



MRC-NIHR Trials Methodology Research Partnership: Webinar recording

Patient and public involvement in numerical aspects of trials (PoINT)

Presented by Beatriz Guolao (University of Aberdeen)

25 January 2022

On behalf of the Health Research Board Trials Methodology Research Network



The slides are also available below.

For any queries, please contact uktmn@nottingham.ac.uk

<https://www.youtube.com/watch?v=K1QtnfDsXKY>



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2017



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Promoting Excellence in Health Services Research

PoINT (Patient and public Involvement in Numerical aspects of Trials)

Beatriz Goulão

HSRU is core funded by the Chief Scientist Office of the Scottish Government Health and Social Care Directorates. The author accepts full responsibility for this talk.



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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)*
- Increased over the years, but quality varies a lot and tokenism remains an issue
- Public partners are most frequently involved in steering committees and protocol development, but interest in PPI in trial methodology has raised

- 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

Beyond subjective and objective in statistics

Andrew Gelman

Columbia University, New York, USA

and Christian Hennig

University College London, UK

[Read before The Royal Statistical Society on Wednesday, April 12th, 2017, Professor P. J. Diggle in the Chair]

Summary. Decisions in statistical data analysis are often justified, criticized or avoided by using concepts of objectivity and subjectivity. We argue that the words 'objective' and 'subjective' in statistics discourse are used in a mostly unhelpful way, and we propose to replace each of them with broader collections of attributes, with objectivity replaced by *transparency*, *consensus*, *impartiality* and *correspondence to observable reality*, and subjectivity replaced by awareness of *multiple perspectives* and *context dependence*. Together with *stability*, these make up a collection of virtues that we think is helpful in discussions of statistical foundations and practice. The advantage of these reformulations is that the replacement terms do not oppose each other and that they give more specific guidance about what statistical science strives to achieve. Instead of debating over whether a given statistical method is subjective or objective (or normatively debating the relative merits of subjectivity and objectivity in statistical practice), we can recognize desirable attributes such as transparency and acknowledgement of multiple



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

[Check availability](#)

 SECTIONS

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Abstract

-
- There are numerous statistical and numerical aspects of trials that are up for discussion, but often the discussion involves statisticians, clinicians or other researchers
 - How can we better involve patients in these discussions?

What do we mean by...?

- Numerical aspects
 - ‘any aspects of a trial where people measure (or plan to measure), manage, analyse or share information that is presented as one or more numbers’



Application to funder



Pre-set-up



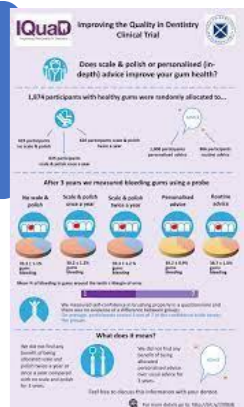
Set-up & management



Data analysis



Dissemination



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

Trials

RESEARCH

Open Access

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 in the analysis: "Determinants of PPI in numerical aspects", "Identity and roles", "Impact of involving patients and the public in numerical aspects". Patient and public partners believed being involved in numerical aspects of research is important and should be facilitated, but communication about these aspects needs to be clearer. An environment and relationship with researchers that facilitates that will include time for discussion, support to improve knowledge

- Stage 1: Focus group with public partners to discuss experiences and interest in involvement in numerical aspects of research
- Stage 2: Prioritise patient and public involvement in numerical (number) aspects of trials

Stage 1: What are patient and public partners experiences?

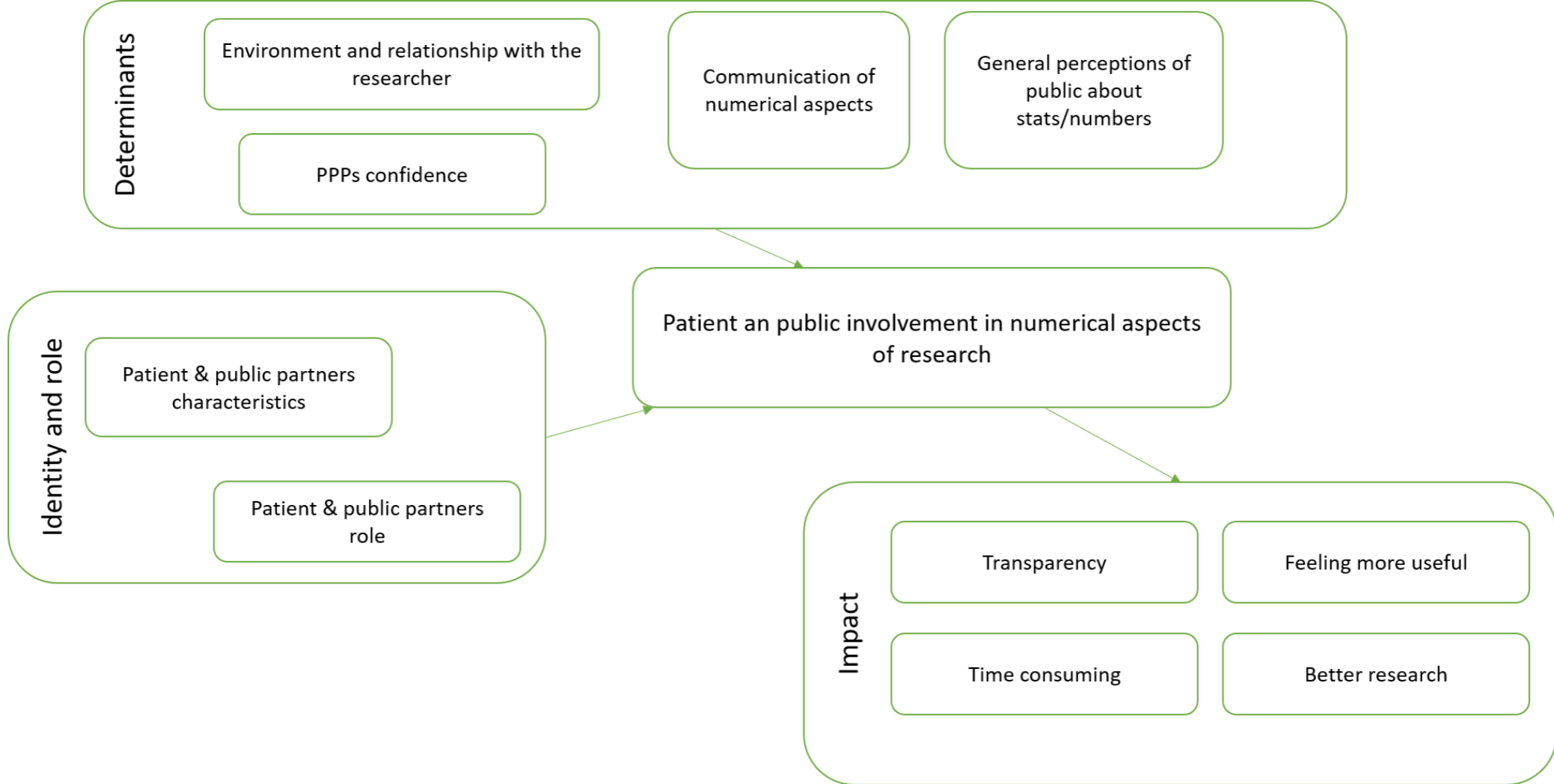


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We aimed to understand public partners' experiences and opinions regarding their involvement in numerical aspects of research

- online focus groups with public partners recruited via online platforms and analysed using inductive thematic analysis
- Eligible participants were
 - adults, UK-based public partners

Stage 1: Results



Theme 1: Determinants of PPI in numerical aspects



- **Subtheme: Relationship with researcher and research environment (key in ensuring partners feel comfortable and confident asking questions about numbers)**

“It’s doing it in such a way that you’re not devaluing the other person; you’re not making them feel as though they are stupid, and you really want to engage with them and understand their perspective, and that’s people skills.” [P2, FG1]

Theme 1: Determinants of PPI in numerical aspects



- **Subtheme: Public partner's confidence**
 - Numerical aspects are seen as particularly challenging to discuss and even intimidating to some, so confidence is key

“But I'd like to learn a bit more, so I could ask questions and start to be more effective in this kind of setting where there's a whole bunch of things I don't know about, as well as the numerical stuff. But I sense that the clinicians aren't that confident either and they just get in a statistician as soon as they get to the numbers bit.” [P3, FG2]

YOU'RE THREE
STANDARD DEVIATIONS
ABOVE THE NORM



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LOVE LETTER FROM A STATISTICIAN

Theme 1: Determinants of PPI in numerical aspects



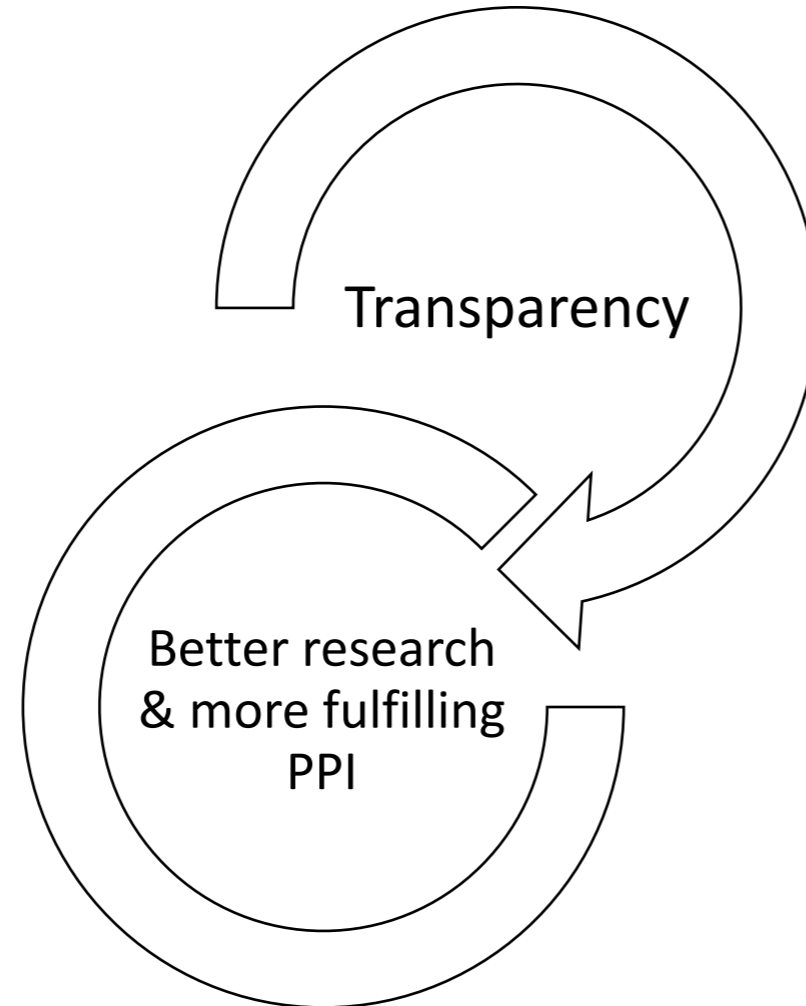
- **Subtheme: Communication of numerical aspects**
 - Participants strongly emphasised the importance of communicating in a clear and non-technical way
 - **“Somehow or another, I think there needs to be a statistics for dummies book. We had, several years ago now [...] a speaker at one of the consumer forum meetings [...] he came and talked to us, and somehow or another, statistics became very human, and it needs someone like him, someone with his skills, to write statistics for dummies.” [P1, FG2]**



Theme 2: Identity and role

- Public partners felt they should be involved in the assumptions that go into defining numerical aspects and the analysis of research
- Public partners' role in the analysis was felt to be less obvious and more controversial
- Public partners showed interest on the translation and dissemination of the outputs to a wider audience.
- **“So I think we should leave the statisticians to do the real heavy duty stuff, but I think we need to be involved at both the start and the end of the process so that the ordinary person in the street can understand and see what’s going on.” [P4, FG1]**

Theme 3: Impact



- Scrutinise researcher's assumptions
- Discuss them openly



Stage 1: Reflections

- Public partners believed being involved in numerical aspects of research is important and should be facilitated, but communication about these aspects needs to be clearer.
- An environment and relationship with researchers that facilitates that will include
 - time for discussion,
 - support to improve knowledge and confidence,
 - clear language and definitions &
 - trust



Stage 2: What numerical aspects are a priority?

- We aimed to identify priorities, according to multiple stakeholders, around the most important numerical aspects in trials to involve patients and the public.
- We focused on a sub-set of numerical aspects - either numbers or their interpretation (for example, target differences, recruitment projections)
- Online priority setting meeting with UK- and Ireland-based stakeholders and followed James Lind Alliance methodology.

Stage 2: The priority setting meeting



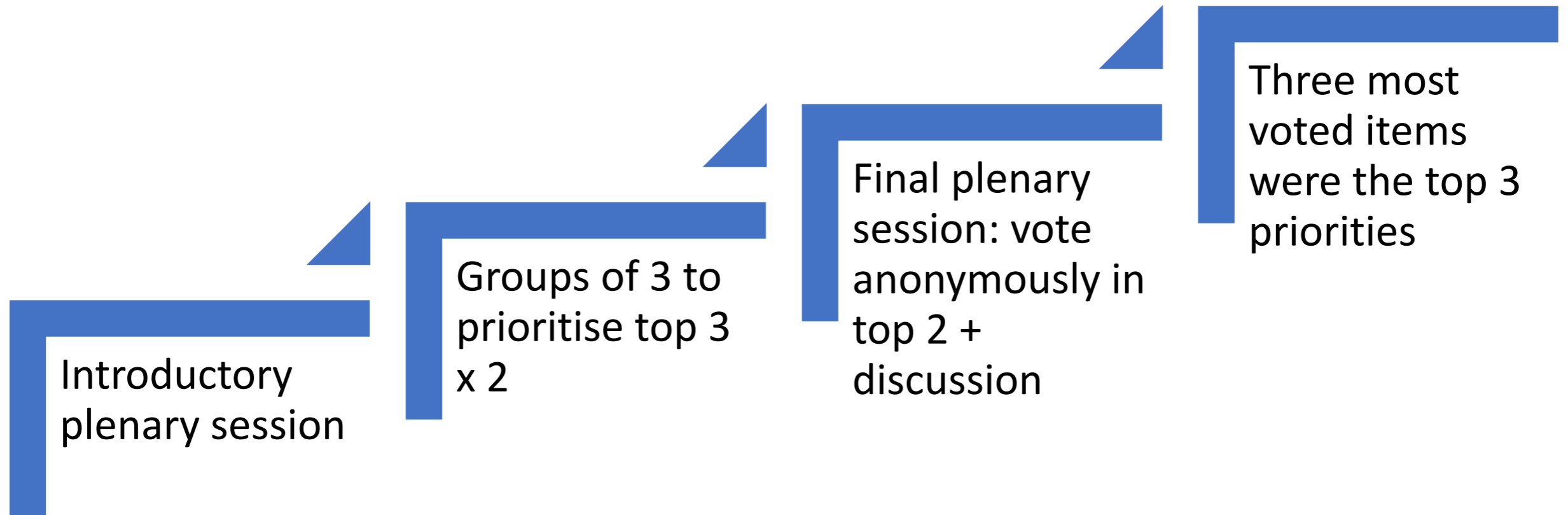
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-
- **14 experts from different backgrounds** (including trial management, statistics, patient and public involvement coordination and three experienced public partners in trials)
 - **12 numerical aspects were pre-selected via:**
 - Literature review
 - Expert knowledge
 - Survey of trialists

Stage 2: The priority setting meeting

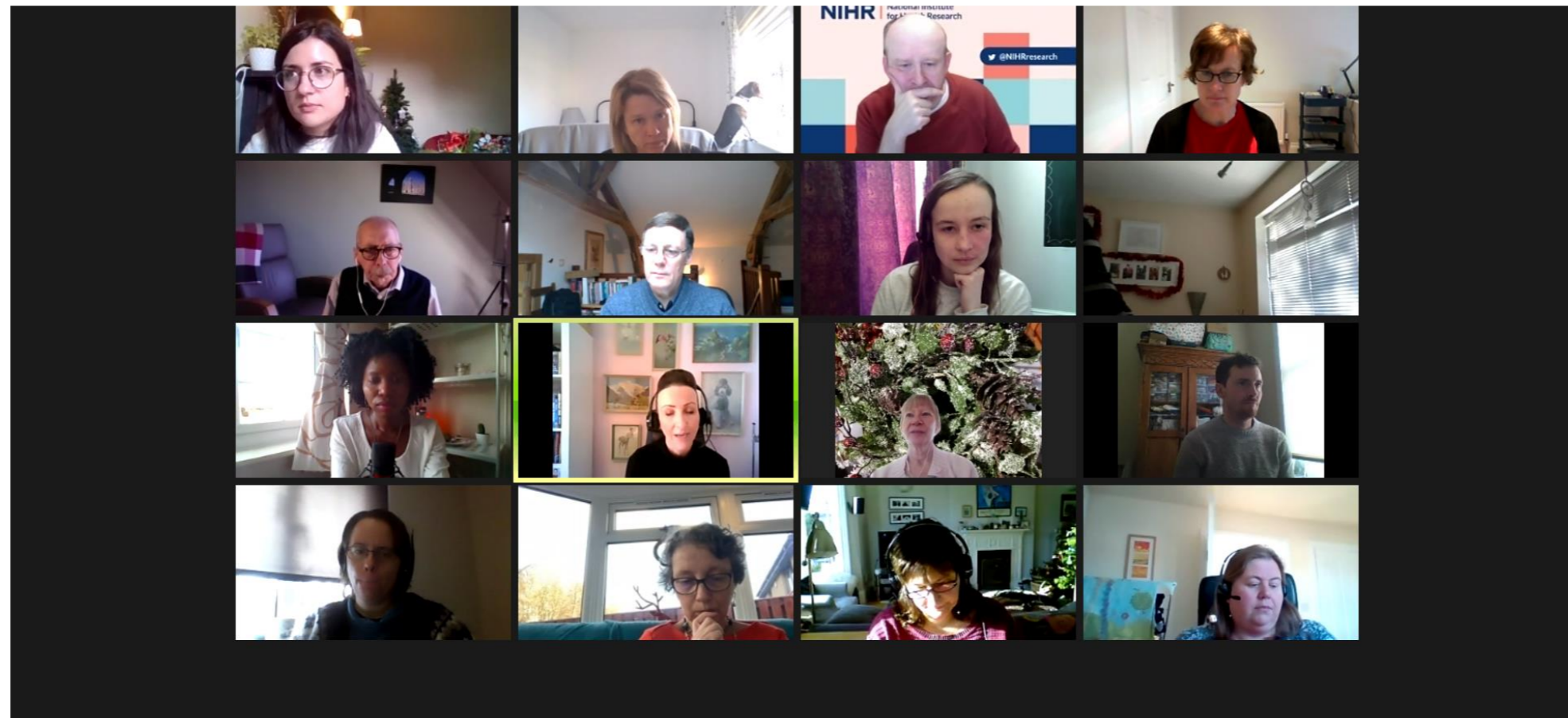


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Zoom Meeting



Stage 2: The Guide

POINT

Involving Patients and the Public in Numerical Aspects of Trials Prioritisation Workshop

Guide to Each Numerical Aspect

This document provides further information on each of the numerical aspects that will be prioritised for research. It accompanies the *Participant Worksheet*, which you have been asked to complete and bring to the workshop.

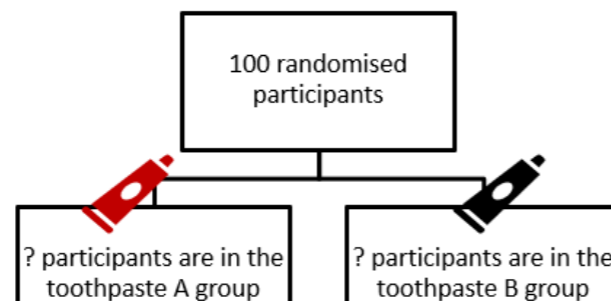
What is a numerical aspect of a trial?

We defined numerical aspects as any quantifiable or measurable aspect of a trial and/or their interpretation and dissemination.

How were these numerical aspects selected?

Through a review of the literature along with expert discussion. However, this is not necessarily an exhaustive list of all possible numerical aspects of trials that patients can be involved in. Examples about each numerical aspect are adapted from published literature, although they did not necessarily involve patients in the original studies.

We are introducing a fictional trial to illustrate what each of the numerical aspects mean. Our fictional trial aims to test whether a strong **toothpaste A** improves dental pain, measured from 1 (pain free) to 10 (very painful) compared with a weaker **toothpaste B**. In this trial we aim to recruit 100 participants.



<https://www.abdn.ac.uk/hsru/what-we-do/tools/index.php>

Stage 2: The 12 numerical aspects

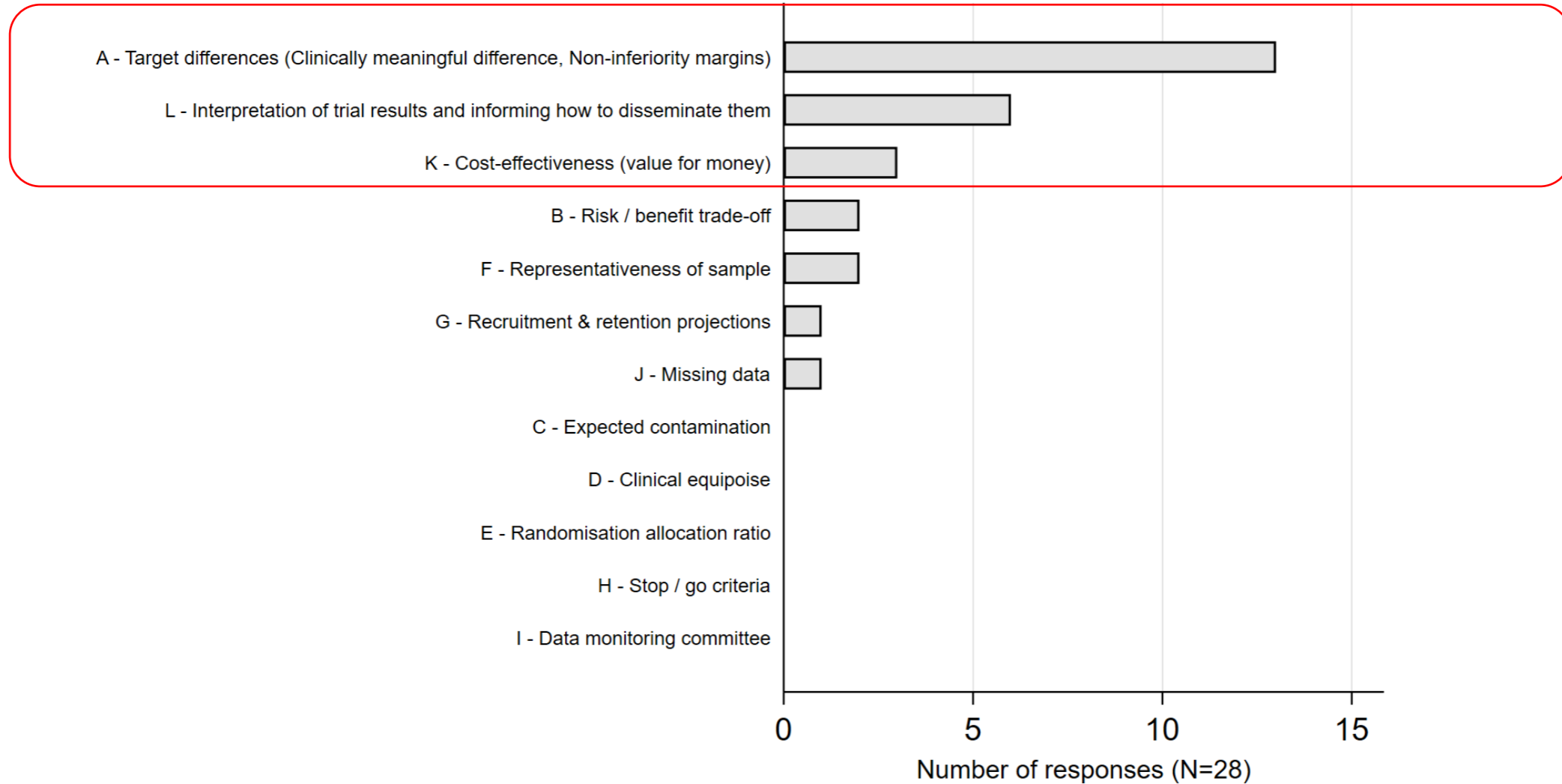


-
- A. Target differences (clinically meaningful differences and non-inferiority margins)
 - B. Risk benefit trade-off
 - C. Expected contamination
 - D. Clinical equipoise
 - E. Randomisation allocation ratio
 - F. Discussions about representativeness of sample
 - G. Recruitment and retention projections
 - H. Stop/go criteria
 - I. Data monitoring committee discussions
 - J. Missing data
 - K. Cost effectiveness
 - L. Interpretation of trial results

Stage 2: Results



Numerical aspects where patient and public involvement should be prioritised





Stage 2: Results

- Target differences were consistently selected as part of participant's priorities because they were seen as the closest numerical aspects to patient's experience ("what is meaningful to patients?");
- Interpretation of results and dissemination of findings were considered important to improve communication with patients and trial participants and to ensure implementation of findings;
- Value for money was also seen as close to patient's experience and as a key piece in health policy decision making.

12 NUMERICAL ASPECTS OF CLINICAL TRIALS

- A. Target differences
- B. Risk benefit trade off
- C. Expected contamination
- D. Clinical equipoise
- E. Randomisation allocation Ratio
- F. Discussions about representiveness of sample
- G. Recruitment and retention projections
- H. Stop/go criteria
- I. Data monitoring committee data
- J. Missing data
- K. Cost effectiveness
- L. Interpretation of trial results and their dissemination.

Why are we doing this?
There is a murkiness about this

POINT FOCUS GROUP PRIORTISATION




PRIORITIES

- 1 A Target differences
- 2 L Interpretation and dissemination
- 3 K Cost effectiveness

How can we facilitate this?

- Survey 187 respondents (trialists in the UK)
- Used TDF to identify barriers and facilitators to PPI in numerical aspects of trials
- Examples of relevant domains
 - Knowledge
 - Skills and beliefs about capabilities (of partners)
 - Beliefs about consequences

Original research

Patient and public involvement in numerical aspects of trials: a mixed methods theory-informed survey of trialists' current practices, barriers and facilitators 

 Beatriz Goulao ,  Camille Poisson ,  Katie Gillies

Correspondence to Dr Beatriz Goulao; beatriz.goulao@qabdn.ac.uk

Abstract

Objective We aimed to find out if trialists involve patients and the public in numerical aspects of trials, how and what are the barriers and facilitators to doing it.

Design We developed a survey based on the Theoretical Domains Framework. We used a mixed methods approach to analyse the data and to identify important domains.

Setting Online survey targeting UK-based trial units.

Participants Stakeholders working in UK-based clinical trials, 18 years old or over, understand English and agree to take part in the study.

Outcome measures Trialists' behaviour of involving patients and the public in numerical aspects of trials and its determinants.

Results We included 187 respondents. Majority were female (70%), trial managers (67%) and involved public and patient partners in numerical aspects of trials (60%). We found lack of knowledge, trialists' perception of public and patient partners' skills, capabilities and motivations, scarce resources, lack of





Taking the conversation forward

MAKING THE DIFFERENCE

The experience, voice and presence of patients with research

About me

A History of Patient Involvement (particularly in cancer research)

20TH APR 2021 by DEREK OBE

FIGURING IT OUT

UNCATEGORIZED

NUMBERS, PATIENT, PPI, PUBLIC INVOLVEMENT, STATISTICS

1 COMMENT

Reflections on Patient/Public Involvement with Numbers, Statistics and Data

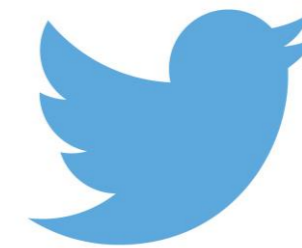
Derek C Stewart, Lynn Laidlaw, Irene Soulsby and Beatriz Goulão

*The following notes were initially written in preparation for a meeting about **Patient, Public Involvement with Numbers, Statistics and Data** run by Beatriz Goulão in December 2020. Since then, it has developed into this article with contributions from the public partner participants and Bea.*

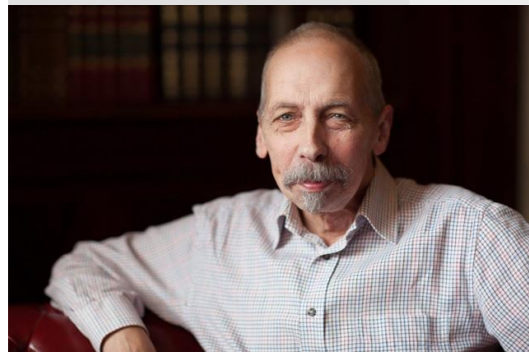
It seems to us that that the active involvement of patients and the public with data, statistics and numbers is more than just possible – it adds value! The features and benefits we have categorised into tasks we were being invited to consider. It was this might have value.

ore ab
nd and

ow already rather than
d/or statistics!



#PPINumbers
1 hour tweetchat
100 tweets
128k impressions



What comes next?



thebmj covid-19 Research ▾ Education ▾ News & Views ▾ Campaigns ▾ Jobs ▾

Research

Evaluating the credibility of anchor based estimates of minimal important differences for patient reported outcomes: instrument development and reliability study

BMJ 2020 ; 369 doi: <https://doi.org/10.1136/bmj.m1714> (Published 04 June 2020)

Cite this as: BMJ 2020;369:m1714



What comes next? INITIAL



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INvolving patients and the public In sTatistical Analysis pLans

- Explored creative methods to communicate about statistics
- Delphi survey coming soon – February 2022



What comes next?



Estimands project
SAVE THE DATES – 28th and
29th of April (online)

Eliciting and incorporating patient's opinions about missing data in randomised controlled trials

Aberdeen University > Institute of Applied Health Science

Dr Beatriz Goulao Friday, February 18, 2022

Competition Funded PhD Project (Students Worldwide)

Aberdeen United Kingdom Epidemiology Medical Statistics Medicine Statistics

About the Project

Missing data occurs when data is unavailable to be analysed and is a common challenge within clinical trials that can have serious consequences for the validity of results. The analysis of trials with missing data usually assumes the missing data are "missing at random", i.e. given an individual's past observed data, their probability of dropout does not depend on their present (or future) unobserved outcome (reference: <https://bit.ly/307KGTW>).

In many settings this assumption is implausible. For this reason, it is crucial to develop methods to assess the robustness of conclusions to departures from the missing at random assumption. Since we cannot base assumptions on



About the Project

Funding Notes

Email institution

Institution website

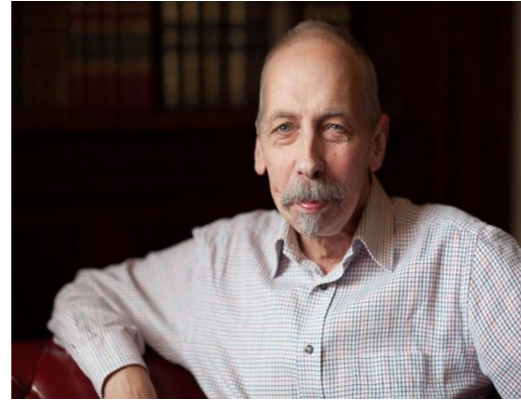
Add to shortlist



Thank you



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Thank you

If you have any questions please contact:

- Beatriz Goulão
- beatriz.goulao@abdn.ac.uk
-  @beagoulao

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