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OPEN LETTER

Patient and community engagement in clinical platform trials

[version 1; peer review: 1 approved, 1 approved with reservations]

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Abstract

Platform trials measure the effects of multiple treatments against each other and against one control group simultaneously. Such approaches have the potential to revolutionize how clinical trials are conducted. Treatments can be developed faster, comparisons be made more easily, resources can be saved, and less participants are needed for trials. Within the Innovative Medicines Initiative (IMI) EU Patient Centric Clinical Trial Platform (EU PEARL) project, a public-private consortium aimed to build a framework for patient-centric platform trials together with patients and members of communities affected by four different conditions. The structured involvement of patient and community perspectives and input in research projects is increasingly viewed as a crucial and beneficial trial design factor. However, platform trials are complex endeavours, posing the question of how to involve patients and communities meaningfully. In this paper, we present a repository for Patient and Community Engagement in Platform Trials (PaCEPT), how it was developed, and how it can be used. PaCEPT contains information on general patient and community engagement, which is applicable to platform trials, as well as materials specifically developed for platform trials. The repository can support patients and communities in understanding what platform trials are and how they can benefit them, so that they can make evidence-informed decisions about participating in a platform trial. Further, people interested in becoming actively involved as an advisor for platform trials can gain in-depth insights that can prepare them well for such a role. PaCEPT also features materials that can help researchers to learn about the benefits of patient and community engagement for trial design and execution, and how to

Open Peer Review

Approval Status ? ✓

	1	2
version 1 03 May 2023	? view	✓ view

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2. **Teresa Finlay**, University of Oxford, Oxford, UK

Any reports and responses or comments on the article can be found at the end of the article.

approach and include this stakeholder group. We encourage researchers to make use of this resource and call for evaluations of the implementation of the developed materials and the repository as a whole.

Keywords
platform trial, patient engagement, community engagement, master protocol, clinical trial, clinical trial development



This article is included in the Horizon 2020 gateway.



This article is included in the Health Sciences gateway.



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This article is included in the Healthcare and Policy collection.

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Plain language summary

After medicines are checked for side-effects they usually undergo a next phase, where a large group of people takes the medicine (treatment group) and the effect over time is compared to a group with similar characteristics who is not receiving medication or the current standard of care (control group). An emerging form of trials reduces the needed resources and speeds up this process by testing multiple treatments and compares them to each other, and to only one control group. To make trials more participant-friendly, researchers increasingly turn to patients and members of communities affected by the condition they study. Such Patient and Community Engagement, however, becomes more challenging for complex designs like platform trials. In this paper, we introduce a repository for Patient and Community Engagement in Platform Trials (PaCEPT), which can be used by patient and community members, patient advocates, and researchers, to make this process easier and well-structured.

Introduction

Platform trials—sometimes also referred to as adaptive platform trials— offer many advantages for the development of medical treatments through higher efficiency compared to trials with only one treatment arm (Park *et al.*, 2019) and have been identified as an opportunity not only for drug development but also for behavioural interventions (Gold *et al.*, 2022). Their potential has most recently been demonstrated during the COVID-19 pandemic, where research teams around the globe rushed to find treatments for the virus and its many different symptoms (Collignon *et al.*, 2021; Pericàs *et al.*, 2022; Stallard *et al.*, 2020). In most clinical phase III trials (US Food and Drug Administration, 2018) the effect of a treatment on a large sample of participants (treatment group) is usually compared to the development of the disease under no intervention, or the standard of care (the control group). Alternatively, the effect might be compared to another treatment. The core concept of platform trials is that multiple treatment groups are compared to one control group and against each other within one common framework or one 'platform', rather than running a separate trial for each treatment (Parmar *et al.*, 2017; The Adaptive Platform Trials Coalition, 2019). The simultaneous development of multiple treatments for a specific health condition overseen by one consortium can increase the speed at which treatments are developed and decrease the number of participants needed for a trial, which in turn also decreases the number of trial participants in the control group receiving no treatment or the standard of care. This can reduce the burden on participants and patients considerably and save significant amounts of time and money (Parmar *et al.*, 2017). Albeit only under specific circumstances, switching to another treatment group can also be an option for some participants who might react negatively to one experimental treatment - likewise, new arms may be added in the process (Choodari-Okooei *et al.*, 2020). This is especially beneficial for treatment development in rare diseases for which, by definition, people affected by the health condition are hard to find. Platform trials also operate with a master protocol—a common infrastructure to evaluate multiple hypotheses in a standardised way (Meyer *et al.*, 2020)—which makes it easier to compare effects and enhances the replicability of the trial. While some platform trials have already been conducted in the past in various areas, including COVID-19, prostate cancer (James *et al.*, 2016), oncology in general (Park *et al.*, 2019), and amyotrophic lateral sclerosis (Cudkowicz, 2020), a game changer would be platform trials which are jointly conducted by major players in the pharmacological industry.

Patient-centricity and community involvement

The people affected by a disease are arguably the key stakeholders when it comes to their condition. Involving them in research beyond being solely participants, has increasingly become a crucial and beneficial factor, specifically in clinical trials (Patrick-Lake, 2018) and can be a critical factor to their success, as well as to ensure that participating communities benefit (Geißler *et al.*, 2022). By including patients early in clinical trials, it is possible to receive direct feedback on the design by a patient affected by the condition and identify pitfalls that may have not been uncovered before by research. It further shows that people and their concerns are valued as individuals and can help build trust in affected communities (Duffett, 2017) and the sense of a shared goal of treating the condition together with researchers and clinicians. By having patients and affected communities involved, motivations and barriers to participate in a trial may be better understood and reimbursement expectations anticipated. Patient and community involvement can also contribute to the inclusion of condition-specific relevant measures, specifically through Patient Reported Outcome Measures (Wiering *et al.*, 2017). In fact, in a literature review including 91 papers, Vat and colleagues (2020) find that patient engagement initiatives in medicines research and development add value through 18 different benefits across patient partners, on societal level, research participants, researchers, research institutes, funders, industry, and regulators. However, involving patients and communities is not simply shoehorning a patient to an advisory board, but a serious endeavour that should be structured, guided, and follow clear ethical standards (Beier *et al.*, 2019).

The EU Patient-cEntric clinicAl tRial pLatforms (EU PEARL) (https://eu-pearl.eu/) (Cash-Gibson *et al.*, 2021) project aims to establish a framework for platform trials that centres around patient needs through a strategic partnership between multiple pharmaceutical companies, clinicians, researchers, academics, regulators, as well as patient and community representatives. To that end, the EU PEARL consortium (https://eu-pearl.eu/partners/) has worked with the European Patients' Forum (EPF) (https://www.eu-patient.eu/) —a not-for-profit umbrella organisation of patient and community organisations—to include representatives of communities (Patient and Community Advisory Group; PAG) affected by the four disorder/disease areas included in the project: major depressive disorder (MDD), tuberculosis (TB), non-alcoholic steatohepatitis (NASH), and neurofibromatosis (NF). In addition to the inclusion of patient and community input in itself throughout the project, one key output of EU PEARL is therefore a repository containing resources on engaging patients and communities in future platform trials.

PaCEPT: A Repository for Patient and Community Engagement in Clinical Platform Trials

Repository concept and creation

When building the foundations of the repository (https://patientengagement.synapseconnect.org/initiatives/eu-pearl-patient-and-community-engagement-in-platform-trials?tag=32), we first identified key stakeholders who could benefit from it. We concluded it should help patients or people affected by a disease or health condition in understanding what platform trials are, i.e., what makes them different from regular clinical trials, and which benefits they bring for affected communities as a whole, as well as for individuals who participate in such a trial. Further, we wanted to support patient and community representatives who are interested in actively shaping a platform trial by drawing on their personal or shared experience with the disease or trial participation. Lastly, researchers and funders interested in and curious about how they can actively involve patients and communities in their platform trials should also have resources to help them get started in the process and an opportunity to learn about best practices.

While EU PEARL is, to the best of our knowledge at the time of writing, the first project that includes an effort to put together a repository for patient and community engagement (PCE) specifically in platform trials, much work already exists already for PCE in clinical research in general. As such, we conducted a review[1] of the landscape to identify already existing materials for clinical research and trials which can also be used to involve patients and communities in platform trials (see Jarke & Consortium, 2023), while also identifying which gaps would be left in terms of materials which need to be developed. Further details on these steps can also be found in the upcoming technical report (Strammiello *et al.*, forthcoming). We found that the majority of PCE activities applicable to platform trials are already covered by existing materials and can be used in platform trials just as they are used in standard trials. All these materials are now included in the PaCEPT repository.

What is different in platform trials for patient and community engagement?

Materials not covered by the existing literature were those related specifically to platform trials, which led us to the development of infographics and a video explaining platform trials to lay audiences, a video showing a trial participant and a researcher answering the same questions (to provide users with an example of hands-on experience) adding a list of previous and active platform trials (so that interested parties could see areas in which platform trials have been conducted), as well as a list of potential benefits for participants and communities (Figure 1). In many cases—including decisions regarding their health—people do not have all relevant information, and even if they have, they are often not rational decision-makers (Jarke *et al.*, 2021). As many are likely not intrinsically motivated to learn about specific scenarios such as participating in a platform trial, it was important to break down benefits in a short and intuitive manner. These benefits were identified through a survey among disease and methodological experts across the consortium, as well as members of the PAG, then summarised and structured by the lead author

[1]Please note that patient engagement resources are primarily not scientific papers. As such, the term review here does not refer to a systematic literature review of databases, but rather an iterative process in which patient and community engagement experts involved in EU PEARL provided a list of known resources followed by a web search

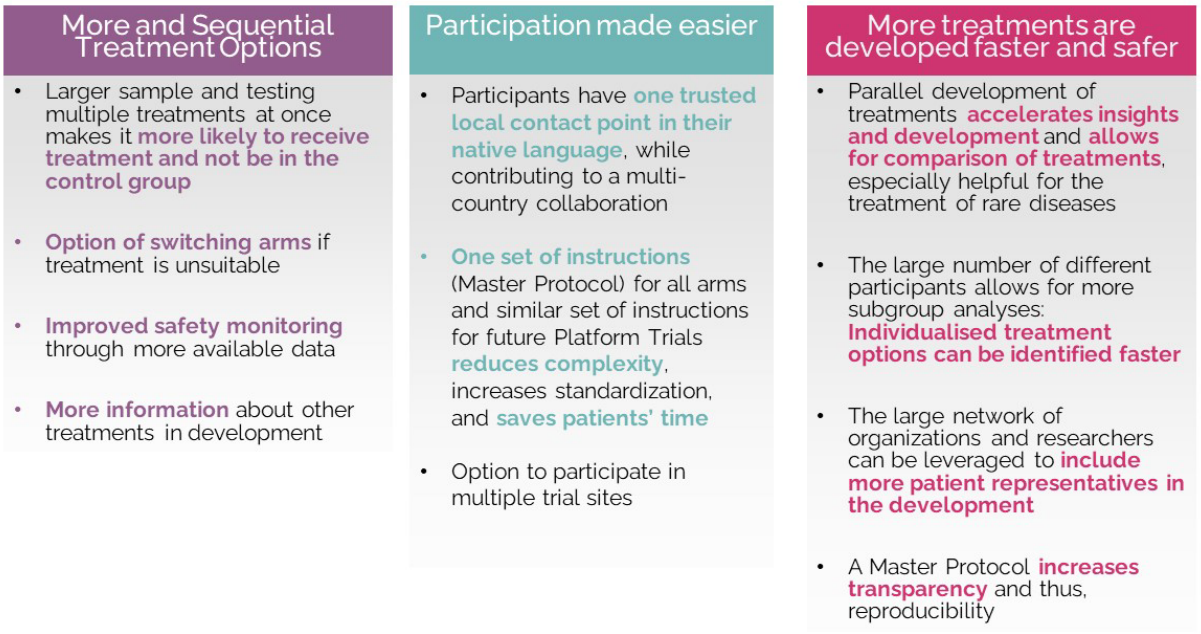


Figure 1. Potential benefits for patients and communities in platform trials.

of this paper, and then again checked for applicability and limitations by PAG and specialised consortium members. We specifically label these benefits as 'potential' because they may not always be applicable, or only be applicable in certain disease areas or specific circumstances (for detailed explanations and comments regarding applicability and limitations of these from consortium experts, see Jarke & Consortium, 2023).

In addition to explaining the advantages that multiple arms within one platform provide, platform trials can be more confusing when it comes to informed consent. Within a multi-stakeholder panel conducted throughout EU PEARL (Seidel *et al.*, 2022), it became clear that potential participants are likely to be put off by the fact that they will have to agree to multiple consent forms: one for the overall trial, one for the specific arm they are sorted into, and potentially one more if switching arms becomes an option. As platform trials are becoming more common, we hope that evaluations include these aspects in the future, so that knowledge on best practices can be shared.

Using PaCEPT

The PaCEPT repository itself is hosted within the existing SYNAPSE platform (https://patientengagement.synapseconnect.org/), which is a wider framework with resources for patient involvement and engagement, but adapted to a bespoke design. Users are welcomed by a banner introducing the goal of the repository, immediately followed by the three stakeholder groups, where it is possible to choose whether the user is:

1. A patient or carer looking for information to understand what a platform trial is and if participation in a platform trial might be an option;
2. A patient and community representative looking for information to understand what a platform trial is and how to contribute to the research & development aspects of platform clinical trials; or
3. A member of the Medical and Research Community looking for information to understand how to involve patients in the research & development process or support patients participating in platform clinical trials.

Upon clicking on any of the categories, users will find the fitting materials, both already existing and those developed specifically for the PaCEPT repository. In many cases, these resources are linked through the wider initiatives they have been established in and with author profiles linked, making it easier for users to connect to the those involved in the creation of the material in question.

Conclusion

If we want to reap the benefits of platform trials, we need to ensure they are conducted fairly and ensure to account for their specific hurdles. While platform trials offer a number of advantages compared to one-armed trials, their complexity makes them a complicated endeavour to navigate methodologically and in terms of analysis approaches (see Ballarini *et al.*, 2021; Posch & König, 2021; Schiavone *et al.*, 2019; Sydes *et al.*, 2009) and can put high stress on the people delivering them (Morrell *et al.*, 2019). As such, it is key that patient engagement activities—and evaluations of practices—are approached with a clear outline. Starting early in the design phase and fostering a positive mindset which focuses on the benefits of including patients and communities and avoids patient engagement being seen as an additional burden to the already high workload in a complex working environment is key. It is our hope that the PaCEPT repository will support patients and members of affected communities, patient and community advisors, as well as researchers and funders in creating or contributing jointly to platform trials in a mutually beneficial way. In addition to using materials hosted on the PaCEPT repository, we specifically would like to advise researchers to make use of the EU PEARL Platform Trial Best Practice Tool (Baffert *et al.*, forthcoming; see also Parke *et al.*, forthcoming), which was developed in parallel to the PaCEPT repository, early on in the set-up of their trial. The tool includes a checklist of ways to engage patients and communities in platform trials, which can be adapted to fit a bespoke master protocol. Materials to achieve the suggestions from the tool can be found in the PaCEPT repository. Given the low prevalence of patient engagement across clinical trials in general and that further evaluation of best practices remains a major ask (Fergusson *et al.*, 2018), we encourage researchers to share their adaptations of the Clinical Operations Tool and evaluations[2] of their engagement strategies derived from the repository. As such, we invite people to get in touch with EPF not only with any additional tools and resources for any stakeholder group, but also evaluations of using the resources provided on PaCEPT. This way, we hope the repository will grow and increase its benefits for everyone over time.

Data availability

Underlying data

OSF: EU PEARL PaCEPT Supplementary. https://doi.org/10.17605/OSF.IO/5D8QG. (Jarke & Consortium, 2023).

This project contains the following underlying data:

- Expert_Advisory_Group_Comments_on_Feedback_on_Patient_Benefits_of_Platform_Trials.xlsx. (Explanations and comments regarding applicability and limitations of these from consortium experts).
- Landscape review – Existing Materials for Patient and Community Engagement.docx. (Results of the landscape review on existing materials for clinical research and trials).

Data are available under the terms of the Creative Commons Attribution 4.0 International license (CC-BY 4.0).

[2]For a potential evaluation framework or measurements, see for example Vat *et al.* (2021) or Moore *et al.* (2022).

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✓ **Teresa Finlay**
University of Oxford, Oxford, England, UK

This open letter describes the launch of a new repository for people considering being involved (engaged) in the design of a clinical platform trial or being a participant in such a trial. This is a commendable endeavour in the growing collection of tools to support patient and citizens' engagement in health research and development. The authors appropriately summarise the aims and challenges of platform trials to provide context before going on to outline the development of the repository PaCEPT.

The literature reviewed in the patient-centricity and community involvement section is very succinct but appears to include the principal, most recent publications of direct relevance.

A description of the repository and its development follows; here it would have been interesting to have some detail on the activities that led to the final output (the repository), how the patients and their representatives (from four different PAGs) were included and their contributions influenced the project. Given this is a project to develop resources for patients and communities this kind of information would lend authority and validity to the project as well as enabling the article to be of direct relevance to a wider audience. I agree with the other reviewer that inclusion of some of the challenging aspects of platform trials, particularly those that will affect participants, would be helpful to know.

The Plain Language Summary could be slightly more straightforward; for example, after the first sentence, explain that this describes a clinical trial before going on to use the word 'trial' in the following sentence. Proof-reading before finally indexing would provide final polish; there are a few typographical and syntax errors.

Is the rationale for the Open Letter provided in sufficient detail? (Please consider whether existing challenges in the field are outlined clearly and whether the purpose of the letter is explained)
Yes

Does the article adequately reference differing views and opinions?
Partly

Are all factual statements correct, and are statements and arguments made adequately supported by citations?
Yes

Is the Open Letter written in accessible language? (Please consider whether all subject-specific terms, concepts and abbreviations are explained)
Partly

Where applicable, are recommendations and next steps explained clearly for others to follow? (Please consider whether others in the research community would be able to implement guidelines or recommendations and/or constructively engage in the debate)
Yes

Competing Interests: No competing interests were disclosed.

Reviewer Expertise: Qualitative health services researcher with an interest in the politics of evidence as related to experiential expertise and public engagement. I have experience of using co-design and participatory action research methods in addition to conducting ethnographic studies in healthcare settings that are informed or driven by patient and community engagement . Whilst having a working knowledge of clinical trials as a healthcare professional and lecturer in prescribing, I do not have detailed knowledge of the conduct of clinical trials including platform trials.

I confirm that I have read this submission and believe that I have an appropriate level of expertise to confirm that it is of an acceptable scientific standard.

Reviewer Report 12 June 2023

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?

Douglas H Fernald
University of Colorado School of Medicine, Aurora, Colorado, USA

This article describes the rationale for and design of a repository of resources for mutliple audiences participating in platform trials, a relatively new area for clinical trial designs. Overall, the writing was clear and easy to follow. However, given the relative newness of such trial design and limited research or evaluation of the patient or carer experience, there are places where the article overemphasizes the potential benefits, while mentioning only a minor disadvantage. I think a

more balanced presentation would be warranted. Further, the patient and community input on the design of the repository seems insufficient given that a major focus of this article is about patient and community engagement. Here are a few specific comments:

Introduction: Good introduction to platform trial designs. For a balanced view of platform designs, I think it is worth mentioning--in one or two sentences--what the primary known disadvantages of a trial design are, especially for patients. If there is no known literature on the subject, please state that.

Patient-centricity and community involvement: No comments. Concise review of some of the literature.

PaCEPT: A Repository for Patient and Community Engagement in Clinical Platform Trials: I think this section needs a bit more information on the extent to which PAG or patients/community members were involved in the design of the materials that were created (e.g., infographics). Were their comments given equal weight as other "specialized consortium members"? While it's mentioned and referenced in the data availability, I think readers would like a few more sentences describing the specifics of how exactly PAG or patients influenced the design of the materials (e.g., ethical concerns raised, specific language or content). This would give us more confidence in the commitment to patient engagement principles. For example, is there a resource that addresses informed consent (noted as being more confusing)? Or, for balance, is there an infographic on the disadvantages or challenges (for patients) of participating in a platform trial?

Conclusion:

- I think the conclusion needs a statement somewhere acknowledging that there may be unknown burdens to patients or carers participating in platform trials because of the limited evaluations of the patient experience in such trials.

- I don't know if the last two sentences add much to this article. Consider deleting.

Is the rationale for the Open Letter provided in sufficient detail? (Please consider whether existing challenges in the field are outlined clearly and whether the purpose of the letter is explained)

Yes

Does the article adequately reference differing views and opinions?

Partly

Are all factual statements correct, and are statements and arguments made adequately supported by citations?

Yes

Is the Open Letter written in accessible language? (Please consider whether all subject-specific terms, concepts and abbreviations are explained)

Yes

Where applicable, are recommendations and next steps explained clearly for others to

follow? (Please consider whether others in the research community would be able to implement guidelines or recommendations and/or constructively engage in the debate)
Yes

Competing Interests: No competing interests were disclosed.

Reviewer Expertise: More than a decade of community-based, participatory research and evaluation, especially related to the clinical practice of family medicine in community settings. More than two decades of mixed-methods research and evaluation experience across community and clinical settings.

I confirm that I have read this submission and believe that I have an appropriate level of expertise to confirm that it is of an acceptable scientific standard, however I have significant reservations, as outlined above.