

Short Communication

Including People with Parkinson's Disease in Clinical Study Design and Execution: A Call to Action

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Abstract. The proactive inclusion of patients in the design and execution of clinical studies has been an emerging focus for decades. Such participatory research helps to design studies better, by addressing relevant research questions and defining outcomes that matter to patients. Yet, much remains to be learned about the best methods and exact impacts of patient engagement in research in general, and more specifically, about the specific challenges that come with Parkinson's disease. Here we present the lived experiences of patient researchers living with Parkinson's disease, as a motivation for the value of their perspectives in research and as a call to action for empirical research on how to successfully include patient researchers.

Keywords: Patient participation, patient advocacy, patient preference, Parkinson's disease

INTRODUCTION

The inclusion of patients in the design and execution of clinical studies, a concept also known as participatory research, has been an emerging focus for decades. The term 'patient' in this context is used to identify individuals with the lived experience of a health condition. Apart from a strong ethical imperative that research *for* patients should be designed and executed together *with* patients [1], evidence demonstrates the potentially positive impact. Including patients can help ensure that protocols are tolerable and that the trial outcomes actually matter to patients [2]. Evaluations report cost savings

because of faster recruitment, lower drop-out rates due to successful engagement strategies, and consequently, faster completion of the clinical trial [3]. Researchers perceive their work as more rewarding, enjoyable, and meaningful when patients are actively involved [4, 5]. Patients can also take a shared responsibility for ascertaining that the study findings actually find their way to the scientific community and wider general audience via accessible publications [6]. Yet, there is still much to be learned about the best methods and exact impacts of patient engagement in research [7]. In this short communication we present a call to action for researchers to address the specific challenges that Parkinson's disease brings for participatory research. Based on the lived experiences of two of the authors, who are patient researchers, we provide initial guidance for effective involvement of people with Parkinson's disease in designing and executing clinical studies.

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Box 1. Points of principle for including patients in clinical study design and execution*.

- Involving people with the lived experience of the health condition under study is a prerequisite for any research project.
- Involve patients in all aspects of research, from inception to closure. For example, in defining outcome measures, contributing to the design of study logistics; pilot testing study protocols; agreeing on study terminology; and developing recruitment and retention strategies. After study completion, patients can become involved in interpreting data; reviewing final reports and papers; and acting as a study advocate and presenter at conferences.
- The appropriate degree of involvement may vary across the aspects within the study, ranging from informing the patient researchers to giving them a “powerholder” status, with the authority to make material decisions.
- Take measures to increase diversity and inclusivity, acknowledging, for example, gender, ethnicity, race, health literacy, severity of disease, and age of onset.
- Make sure everybody in the research team knows what is expected of the patients by means of a role description. The role should be described in terms of what it means to be a full member of the research team, what is expected of the patients in general, as well as more specifically in what ways and how often they will be expected to participate. Vice versa, the patient researchers should also know what to expect from the other team members.
- The role description should also be used to clarify expectations in terms of time, travel, potential compensation, reading, reviewing, writing, and speaking obligations.
- Create mutual respect within the research team.
- Explain that the patient’s expertise in living with the condition is what they are expected to bring to the table; the researcher’s expertise is on research methodology. If needed, offer training to patients on how they can fulfill their role as a representative for the patient’s perspective at large.
- Remove barriers to patients contributing to the research, such as language, access to study team offices, and the like.
- Reimburse patient researchers appropriately, not only for their expenses, but also for their time. Include these costs when applying for funding.
- To maintain a productive relationship, continuously evaluate the effectiveness of patient involvement and re-define the process of involvement if needed. As effectiveness is not a predefined endpoint, effectiveness measures should be aligned with the defined roles and responsibilities for the patient researchers.

* See the Parkinson’s UK public involvement guide for extensive practical guidance [16].

SETTING THE STAGE FOR PATIENT RESEARCHERS

Approaches, designed to support patient researchers’ involvement in clinical research, can have different foci and purposes. A synthesis of 65 published frameworks identified five distinct purposes: power-focused, designed to overcome researcher-patient power imbalances; priority-setting, aimed to involve patients in defining research priorities; study-focused, on how to involve patients in maximizing recruitment and retention to clinical trials; report-focused, designed to guide writing up research findings; and partnership-focused, to assure transparency in research-patient collaborations [8].

The body of research on patient engagement identified strategies that help to effectively position patient researchers in a research team [2, 5, 8–12] (summarized in Box 1). For example, a review with 18 included articles, identified principles of trust, respect, transparency, partnerships, communication, diversity, confidentiality, and co-learning with respect to patient involvement in research as guiding principles for patient involvement in research [9]. Governmental funding bodies have translated this literature into frameworks for engagement, such as the Canadian Institute of Health Research which launched the Strategy for Patient-Oriented Research (SPOR) [13], and the American Patient-Centered Outcome Research Institute (PCORI) Engagement

Rubric [14]. The UK Standards for Public involvement provides a framework for what good public involvement in research looks like and is designed to encourage reflection and learning [15]. Also Parkinson's UK published an extensive public involvement guide for researchers [16]. However, specific guiding principles for engaging people with Parkinson's disease in research are thus far lacking.

LIVED EXPERIENCES OF PATIENT RESEARCHERS WITH PARKINSON'S DISEASE

Co-authors ACD and MS are both people living with Parkinson's disease, who have served as experts in a project that aimed at incorporating patient preferences into the design and evaluation of a clinical trial [17]. The aim of the project was to develop a tool to collect patient preference data, to be used in the evaluation of medical devices. For the purpose of that study a hypothetical device, one that would never actually go through the U.S. Food and Drug Administration (FDA) approval process, was used. The outcomes of the project would be incorporated into submissions to the FDA for device approval. The project included a wide range of stakeholders, including representatives from the FDA and a patient charity, academics and a consultancy firm with expertise in providing evidence for regulatory approval of new devices.

As part of the project team, ACD's and MS's roles were to provide input into the study from the perspective of living with Parkinson's disease. Both were highly motivated to make an active contribution to this research project:

"In the past we have often felt powerless in the effort to find a cure to our disease. We welcomed the opportunity to join a research team engaged in exploring a different way to evaluate success or failure in clinical trials. It gave us the opportunity to do something." (ACD)

When the study began, ACD and MS did not know what to expect. As the study progressed, it became clear that they could actively participate as full team members. They were invited to offer input at each step of the research process, as the team articulated the focus of the data collection, defined the study terminology, pre-tested study protocols, analyzed data and subsequently presented findings at conferences and in writing. As a result, both patient researchers learned first-hand that the lived experience of patients

does have a unique and essential perspective to lend to research. They were able to affirm the validity of the findings and indicate the need to consider differences that are likely to emerge from different subgroups within the patient population, depending upon how the disease manifested itself.

As patient researchers in the project, ACD and MS provided insights into what is most important to those individuals for whom the research is ultimately designed to offer help:

"During a discussion of what questions to ask to identify those physical issues that are most concerning to people with Parkinson's disease, the researchers were surprised that we strongly suggested including 'pain' to the list of possible responses. They had no idea that this was a big issue for some of us." (MS)

"When reviewing definitions of symptoms of Parkinson's disease, we noticed that the researchers had included dyskinesia. We explained to them that dyskinesia is a side effect of the medication we take, and we need to live with it." (MS)

Moreover, the two patient researchers gave access to communities of patients, to help with recruitment:

"When the research team needed a group of 15 to 20 people with Parkinson's who had not been involved with previous research to pilot test a study protocol, we were able to swiftly recruit one of our Rock Steady Boxing groups (a boxing class for people with Parkinson's disease) to help out." (ACD)

During the project, communication became the key factor for effective involvement:

"It was very useful to engage us from the start of the project. Our perspectives were requested and included often—for example, on all team conference calls and/or meetings and we were copied on all team communications." (ACD)

"This gave us the opportunity to share our voices and make use of the power of momentum and motivation. It avoided big gaps in involvement or interaction, which can sap interest levels. We were happy to dedicate hours to meetings and other tasks as we believed that overall, we were making a contribution." (MS)

Box 2. Challenges and solutions to consider, when engaging people living with Parkinson's disease in research.

- As Parkinson's disease progresses, mobility may be an issue. Travel may be difficult for some—online conference calls enable participation of almost anyone. If in-person meetings are required, some people with Parkinson's disease may only be able to attend if they have a caretaker with them. Best to sort out these logistics before the study begins.
- Organization skills or what is referred to as “executive functioning” may be off and it is not unusual for people with Parkinson's disease to have a difficult time multi-tasking. So, don't expect them to.
- Certain communication skills may be diminished: people with Parkinson's disease often have very quiet voices, making it harder to participate in group discussions. Give them a chance to repeat themselves. When a patient is speaking in a public setting, consider pairing the patient to an expert, as a back-up.
- People with Parkinson's disease often have very small and or illegible handwriting which can also complicate some types of communication. Perhaps best not to ask them to be responsible for the taking notes during a meeting, unless they are facile with a computer.
- Some people with Parkinson's disease may have cognitive complications; for example, the ability to remember, focus, and concentrate may be diminished. A bit of patience and handouts that summarize the status of the study (past, present and future) as necessary can help.
- People with Parkinson's disease may be experiencing a variety of non-motor symptoms, such as pain, apathy, fatigue and depression. These symptoms should be considered, but don't necessarily obviate participation and in fact may add to a patient's motivation to help.
- People with Parkinson's disease often experience “off time”, when their medications aren't working. This can lead to stiffness, tremors and dystonia. Scheduling calls and meetings when patients are less likely to experience off time and less fatigue may improve their ability to participate fully. Usually mornings are better than afternoons.
- Patient researchers may experience barriers and motivations, which can be invisible for the research team. Actively ask for them and discuss how they can best be handled.

RECOMMENDATIONS FOR ENGAGING PEOPLE WITH PARKINSON'S DISEASE IN THE DESIGN AND EXECUTION OF CLINICAL RESEARCH

The experiences of ACD and MS confirm the experiences of other patient researchers in the Parkinson's community, such as Sara Riggare [18] and Soania Mathur [19], who emphasize the importance of patient involvement in research. Yet, Parkinson's disease comes with specific challenges. For example, issues such as depression, apathy, fatigue or frontal executive dysfunction may be factors that could hamper effective patient participation in research. Many patients experience diurnal fluctuations, with a worsening of symptoms during the day, which would imply that group meetings to discuss the research project in the late afternoon or evening can be a challenge. Medication could create further challenges, for example when patients are troubled by compulsions, secondary to treatment with dopamine receptor agonists. And there is obviously the issue of the

tremendous inter-individual variability across different individuals living with Parkinson's disease [20], so inclusion of just a limited number of patient researchers will inevitably only offer a restricted perspective of the overall needs of the worldwide Parkinson's disease population. Researchers should be aware of these Parkinson-specific challenges and develop strategies so that patient researchers can actually make the impact that is so highly needed.

Inspired by the experiences of the authors, we propose a set of measures which can be considered when involving patient researchers with Parkinson's disease (Box 2). These measures need further refinement and evaluation in future research. In addition, the existence of patient advisory committees such as the Michael J Fox Foundation Patient Council and the Parkinson's UK Research Support Network can provide a valuable source of large and diverse patient populations to be approached by researchers when looking for patient researchers and for guidance on how to shape patient involvement [16]. A key issue in this regard is diversity, which deserves

proactive attention in order to obtain a representative perspective of the many different people living with Parkinson's disease [21].

CONCLUDING REMARKS

We challenge researchers involved in health-related Parkinson's disease research to look ahead and purposely address emerging best practice standards for engaging patients in clinical study design and execution. Importantly, systematic identification of certain barriers and facilitators for active patient engagement and finding solutions on how to remove barriers is greatly needed to further develop the guiding principles for effective patient researchers in Parkinson's disease. Ultimately, all researchers should regard patient participation and co-creation in research as a vital aspect of learning and improving the value of research.

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CONFLICT OF INTEREST

The authors have no conflict of interest to report.

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