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2017

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Promoting Excellence in Health Services Research

How to discuss estimands with patients and the public – developing a practical tool

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Background

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- Patient and Public Involvement (PPI)
  - *'Research being carried out 'with' or 'by' members of the public (including patients and carers) rather than 'to', 'about' or 'for' them' (INVOLVE)*

Why?

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Offers a new perspective

Potential to improve research quality

Moral imperative

Mandatory

Patient and public involvement in trials

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Up to 50% of trials report to involve patients in the design of trials in IRAS (Raza et al, 2020)
92% of surgical trials reported to do some type of PPI (Crooker et al, 2019)

James Lind Alliance
Priority Setting Partnerships

COMET Initiative

/hat are core outcome sets? (A COMET Initiative video)

What is Trials Methodology?

Patient and public involvement in statistical aspects of trials

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- Statistics and numerical aspects underpin how trials are conducted and, more importantly, what they can tell us about treatments available to patients
- Statistics and numerical aspects are often based in context – they come from our interpretation of reality

Goulao *et al. Trials* (2021) 22:499
https://doi.org/10.1186/s13063-021-05451-x

Trials

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Patient and public involvement in numerical aspects of trials (PoINT): exploring patient and public partners experiences and identifying stakeholder priorities

Beatriz Goulao*, Hanne Bruhn, Marion Campbell, Craig Ramsay and Katie Gillies

Abstract

Background and aims: Patient and public involvement is increasingly common in trials, but its quality remains variable in a lot of settings. Many key decisions in trials involve numbers, but patients are rarely involved in those discussions. We aimed to understand patient and public partners' experiences and opinions regarding their involvement in numerical aspects of research and discuss and identify priorities, according to multiple stakeholders, around the most important numerical aspects in trials to involve patients and the public in.

Methods: The study had two stages: (1) online focus groups with patient and public partners recruited via online platforms and analysed using inductive thematic analysis and (2) online priority setting meeting with UK- and Ireland-based stakeholders and following James Lind Alliance methodology. Pre-selected numerical aspects were introduced prior to the meeting and discussed and prioritised based on a voting system.

Results: In stage 1, we held two focus groups with patient and public partners (n = 9). We identified four themes

Health Expectations An International Journal of Public Participation in Health Care and Health Policy

VIEWPOINT ARTICLE | Open Access

Public and patient involvement in quantitative health research: A statistical perspective

Ailish Hannigan BSc, PhD

First published: 19 June 2018 | https://doi.org/10.1111/hex.12800 | Citations: 11

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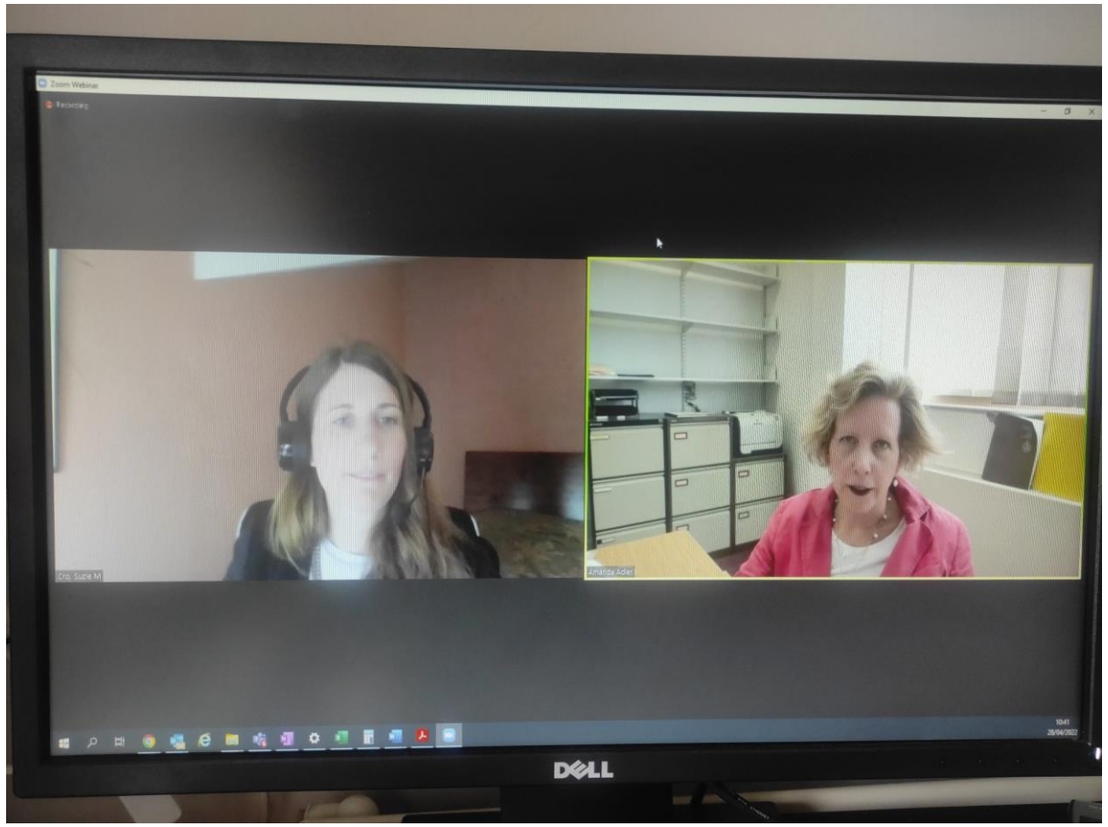
Abstract

Why should we involve patients in discussions about estimands?



- Prof Amanda Adler

- “Estimands are for patients and for the people that will benefit from them the most” but we need to “provide rationale to select one strategy over another”

Why should we involve patients in discussions about estimands?

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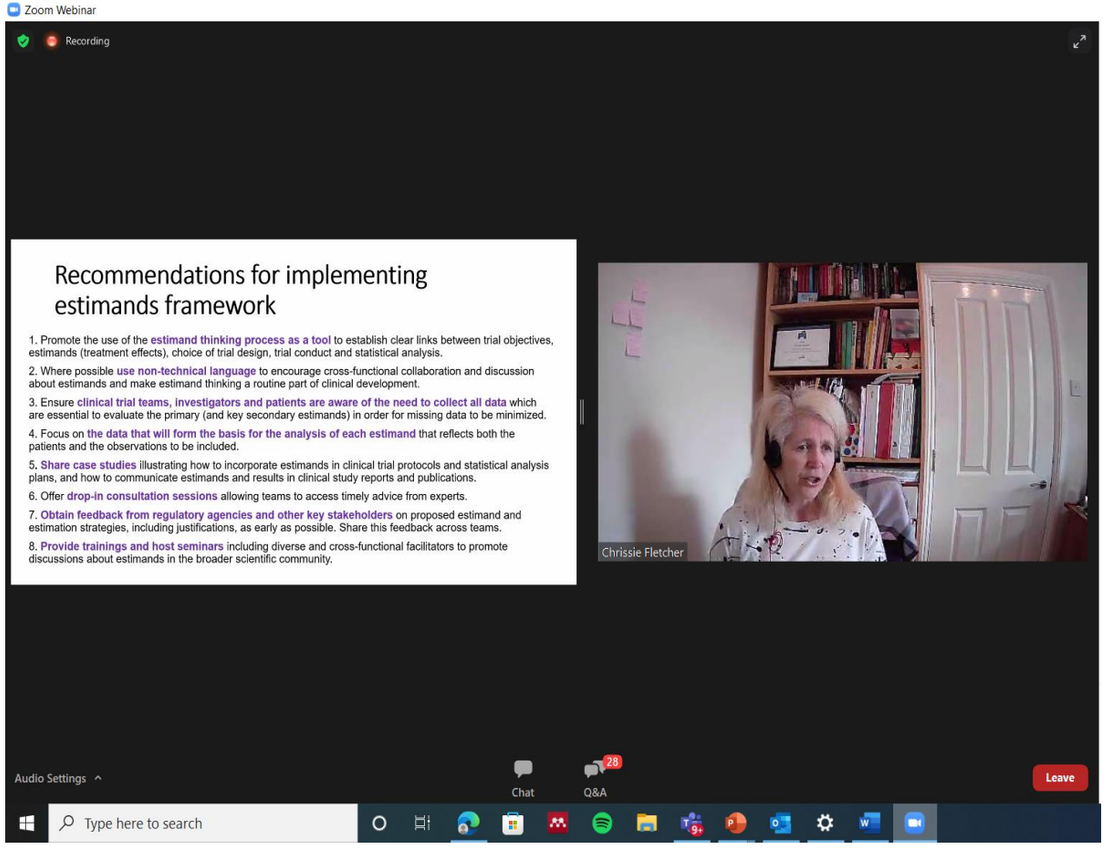

- Chrissie Fletcher

- Use non-technical language to encourage collaboration & discussion across groups
- Consider estimands of importance to difference stakeholder groups

Why should we involve patients in discussions about estimands?

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- Estimands bring clarity to the research question which will have clear impact in patient's lives

- Patients want a say in the research question/direction, and being involved in defining the estimand, is essential to achieve that

"Patients, when they expect to adhere, prefer adherence-adjusted results, such as per-protocol effects."

Our initial work in this field

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- How do we start a conversation with public partners about estimands?
- Methods
- Online meeting with public partners from an established statistical project (HEALTHY Stats, led by Dr Suzie Cro)
- Five public partners aged between 20 and 70 years of mixed ethnicities and sex; four facilitators to facilitate breakout room discussions

Aim of our meeting

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- Explore public partner's perspectives on the importance of discussing estimands with public partners when designing a trial

- Develop a practical tool with public partners that helps explain what an estimand is and what impact it may have in trial results

Results

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Trials that matter to patients

Involve early on

Education & communication

Change of culture

Results – tool feedback

- **General feedback about the tool was incorporated after the first meeting and followed an iterative process with further feedback sought in two rounds of refinement.**
  - Public partners found the tool useful to start a discussion about estimands in a trial design context
  - They recommended the use of storytelling, analogies and visual aids
  - It was felt that the tool should be shared and a chance to discuss it with the trial team/statistician provided
  - Public partners raised that potential trial participants might need to know about the estimand of the trial, however this tool would not be indicated for that

Tool – introduction with an analogy

Analogy

Visual story

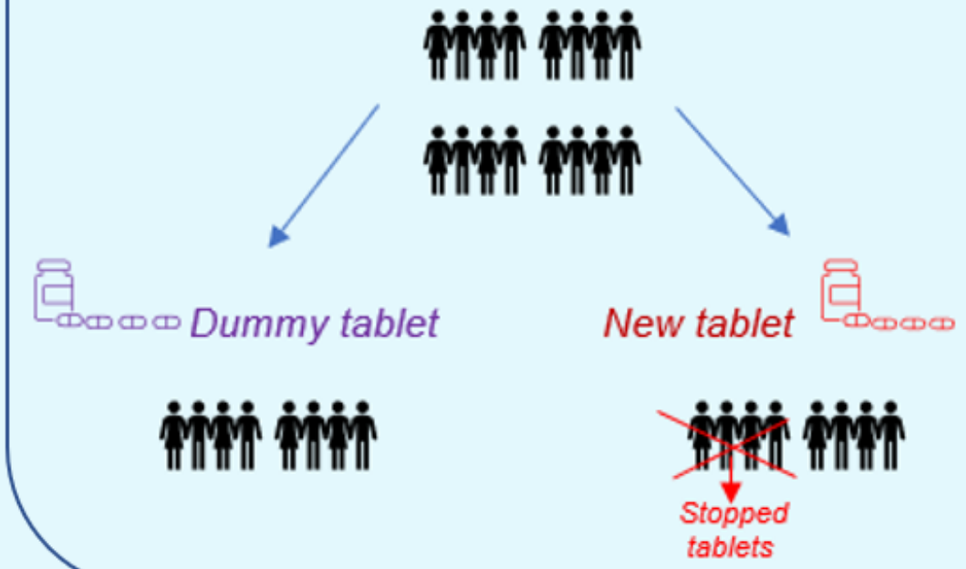
Tool - example

An example (application of estimands)

Visual representation

Headache prevention trial

Investigators tested a new tablet for severe headaches that is taken 4 times a day…



A group of patients were given the new tablet whilst another group were given a dummy tablet with no active ingredient (a 'placebo', also taken 4 times a day). But in the new tablet group, some of the participants stopped taking the tablet because of severe stomach cramps.

Tool - example (cont)

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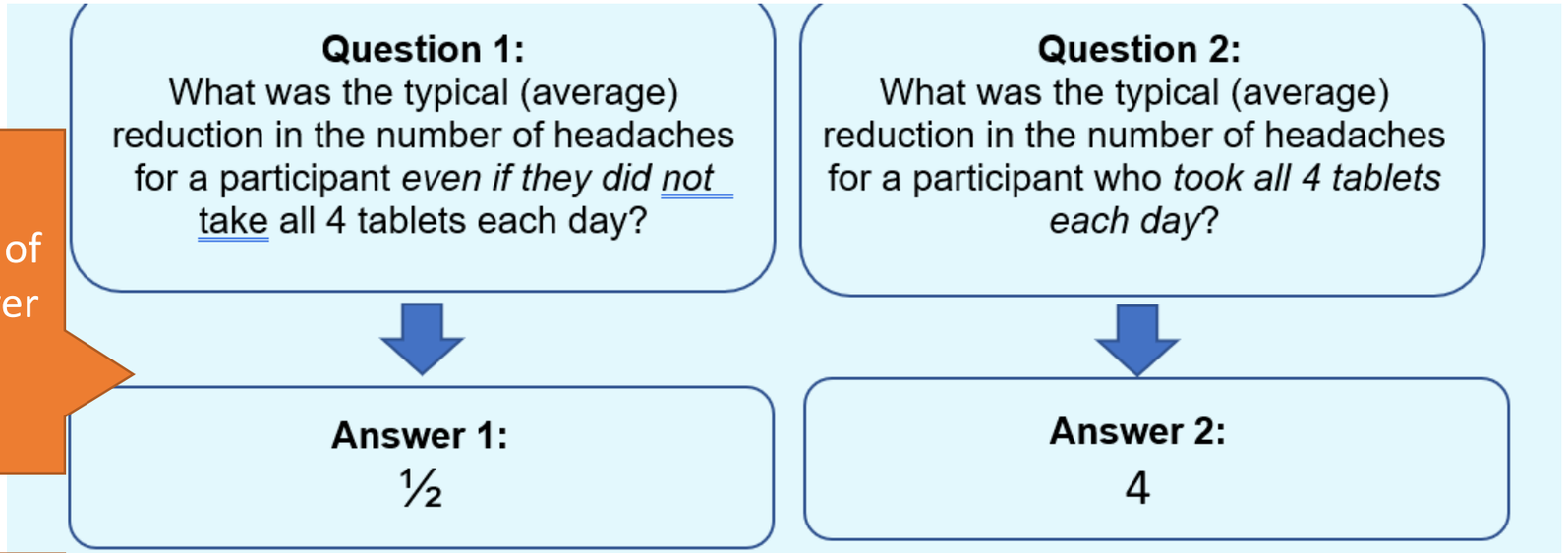
What are the implications of choosing one estimand over another?

Why it is important to get involved



As different questions can lead to different impressions, it is important when helping with a clinical trial you know what questions are going to being asked

esearchers would like your opinion on this so that the question that matters most to you will be addressed

Discussion

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- There was support from public partners to use this tool at the design stage of a trial
- The tool presented today will be available online soon (e-mail update to participants)
- The results & recommendations to facilitate involvement of public partners in estimands are in line with our previous work in PPI in numerical aspects
- In particular:
  - Importance of clear and jargon-free communication to enable involvement
  - Getting involved at the start
  - Different levels of interest
  - Storytelling & analogies


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Discussion

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- This is a first step into facilitating involvement of patients in estimands discussions
- The tool needs to be assessed with larger groups of patients and in different context
- We are interested in hearing your feedback/experience once you have tested the tool
- Should estimand information be available for those consider taking part? How?

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Thank you

If you have any questions, please contact:

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