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

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)
Patient and public involvement in statistical aspects of trials

• 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
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?

- 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

“Patients, when they expect to adhere, prefer adherence-adjusted results, such as per-protocol effects.”
Our initial work in this field

• 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

• 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

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

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

• 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
Discussion

• 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?
Thank you

If you have any questions, please contact:

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