

An evidence base to optimise methods for involving patient and public contributors in clinical trials: a mixed-methods study

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Scientific summary

Involving patient and public contributors in clinical trials

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Scientific summary

Background

Public involvement in research is described as research being carried out 'with' or 'by' members of the public rather than 'to', 'about' or 'for' them [INVOLVE. *What Is Public Involvement in Research?* URL: www.invo.org.uk/find-out-more/what-is-public-involvement-in-research-2/ (accessed 19 June 2014)]. Increased recognition that patients and public are stakeholders in research has led to increasing calls that they be represented within that research process. This has resulted in a growth of patient and public involvement (PPI) in health research both nationally and internationally.

Little is known about how or when researchers incorporate PPI in clinical trials, or what impact may stem from that involvement. Concerns have been expressed that the existing literature is selectively reported to make the case for or against PPI, with many reports aiming to make the case or convince the sceptics about PPI. Furthermore, as much reporting has involved single case studies, generalisability of the PPI literature is limited and may provide a misleading account of how PPI is implemented and its impact. Crucially, these problems make it difficult to predict what type of involvement is most effective and where.

Patient and public involvement in research has been justified in two main ways: normatively on moral, ethical or political grounds consistent with slogans such as 'nothing about us without us', and substantively in terms of the potential for PPI to benefit research. Normative imperatives for PPI are sometimes viewed as sufficient justification regardless of any substantive impact PPI might have on research. If PPI is to be implemented then it should be done in a way that maximises the potential for benefit. In addition, as PPI requires time and resources it therefore warrants scrutiny and evaluation.

Objectives

To establish an unselected cohort of randomised trials to:

1. examine how PPI has been implemented and identify associated impact
2. systematically describe and critically evaluate the process, challenges and impact of PPI from the perspectives of the PPI contributors, chief investigator (CI) and clinical trials unit (CTU) staff.

Design

A cohort of randomised trials was established. The cohort included all randomised trials that were in receipt of funding from the Health Technology Assessment programme during 2006–10. Documentation from the two-stage application process for each trial in the cohort was requested. For each trial, data were extracted on trial characteristics and text referring to PPI in the development of the application process and after the trial was funded, along with funding board feedback and external referee comments. Surveys targeting the experience and opinions of CIs and PPI contributors of each randomised controlled trial in the cohort were developed. Semistructured qualitative telephone interviews with survey respondents were conducted. The UK Clinical Research Collaboration (UKCRC) Registered CTUs were surveyed on their experiences of PPI across trials.

Results

The cohort contained 111 trials. Seventy-three per cent (81 of 111) of CIs and 32 PPI contributors responded to the survey. All PPI contributors who were successfully contacted to complete the survey did; however, obtaining contact details was difficult in the absence of a central register. Interviews were conducted with researchers and/or PPI contributors for 28 trials.

A minority of early-stage grant applications described PPI activity within their development. Although plans for PPI activity increased within later-stage applications and once funding had been achieved, a key finding from this project was the need to instigate early PPI and the benefit of doing so.

Based on the accounts of researchers and PPI contributors, we found that most triallists are putting their plans for PPI, as described within their applications for funding, into action. However, in some cases the plans were minimal and relatively easy to execute. Many trials implemented multiple modes of PPI, which is both surprising and encouraging given that PPI was less prominent when the proposals for the trials in this cohort were being developed. Difficulties finding and retaining suitable contributors, and engaging in PPI too little too late, led triallists to say they would do things differently in future. Many reflected on how they would aim for earlier engagement next time and seek involvement from a more diverse source, such as patient panels or focus groups. PPI contributors themselves mentioned that becoming involved after the trial had begun, or infrequently, resulted in missed opportunities for them to contribute. Some referred to uncertainty about their role and many struggled with jargon, an enduring problem despite the availability of apparently straightforward solutions.

Regardless of statements about PPI in their funding application, some triallists had no expectations of what PPI might achieve, and their only motivation for including PPI was a belief that it was necessary or would help to secure funding for their trial. Such strategic minimalism may be an inevitable side effect of policies to promote or require PPI in trials. It may also reflect researchers' professed inexperience of PPI. A small number of trials did not have documented plans for PPI but all did nevertheless include some PPI, possibly influenced by reviewer and panel comments.

Well over half of the informants indicated that PPI had made a difference to the trial, or influenced the trial team, and none reported unfavourable impacts from PPI. CIs who described goals for PPI and planned its implementation in the light of these goals tended to report impact, whereas those whose goals for PPI did not extend beyond meeting perceived funding requirements usually reported little or no impact from PPI. PPI contributors who spoke of having a good relationship, particularly in terms of feeling part of the team, also tended to report impact from PPI, and both researchers and PPI contributors pointed to the importance of implementing PPI before seeking funding. Many informants believed formative PPI prior to funding was one of the most useful, credible aspects of PPI.

Despite the frequent practice and policy recommendation to include PPI contributors on steering committees, researchers and PPI contributors often reported that such oversight roles made little or no difference within a trial, particularly in contrast to managerial or responsive roles. Whether or not CIs valued PPI seemed to be linked to the goals they described and how they implemented PPI. CIs who expressed scepticism about PPI focused mainly on using PPI to meet funding requirements, whereas those who valued PPI often described in detail how it was of benefit within their trials. CIs that were sceptical of the value of PPI tended to implement it only by including PPI contributors on Trial Steering Committees. Our study confirms that some researchers seem to accord little value to PPI. It also raises the possibility that this may become a self-perpetuating cycle, with such researchers implementing PPI in ways that may provide little opportunity for it to benefit randomised controlled trials and then concluding that PPI made little difference to their trials.

Informants involved in the interviews had reservations about the need for training in PPI, particularly training for PPI contributors. Very few contributors had received training and many were reluctant to engage in it. Researchers shared this lack of enthusiasm for training PPI contributors, although both groups of informants welcomed informal induction 'conversations' to help contributors to understand their roles. There were, nevertheless, indications that current approaches to induction and support for PPI contributors were a problem. Induction seemed to provide little scope for contributors to negotiate their roles. This gap was potentially important, given that the survey results indicated a level of mismatch between areas of interest to contributors, areas of perceived need for researchers and areas of PPI impact. Support for contributors was largely implicit and focused on practical arrangements rather than on helping contributors to function in their roles. Rather than training contributors, researchers used their networks and others' recommendations to identify and select individuals who already possessed attributes perceived as important for the role. Therefore, informants tended to see training PPI contributors as redundant because, through the way they had been selected, contributors were believed to possess the necessary attributes.

Researchers described a tension between needing contributors who could provide an authentic patient perspective and needing contributors who could function in oversight and managerial roles (e.g. as members of trial steering and managerial groups respectively). Some commented that this tension could be resolved by selecting particular PPI contributors for particular roles within a trial. Although few of our informants identified the selection of PPI contributors as a training need, our findings indicate that it warrants consideration as a topic for training.

There was some evidence to suggest that the further the trial deviates from routine clinical practice, the more likely the application is to describe PPI, and PPI was particularly frequent in applications for blinded trials or trials allocating participants to placebo only. This may indicate the beginning of a risk-based approach to PPI. This was supported by the UKCRC Registered CTUs, the majority of which reported using trial characteristics to determine the approach to PPI for a trial rather than adopting the same approach across trials.

There is considerable investment in both time and resources for PPI in randomised trials. However, there is a need for increased collaboration between funders, INVOLVE and the UKCRC network of registered CTUs, to ensure that they are aware of each other's available resources, difficulties and expectations. The majority of UKCRC Registered CTUs indicated that they were in the process of changes in relation to PPI but were not currently utilising the guidance available from INVOLVE in supporting PPI contributors in their trials. CTUs should work together within the network, and with funders and INVOLVE, to bring efficiency in the ongoing developments, research, training and support related to PPI.

Conclusions

In summary, if researchers, PPI contributors and research funders wish to enhance PPI in trials they should consider how PPI can inform or benefit a trial. PPI should be planned to suit these goals. PPI contributors should be involved at an early stage with work to develop good relationships between the PPI contributors and researchers, with PPI contributions favouring responsive and managerial roles in preference to oversight committee roles. The training needs of researchers instigating PPI and PPI contributors should be considered alongside their roles and experience. Funders, INVOLVE and the CTU network should work together to bring efficiency in the ongoing developments, research, training and support related to PPI.

Effective mechanisms to obtain diversity of PPI contributors need to be explored. Selection of contributors has been identified as a training need and the use of mixed models has been suggested, to allow the benefit of experienced contributors on oversight or trial management committees and research-naïve contributors on responsive groups. However, where the aim of PPI is to gain widespread, or diversity of, opinion the role of qualitative researchers to support PPI in delivering such goals should be considered.

We recommend that funders remove PPI tick box sections from their forms and instead request a PPI-specific protocol separately requesting goals, methods and costs of PPI; this approach should enable reviewers to appraise the relevance and appropriateness of such plans. We would also advise funders against specifying the nature of PPI activity, to avoid minimalistic approaches intended solely to comply with funder requirements. We recommend increased availability and levels of funding to support pre-application PPI and the identification of contingency funds to support PPI in response to unplanned need.

We also recommend that PPI contributors be enabled to report on their activities directly to the funders, and that the UKCRC formalise requirements for registered CTUs to support PPI activity. CTUs are ideally placed to lead on the development of a risk-based approach to PPI and of resources to evaluate PPI. They would also be central to encouraging greater peer support between PPI contributors both within and between clinical trials.

Collaboration between funders, INVOLVE and the UKCRC network of registered CTUs should be increased to ensure that all are aware of each other's available resources, expectations and constraints. Such collaboration could be used to identify core materials that should be packaged for CTUs to provide to researchers and PPI contributors engaging in a trial, to enable role negotiation, manage expectations and identify training needs to enable PPI contributors to function in their role.

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