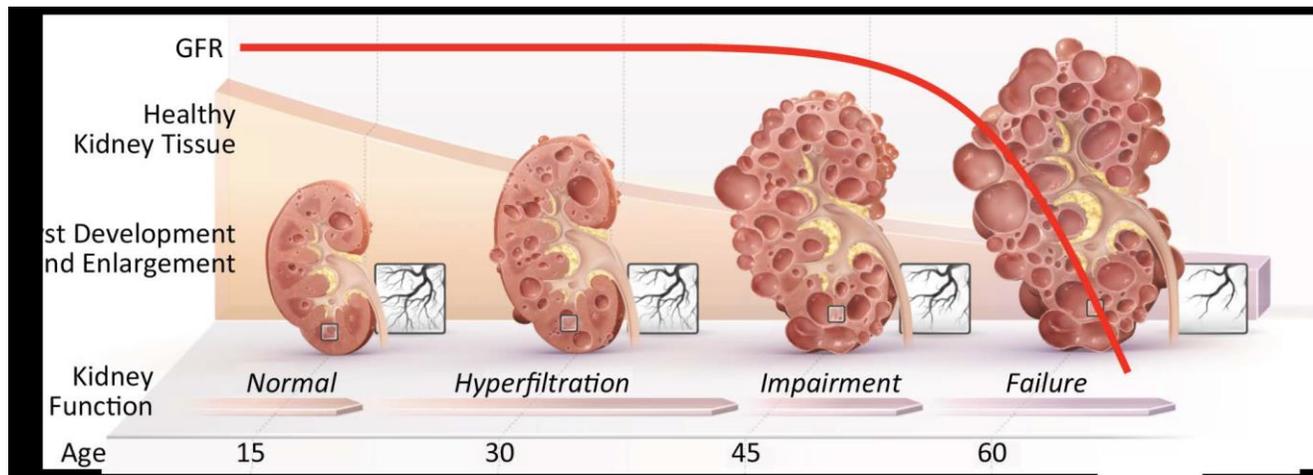
The Polycystic Kidney Disease (PKD) Charity

- Aim to improve lives of ~70,000 UK adults and children affected by PKD:
 - Practical and emotional information, advice and support
 - Awareness raising and advocacy for holistic joined up care
 - Research: capacity building (PhD students) and 'pump-priming'
- Est 2000, 4.5 staff
- Co-founder of PKD International global alliance (President: Tess)



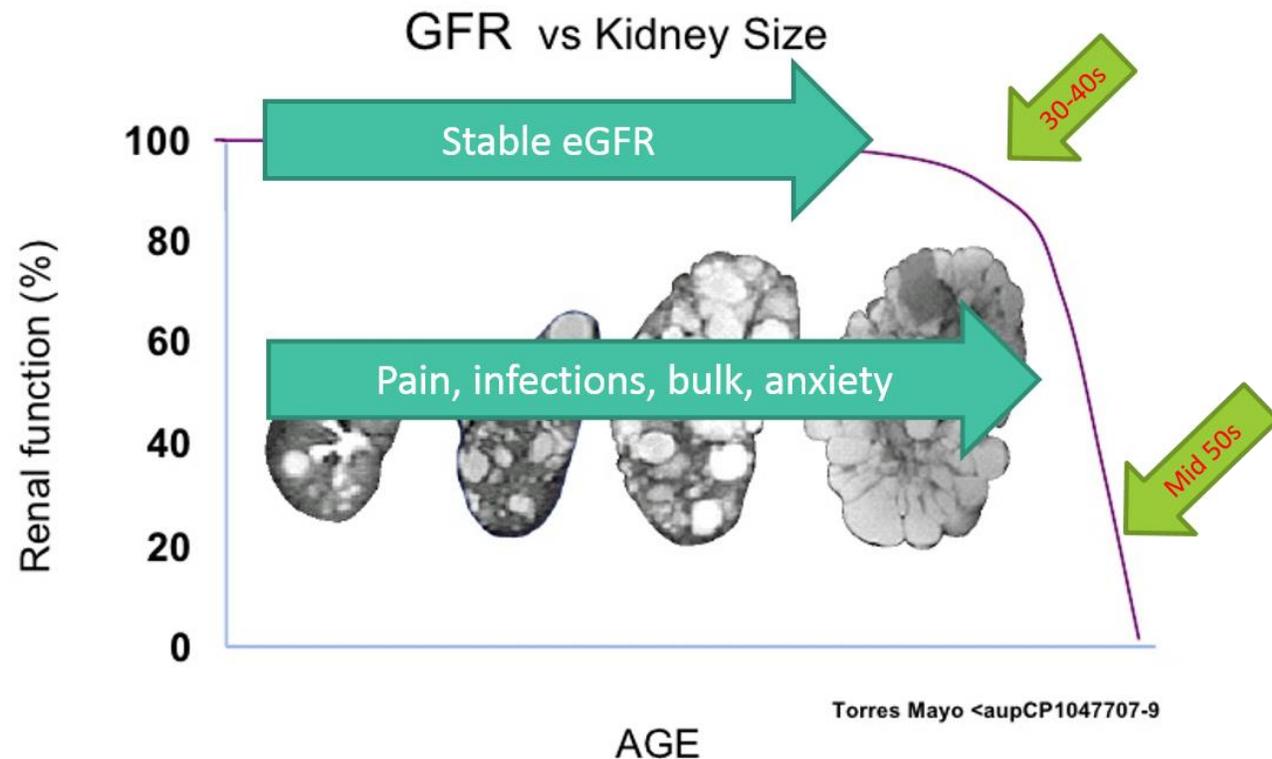
PKD: most common inherited kidney disease; multi-organ; incurable; high socio-economic burden

Stopping cystic kidney growth & function decline is main focus of research



PKD accounts for 1 in 10 people with a kidney transplant or on dialysis

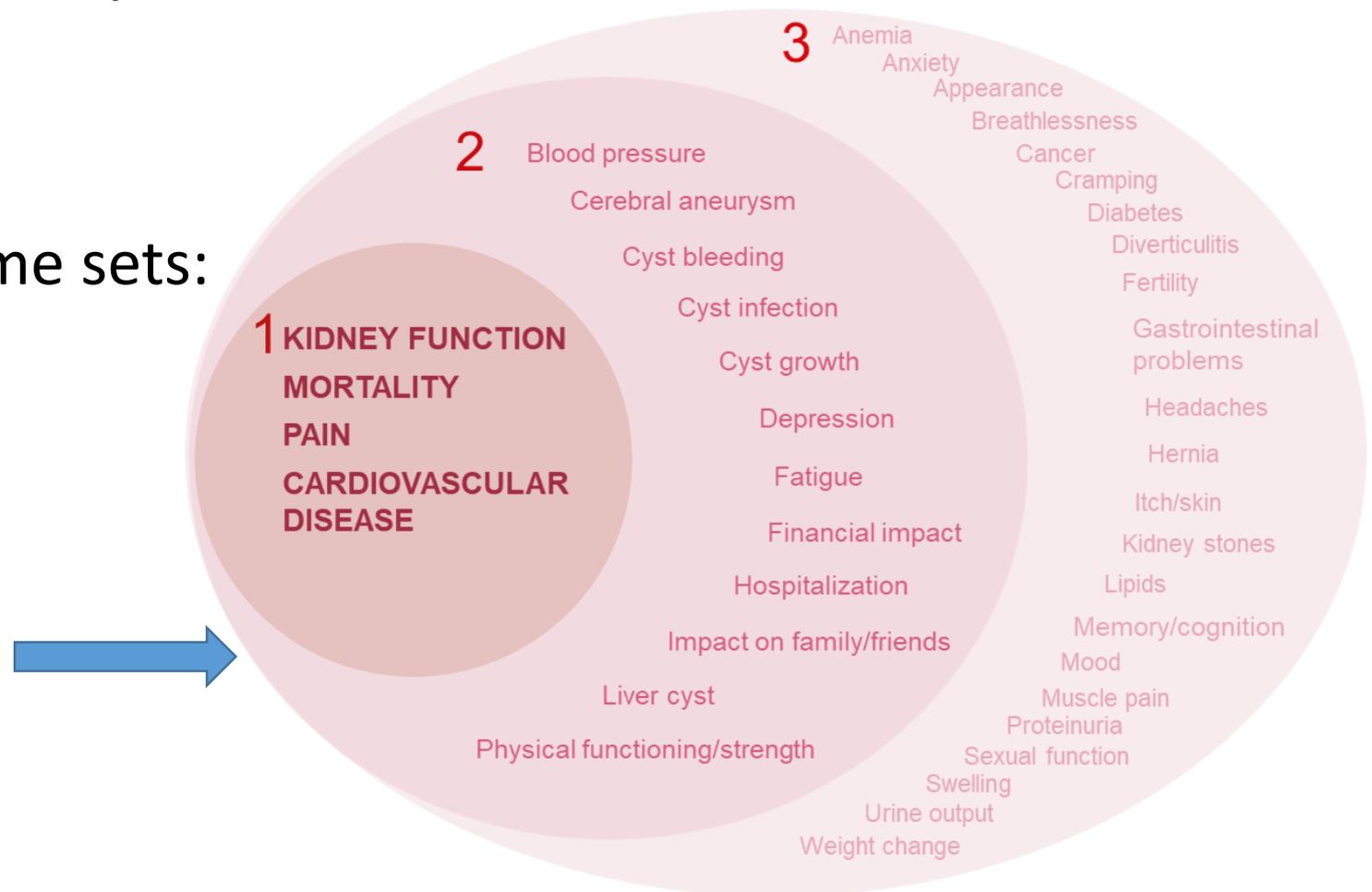
Defining outcomes is challenging in ADPKD



- Only one licensed drug (2015)
- Very few trials
- Highly variable outcomes used
- Emphasis on non-clinically important outcomes
- Composite outcomes considered but no validated PRO

SONG – ‘Standardised Outcomes in Nephrology’ group est 2015

- International Steering Committee inc Tess
- Published core outcome sets:
 - Transplant
 - Haemodialysis
 - Peritoneal Dialysis
 - Kids (Paediatric CKD)
 - PKD (just in press)



How we support SONG & try to make COS the 'norm' in nephrology

- Our contribution:

- Member of Steering Group from day 1 and involved in all SONGs
- Bring long standing knowledge of patient and carer experiences of kidney disease and especially PKD
- Promote the Delphi surveys and help recruit for workshops
- Help promote SONG at conferences and clinical study groups
- Continue to 'sell' SONG

- Challenges:

- Explaining COS to patients, clinicians and researchers
- Communicating to entrenched research networks who are unfamiliar with COS or 'suspicious'
- Demonstrating feasibility and usability; minimising effort
- Recognising conflicting agendas
- Getting buy in and trust from all stakeholders
- Integrating into the research infrastructure

IF YOU ARE ASKED TO TAKE PART IN COS – DON'T HESITATE TO SAY YES!

Including patients in COS development – how is COMET helping?



Bridget Young

What is COMET (Core Outcome Measures in Effectiveness Trials Initiative)?



Brings together people interested in the development and application of Core Outcome Sets (COS)

- Raise awareness of need for COS development and uptake
- Provide database & resources to facilitate these aims
- Encourage evidence-based COS development

COMET endorsement - <http://www.comet-initiative.org/cosuptake>

How is COMET addressing patient input in COS development?



Bridget Young



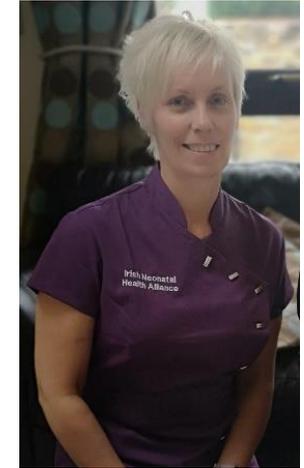
John Turner



Maureen Smith



Jan Geissler



Mandy Daly



Jean Slutsky



Doreen Tembo

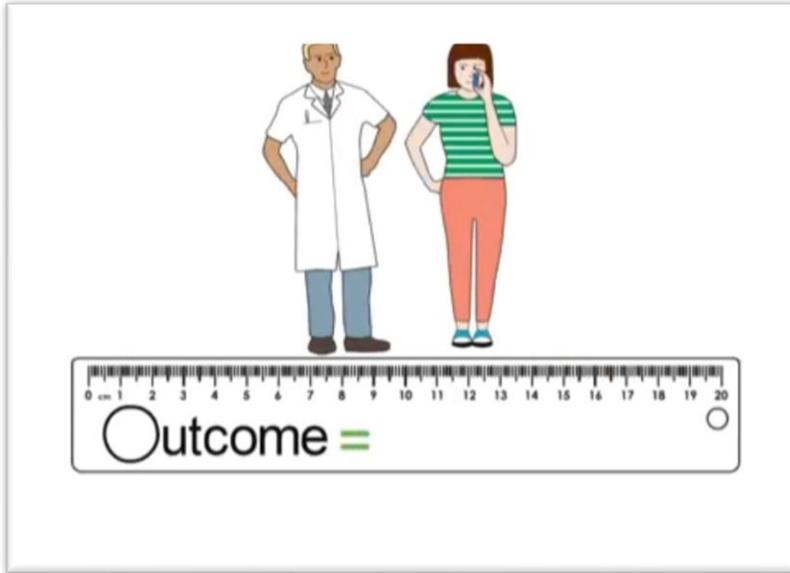


Maarten de Wit



Heather Bagley

COMET / PoPPIE Resources / Activities



COMET INITIATIVE Involving patients and the public in improving research

How health care treatments are developed

To help patients, doctors and other health professionals make decisions about treatments, we need evidence about what works best. Treatments are developed and tested by researchers to make sure they work and are safe. To do this researchers need to look at the effects those treatments have on patients. Researchers do this by measuring an 'outcome'. For example, in a study of how well a new asthma treatment works, 'outcomes' might include:

- A measure of how fast you can blow air out of your lungs
- Night time wheeze
- Asthma quality of life measure

What are the challenges in measuring outcomes?

At the moment, different studies looking at treatments for the same condition often measure different outcomes. For instance, imagine two studies

- The information they do have might be biased

How can we solve this problem?

If all studies in a particular health condition used the same outcomes, they could all be compared and combined. This would reduce waste by making best use of **all** the research. When a set of main outcomes has been agreed for a health condition, it's called a '**core outcome set**'. If all studies in a particular condition, such as migraine, then measured and reported all these core outcomes, we could:

- Bring together all the studies to get a better understanding of which treatments are best
- Avoid the problem of some studies only reporting a selection of the outcomes that were measured.

How are core outcomes agreed upon?

Checklist for public research partners and Core Outcome Set (COS) study developers involved in designing a COS study

Thank you for agreeing to help us plan this research study. During our discussion we intend to cover some of the following topics:

- How you find out about your health condition and news about your health condition (e.g from patient organisations)
- Contact with clinical teams about condition (frequency / hospital / clinics / community)
- Key messages that might encourage people to consider taking part in a COS study
- Best ways of explaining a COS
- Thoughts on the best methods to use in the study (surveys / interviews with



Including patients in core outcome set development

Young and Bagley *Research Involvement and Engagement* (2016) 2:25
DOI 10.1186/s40900-016-0039-6

Research Involvement
and Engagement

COMMENTARY

Open Access

Including patients in core outcome set development: issues to consider based on three workshops with around 100 international delegates



Bridget Young^{1*} and Heather Bagley²



How can we assess the quality of an existing COS?

- COS-STAD – Minimum Standards for COS development
Purpose:
 - encourage researchers to achieve at least the minimum standards for COS development and
 - to help users assess whether a COS should be adopted in practice

Kirkham, JJ, Davis, K, Altman, DG, Blazeby, JM, Clarke, M, Tunis, S & Williamson, PR 2017, 'Core Outcome Set-STAndards for Development: The COS-STAD recommendations', PLoS Medicine, no. 11,

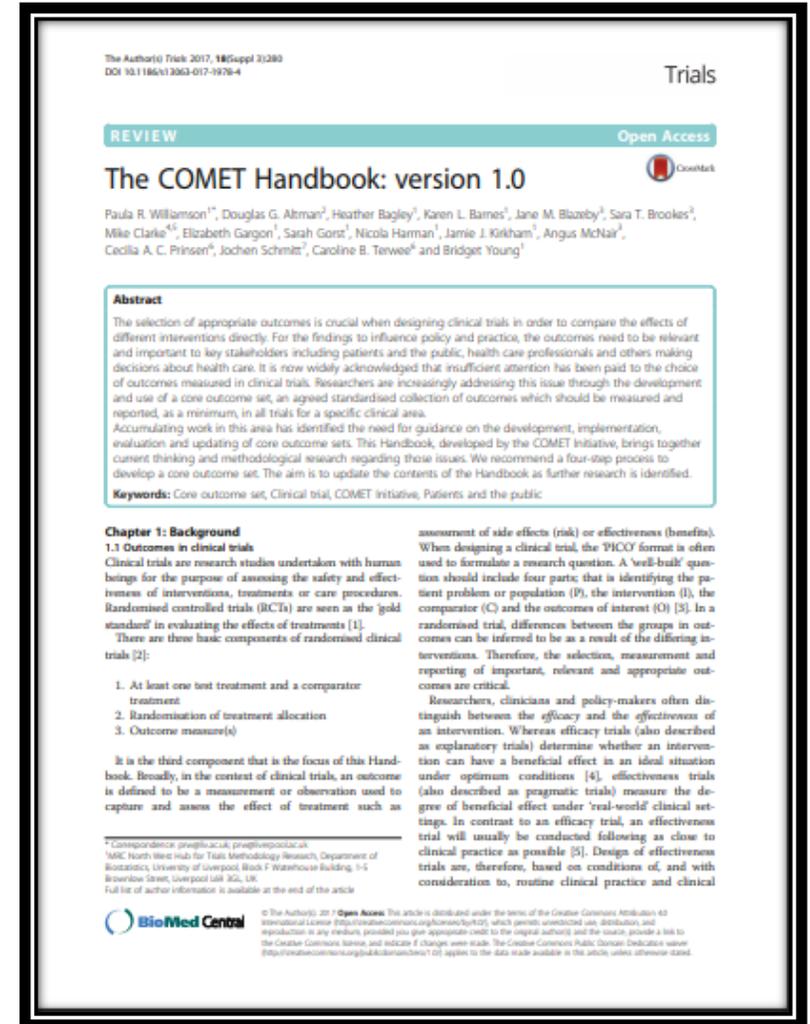
What if no Core Outcome Set exists?

Contact us:

<http://www.comet-initiative.org/contactus>

The COMET Handbook (2017) Williamson P.R. et al *Trials* (Suppl 3) :280

<https://doi.org/10.1186/s13063-017-1978-4>



How can COMET / PoPPIE work with patient organisations:

- To
 - Raise awareness of COS and why important for research
 - Facilitate patient involvement and participation in COS development
 - Improve uptake of COS
 - improve COS studies for patients to input into

Keen to discuss your views? Any other ways we might work together?