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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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-
- 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?



Offers a new perspective

Potential to improve research quality

Moral imperative

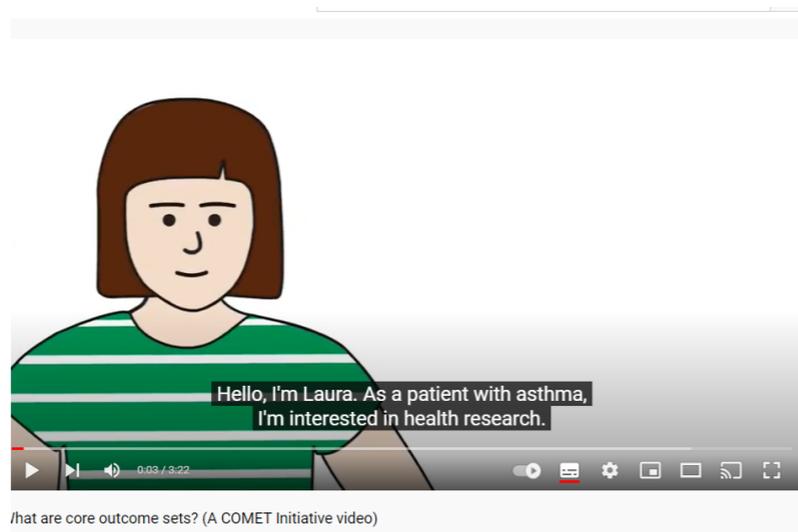
Mandatory

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)



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

RESEARCH

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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



VIEWPOINT ARTICLE |  Open Access |  

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Ailish Hannigan BSc, PhD 

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

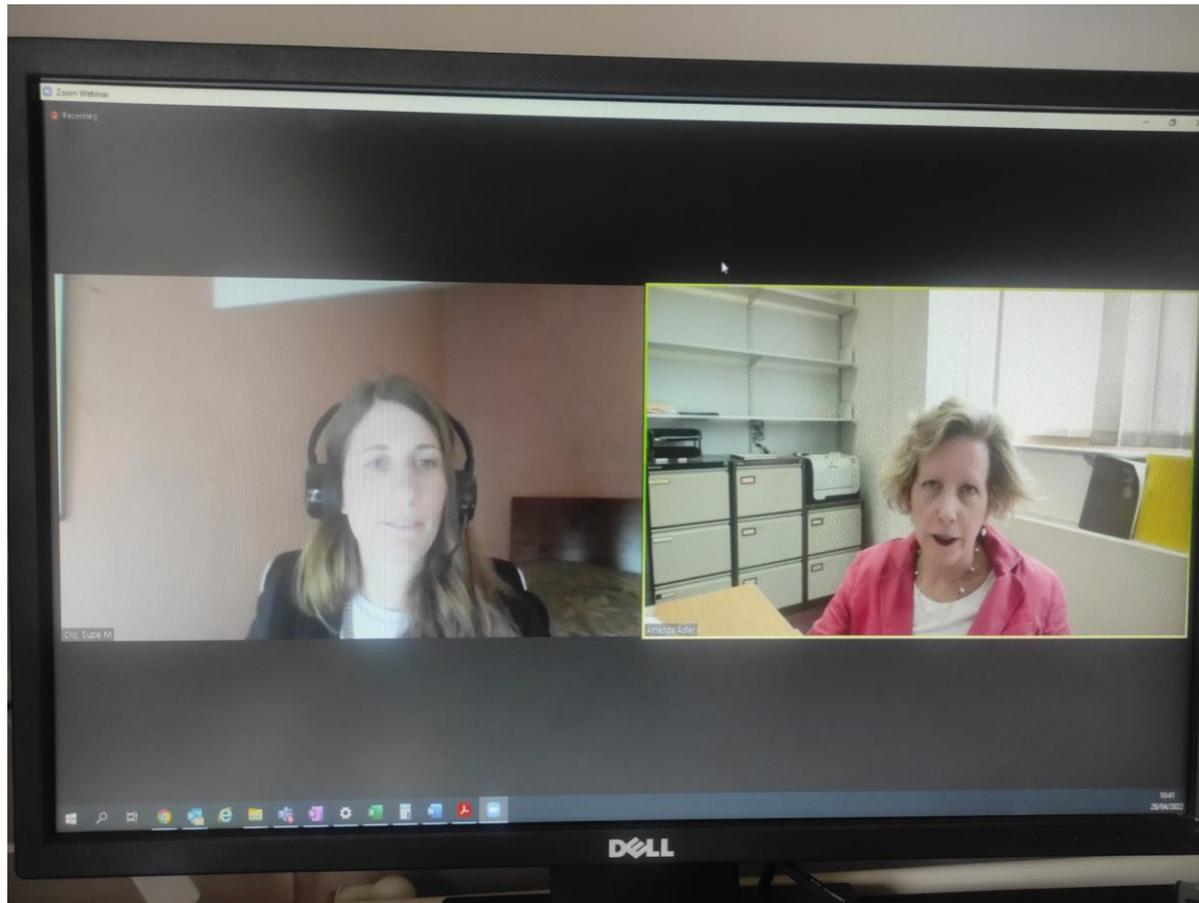
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 SECTIONS

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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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Zoom Webinar

Recording

Recommendations for implementing estimands framework

1. Promote the use of the **estimand thinking process** as a tool to establish clear links between trial objectives, estimands (treatment effects), choice of trial design, trial conduct and statistical analysis.
2. Where possible use **non-technical language** to encourage cross-functional collaboration and discussion about estimands and make estimand thinking a routine part of clinical development.
3. Ensure **clinical trial teams, investigators and patients** are aware of the need to collect all data which are essential to evaluate the primary (and key secondary estimands) in order for missing data to be minimized.
4. Focus on the data that will form the basis for the analysis of each estimand that reflects both the patients and the observations to be included.
5. Share case studies illustrating how to incorporate estimands in clinical trial protocols and statistical analysis plans, and how to communicate estimands and results in clinical study reports and publications.
6. Offer drop-in consultation sessions allowing teams to access timely advice from experts.
7. Obtain feedback from regulatory agencies and other key stakeholders on proposed estimand and estimation strategies, including justifications, as early as possible. Share this feedback across teams.
8. Provide trainings and host seminars including diverse and cross-functional facilitators to promote discussions about estimands in the broader scientific community.

Chrissie Fletcher

Audio Settings ^

Chat Q&A 28

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Type here to search

- 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?



- 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

The screenshot shows the top portion of a research article page from the Journal of Clinical Epidemiology (JCE). The header includes the journal logo and navigation links like 'Submit Article', 'Log in', 'Register', 'Subscribe', and 'Claim'. Below the header, the article title is displayed: 'Patients and investigators prefer measures of absolute risk in subgroups for pragmatic randomized trials'. The authors listed are Eleanor J. Murray, Ellen C. Caniglia, Sonja A. Swanson, Sonia Hernández-Díaz, and Miguel A. Hernán. The publication date is July 02, 2018, and the DOI is provided. A 'Check for updates' button is visible. The abstract section is titled 'Abstract' and contains the following text: 'Objectives Pragmatic randomized trials are important tools for shared decision-making, but no guidance exists on patients' preferences for types of causal information. We aimed to assess preferences of patients and investigators toward causal effects in pragmatic randomized trials.'

“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

Trials that
matter to
patients

Involve early on

Education &
communication

Change of
culture

- **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

Reliable off-road?

Achieves high speed?

Back seats suitable for children?

Like buying a car, clinical trials ask different and very specific questions to find suitable new treatments for patients, for instance:

Analogy
Visual story

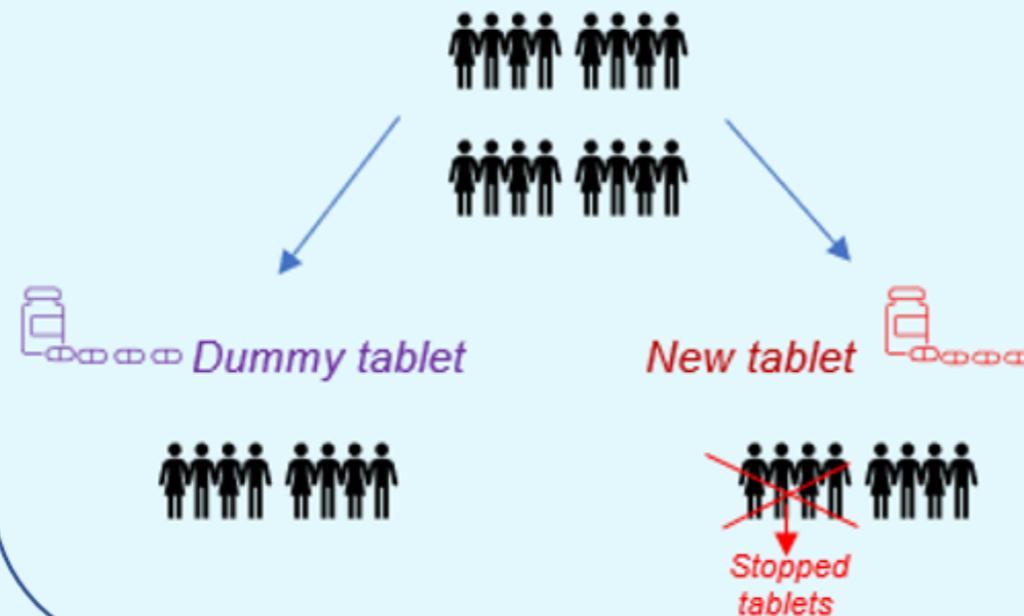
Tool - example

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)

What are the implications of choosing one estimand over another?

Question 1:
What was the typical (average) reduction in the number of headaches for a participant *even if they did not take all 4 tablets each day?*



Answer 1:
 $\frac{1}{2}$

Question 2:
What was the typical (average) reduction in the number of headaches for a participant who *took all 4 tablets each day?*



Answer 2:
4

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

- 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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-
- 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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