Health Research Board
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Trials Methodology Research Network

A guiding principles roadmap

“A sense of elsewhere…”

Patient and Public Involvement with Trials Methodology Research

Health Research Board – Trials Methodology Research Network

Written by:
Prof Derek Stewart, Dr Sandra Galvin and Prof Declan Devane
HRB - Trials Methodology Research Network, University of Galway, Galway, Ireland.

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Address:
HRB-TMRN National Office, Centre for Health Research Methodology,
School of Nursing and Midwifery, Room 235, 1st Floor, Áras Moyola, University of Galway.
t: 353 91 494492
e: hrb-tmrn@universityofgalway.ie
w: www.hrb-tmrn.ie
X: hrbtmrn

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Introduction

A Sense of Elsewhere

Colm Tóibín, the writer, describes Wexford on his early childhood visits as having a sense of elsewhere, in his book - *A Guest at the Feast*. The phrase portrays that feeling of being in another place - somewhere perhaps exclusive, unfamiliar, strange, more complex, yet potentially interesting. Tóibín's use of the term "elsewhere" conveys a likely mixture of nostalgia, curiosity, and the enchantment of discovering or experiencing something new and different from the familiar. It highlights the ability of certain places to leave a lasting impression, shaping our memories and perceptions in ways that feel both foreign and intimately personal.

A sense of elsewhere may also be a useful means of capturing the concerns, of both the public and researchers alike, when involving the public and patients in methodology research.

Trials methodology research, which focuses on the methods, practices, and processes used to conduct, analyse and report clinical trials, can feel as much like somewhere else for the public and patients as well as for researchers.

However, such involvement can provide specific and deliberative improvements to inform and enhance our understanding of what such studies are and how to conduct them. Simply by improving the methods that are used to study health care experience, we improve more of the subsequent studies.

HRB – Trials Methodology Research Network (Ireland)

The HRB-Trials Methodology Research Network has been at the forefront of actively involving patients and the public in many aspects of its work across the island of Ireland since 2014. The Network is also a member of the MRC NIHR Trials Methodology Research Partnership (MRC-TMRP), which brings together a number of networks, institutions and partners working in trials and trials methodology research. The Network is also a National Partner in the HRB-funded PPI Ignite Network, which aims to promote excellence and inspire innovation in public and patient involvement (PPI) in health and social care research in Ireland.

Since its establishment, the HRB-TMRN has significantly advanced the integration of PPI within trials methodology research. The Network has incorporated the patient voice at the core of its governance structures through the addition of Prof Derek Stewart on its Executive Management Committee. Derek Stewart contributes extensive experience both as a patient advocate following his own cancer treatment and a senior PPI strategist. His background includes collaborating with international organisations to develop and implement strategic roadmaps and guidelines that enhance PPI which led to his honorary professorship at the University of Galway.

On an operational level, the Network prioritises the allocation of seed grant funding to projects that incorporate PPI into their proposals and has also taken a leadership role in forming priority-setting partnerships with the James Lind Alliance to ensure the patient and public voice is included within the trials methodology research agenda nationally and internationally.

In 2024, the Network formally joined EViR (Ensuring Value in Research), a forum for funding organisations from around the world to collaborate to develop new approaches to increase the value of health-related research. The core guiding principles agreed by members, such as the HRB-TMRN, start with Principle 1: *"Health-related research agendas and priorities should be set with the meaningful involvement of those who will use and be affected by health-related research"*.

Other practical ways the Network bridges the gap to support PPI in trials methodology are offering free places on all its training programmes to members of the public/patients, and including the patient voice and experience in its invited speakers and chairpersons at key events, such as the annual Trials Methodology Symposium. The Network successfully celebrates, advocates and encourages the active involvement of the public and patients in all aspects of its work.

The HRB-TMRN website provides an introduction to being involved with trials methodology co-developed with public contributors and researchers across Ireland and the UK.

Background

Numerous areas of uncertainty in the design, conduct, analysis, and reporting of clinical trials could benefit from more open discussion and a cultural shift in thinking and practice within trials methodology research. We can improve how trials are designed, conducted, analysed, and reported to researchers and policymakers. The public and patients can help reduce research waste and lead to better health outcomes.

Actively involving the public and patients in discussions around these uncertainties can be a useful starting point and may be beneficial across all areas of trials methodology research, from question formation to dissemination of findings. It is also a reminder that public money is being spent and that involvement leads to greater transparency and impact.

The heart of this roadmap is exploring exactly what involvement might mean and how it might be mutually beneficial – for the research community and for public and patients. According to a recent review by Nicholls and Campbell (2024), there are already over 60 frameworks designed to involve patients and healthcare professionals in research[1]. Their review emphasizes that the priority should now be on implementing these existing frameworks rather than creating new ones, to avoid duplication and confusion. This will be used as a foundation for this roadmap.

What do we know so far about involvement with Trials Methodology Research?

There is an increasing interest and activity in PPI with trials methodology, yet it is still relatively new for both researchers and those public partners who get involved. It can feel daunting for all. The existing evidence tends to relate directly to individual pieces of research rather than all trials methodology work; therefore, this remains an area of enquiry and developing understanding. While there are many similarities with PPI in broader clinical research, there are also significant differences.

At its core, PPI with any clinical research involves meetings and dialogue between researchers and the public. The discussions often focus on communication and the interpretation of what the research is trying to achieve.

Involvement with research into the actual methods used (research on research or trials methodology research) requires a little more thought and preparation than perhaps with other clinical research studies. This may be due to the nature of trials methodology research, which is less related to a direct health experience, the complexities of trials, or a combination of both.

For the public and patients, this shift in how we look at the research takes time to absorb and assimilate. Often, both sides may not fully understand the role of the other: The public partner is not trying to become the researcher or a statistical expert, and neither are they expected to walk in the footsteps of a patient or another's life experiences.

Engaging the public and patients in trials methodology research requires shifts in perspective and understanding to ensure that everyone's roles and contributions are clear and valued.

However, we have much to learn from each other, especially about uncertainty, risk, and bias.

Effective collaboration between the public, patients, and researchers in trials methodology requires open communication, mutual respect, and a willingness to learn from each other. By working together, they can identify and address areas of uncertainty, assess potential risks and biases, and ultimately improve the quality and relevance of trials methodology research.

This collaborative approach extends beyond the direct interaction between public partners and researchers. It also involves creating a supportive environment that encourages ongoing dialogue, reflection, appreciation of diverse insights and learning across the wider research community.

By fostering a culture of openness and inclusivity, we can break down barriers, challenge assumptions, and drive meaningful progress in trials methodology research. An example of this approach is evident in the Priority III Study where Dr Nikita Burke talks about the need for "a safe space" – creating a place to talk, a coming together, making people feel welcome and having the necessary support, as well as clarity of purpose in the context of uncertainties in Rapid Reviews[1].

Equally, many researchers working in this area do not have direct contact with patients and, therefore, may also feel that sense of elsewhere. They may feel that involving the public is too "far removed" to impact their area of work. The breadth of this work and the complexities of some of the research are other important factors that require some bite-sized explanatory stages.

A primary example of two seminal trials methodology projects, which involved patients and the public as members of the research team, are the PRioRiTy I & II studies[2,3]. These studies mark some of the earliest involvement of the public in trials methodology research. These studies, which began in 2016, looked at how we might improve the process of how people are recruited (led by the HRB-TMRN) and retained (led Prof Gillies, University of Aberdeen and supported by HRB-TMRN) in randomised trials. Although the PPI was not formally evaluated, the shared interest in improving these areas created a common ground for discussion that led to this strategy. As of April 2024, the first paper in these studies by Healy et al. has been cited 87 times and accessed over 9,100 times, showing the reach and impact of the findings of this inclusive study[2].

How can diversity and accessibility in PPI be ensured?

The HRB-TMRN has worked extensively with the team at Trial Forge at the University of Aberdeen, and a key area of focus to date has been improving the diversity and inclusion of clinical trials through trials methodology research[4]. Trial teams should make every effort to ensure their study is relevant and applicable to those who will benefit from its findings (typically patients) and the healthcare professionals who will use these results in practice.

By involving people from diverse backgrounds (such as ethnic minorities, the elderly, or people with disabilities) on PPI groups and ensuring that trial designs are accessible to a broad demographic, including those often underrepresented in research, these efforts aim to enhance the relevance and applicability of research outcomes. This inclusivity ensures that the findings are more comprehensive and reflective of the population at large, thereby increasing the generalizability and utility of the research.

Why should we use an enquiry-based approach?

The unique nature of trials methodology research calls for an approach based on conversations, enquiry, and shared learning to foster a culture of transparency, inclusion, partnership, and collaboration. As both researchers and the public venture into uncharted territory, they embark on a shared journey to explore the unknowns of research methodologies. This uncertainty mirrors the very essence of trials methodology research.

To enhance trials methodologies effectively, the enquiry approach should prioritise meaningful dialogue and a clear purpose for PPI[5]. Pragmatism is essential, given the current experiences, challenges, and complexities within this landscape. A shared learning community of practice can facilitate this by encouraging a spiral approach to learning and knowledge exchange, focusing on three main strands: research on trials methodology, supporting public partners/contributors, and supporting researchers.

Spiral learning is a teaching method based on the premise that a learner learns more about a subject each time the topic is reviewed or encountered. The idea is that each time a learner encounters the topic, they expand their knowledge or improves their skill level.

This spiral approach has already proven effective in engaging more public partners with topics such as surrogate endpoints, demonstrating how involvement in one activity can build understanding and increase participation in others. The Spirit/Consort Reporting Guidelines for Surrogate Endpoints study aimed to develop consensus-driven reporting guidelines for Randomised Controlled Trials and protocols that use surrogate primary endpoints[6]. Central to the Project Management group in this study was a PPI Lead, who led the delivery of a plain language summary of the work involved to remove complex language barriers and to ensure equal participation of PPI stakeholders contributing to the study.

How might we make sense of PPI in the context of Trials Methodology Research?

Establishing a dynamic shared learning community is crucial for enhancing the tangibility and effectiveness of PPI in trials methodology research. By creating a collaborative platform that encourages two-way dialogue, researchers can gain broader perspectives on effective PPI implementation strategies, while PPI contributors can share their valuable insights and experiences. This approach aims to create a more inclusive, responsive, and enriched research environment where all stakeholders' collective wisdom and diverse viewpoints drive improvements in trials methodology.

Effective involvement in trials methodology research often starts with a clear purpose, such as addressing recruitment and retention issues, improving what outcomes are measured and reported in trials, or communicating with the public. For example, the earlier example of the SPIRIT/CONSORT Reporting Guidelines for Surrogate Endpoints study sought to establish key questions along with a plain language summary of the work, then invited other public partners to help plan a meeting. Similarly, Shiely et al. looked at PPI in outcome selection in breast cancer and nephrology trials, illustrating the need for a clear purpose[7].

By focusing on specific issues or uncertainties rather than simply trying to implement PPI, involvement becomes more meaningful and practical. Exploring the reason for involvement in the context of the methodology may reveal that the process itself is more important than finding a one-size-fits-all solution or model. Dr. Suzie Cro (Imperial College London), in a 2024 HRB-TMRN webinar, highlighted the value of using pictures, creating useful guides, and employing metaphors to explain and make the use of estimands in statistics research understandable, demonstrating the importance of effective communication in PPI[8].

What specific supports and learning are required?

To support this process, it is essential to ensure that researchers and those involved in PPI initiatives have the necessary support, training and sensitivity to engage effectively with diverse communities. An awareness and appreciation of the mutual benefit of respective stakeholders is crucial in securing a successful partnership. Additionally, it is crucial to address how to fund this work and fairly reimburse public partners, easily and appropriately not only for their expenses, but also to provide adequate recognition for their valuable contribution in shaping the research study in question.

What should we gather, share, and publish?

As we progress, it is essential to not only highlight and share the benefits, challenges, and impacts of PPI in trials methodology research but also to elucidate the subtleties that facilitated these successful partnerships. This is crucial regardless of the perceived complexity of the research area or question. Such transparency represents a pivotal advancement toward more inclusive, relevant, and impactful clinical research, ensuring that the endeavours align with the needs and priorities of the communities they are intended to serve.

How can the impact of PPI in trials methodology research be measured and understood?

Measuring and understanding the impact of PPI in trials methodology research is crucial to ensure that interventions are effective and meet the needs of the populations they are designed to serve. This can be achieved through several methods, including:

- Defining specific, measurable outcomes that reflect the goals of PPI, such as participant diversity, participant satisfaction, or the relevance of research questions to patient needs.
- Conduct studies that track the outcomes of trials over time to see how PPI has influenced the design, execution, and results of the research. This could include comparisons of trial outcomes with and without PPI.
- Using both qualitative methods (like interviews and focus groups) and quantitative methods (like surveys and statistical analysis) to gather comprehensive data on the effectiveness of PPI.
- Document and publish detailed case studies that highlight successful instances of PPI and outline the methods used, the involvement process, and the outcomes achieved.

By systematically measuring these aspects, researchers can gain a clearer picture of how PPI contributes to the scientific rigour and relevance of trials. This will ultimately lead to more effective healthcare interventions tailored to patient needs.

Guiding Principles Roadmap Aims

This roadmap describes how the HRB-TMRN will establish a sense of direction for PPI in trials methodology research rather than outline the destination through a defined strategy. This will allow for continual learning and improvement. Its intention is to help the public and patients as contributors feel they belong within, are needed for, and can impact how clinical trials are planned and conducted and how trial findings are shared. Specifically, it helps identify their role in the Health Research Board - Trials Methodology Research Network, Ireland.

Equally, our intention is to support trial teams to implement and acknowledge such involvement in a meaningful manner.

The challenge with involving the public in trials methodology research is that it requires us to create an environment that is inclusive, familiar, and accessible to all while still maintaining the engaging and thought-provoking nature of exploring new territory—transforming the sense of "*elsewhere*" into a shared journey of discovery and collaboration.

Proposal

The aim is to create and develop a Shared Learning Community that will impact the way trials methodology research is conducted in Ireland (through the HRB-TMRN) and influence this area internationally.

This work will build on the foundation laid by the workshop held in Manchester (in November 2019), led by the MRC-NIHR-TMRP, and the ongoing discussions within both the HRB-TMRN and MRC-NIHR-TMRP.

The primary aim is to support methodology research, foster better engagement between public partners and trialists / trial methods researchers. This should ultimately contribute and improving health outcomes in Ireland and the UK.

The Shared Learning Community will also contribute significantly to the PhD Scholars programme and other initiatives.

Key questions

The following key questions, as explored above, will be used as a guide for this work:

- What do we know so far about PPI with Trials Methodology Research?
- How can diversity and accessibility in PPI be ensured?
- Why we use an enquiry-based approach?
- How might we make sense of PPI in the context of Trials Methodology Research? (ensuring a clear understanding of perspectives, skills, insights, values, and contributions).
- What specific supports and learning are required to empower all involved?
- What information should we gather, share and publish?
- How can the impact of PPI in trials methodology research be measured and understood?
- How can we implement the findings?

Practical Steps

The HRB-TMRN will set up a series of three meetings (two online and one to be held, in-person, in Ireland), targeting people nationally with an interest in PPI in trials methodology research. National charity organisations, the national PPI Ignite Network and the main HRB-TMRN communications channels will be used to recruit members to this Shared Learning Community.

- **Meeting 1 – Early November 2024**
  During this meeting, a presentation will be given on the overview of trials methodology research conducted to date within the HRB-TMRN, and a proposal for the scope of the Shared Learning Community (SLC) will be decided and agreed by participants.
- **Meeting 2 – Mid-January 2025**
  Exploring the mutual benefits involving the public with trials methodology research. This meeting will facilitate in-depth discussions of the perceptions of PPI in trials methodology research and explore the unique opportunities and benefits of such work. The views and perspectives will be collated and summarised into a report, which will include items such as obstacles to involvement, value of involvement and ways to improve communication of opportunities.
- **Meeting 3 – Mid-February 2025**
  This will be an opportunity for the community to re-group and review the report from meeting 2. This will also be an opportunity to develop a plan for future work and areas of interest.

Implementation of findings

These findings will be reported to the HRB-TMRN Executive Management Committee to ensure alignment with the network's goals and objectives.

Final note: *The sense of elsewhere that Colm Tóibín described in Wexford included there being a main street with a Woolworths shop.*

The purpose of this PPI Roadmap is to make 'somewhere' to meet and learn from each other.

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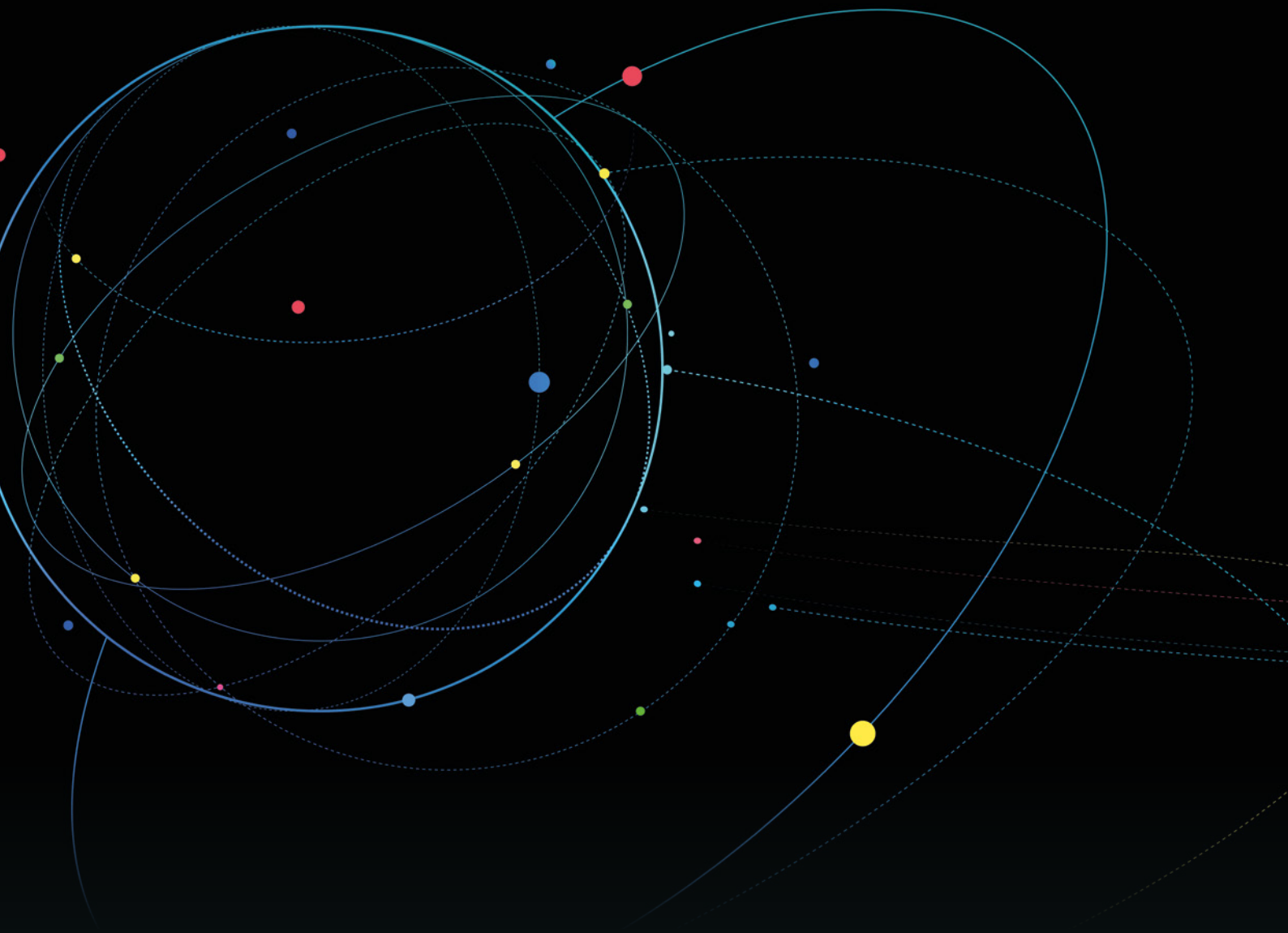

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