# Patients, Advocates, and Caregivers as Authors

## Perception

Many images are conjured up when patients, advocates, and caregivers (PACs) are suggested as authors on medical publications (Figure 1). While it is accepted that PACs often have compelling personal experiences that may be appropriate for a lay publication, they are perceived not to have the appropriate expertise to assist in the development of medical manuscripts. Thus, PACs are considered not to be qualified as authors based on the criteria set by the International Committee of Medical Journal Editors (ICMJE).[1]

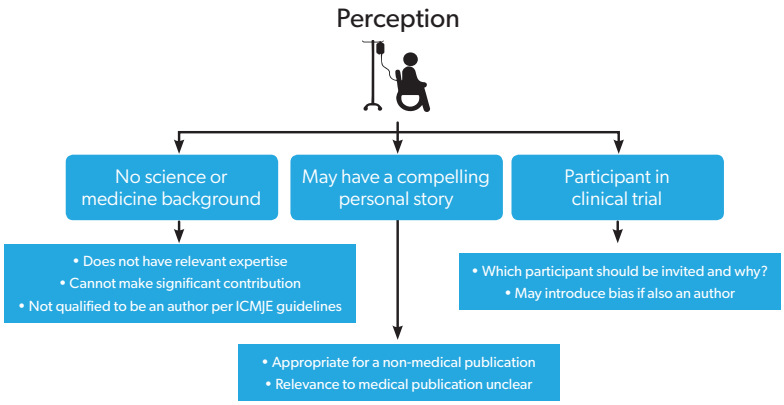



**Figure 1**

## Background

In recent years, there has been a push towards expanding the role of PACs (also referred to as patient and public involvement in medical literature; "patient" includes advocates and caregivers in this context) in medical research and publications on ethical, normative, and substantive grounds.[2,3] In fact, in the UK, involvement of PACs in medical and social research is now required for all programs funded by the government.[4,5]

## Traditional clinical research paradigm

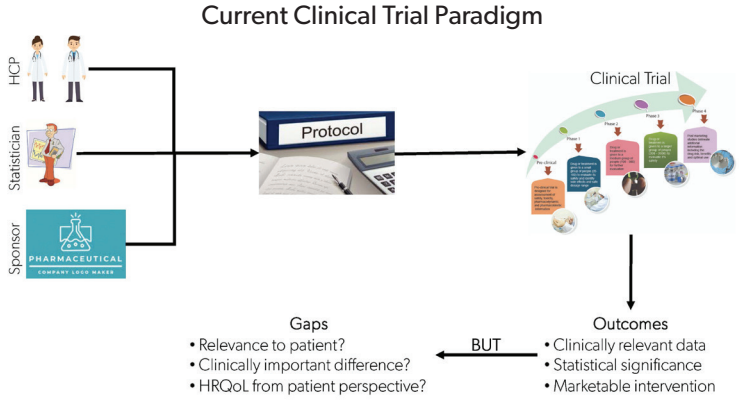
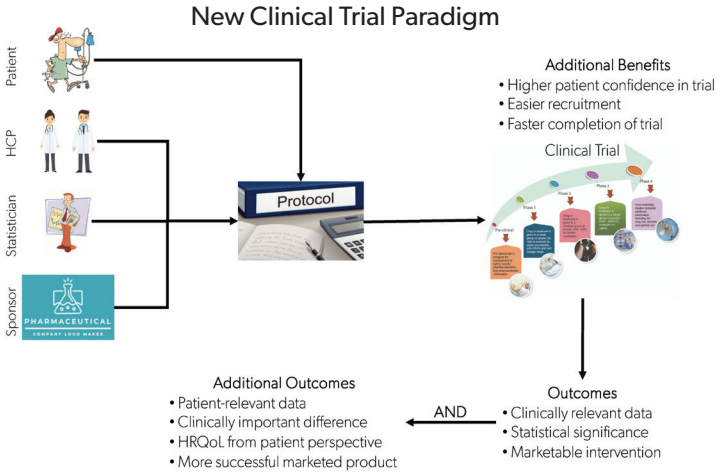



**Figure 2**

The traditional paradigm for clinical research is for the sponsor to work with healthcare professionals (HCPs) and statisticians to develop a trial protocol and enroll patients who undergo the intervention to which they have been assigned (Figure 2). The sponsor, HCPs, and statisticians analyze the results, report them to regulatory agencies, and discuss the data in peer-reviewed publications. The focus is exclusively on what the HCPs consider important and statisticians find to be significant. In this process, there is no input from PACs, from clinical trial protocol development through completion and reporting of the study. This approach does not consider which outcomes are most meaningful to patients. In this paradigm, patients have a passive role, akin to human guinea pigs (ie, studies were conducted "on", "about", or "for" PACs). At most, PACs have only an auxiliary role such as providing financial support or comparative samples, especially in rare diseases.[6] Although PACs have only recently begun to participate as authors on lay articles or narrative reviews discussing disease burden and/or patient and caregiver journeys, they are still not considered as qualified authors on medical articles.

## New clinical research paradigm (especially for rare diseases)

The new paradigm, as supported by the National Institute for Health Research in the UK and other similar funding agencies now includes patients and the public at every phase of the clinical program.[4,5] This model was developed in response to PAC and public demands for greater transparency and engagement (Figure 3). This paradigm shift involves clinical research being conducted "in collaboration with" or "by" PACs and democratizes the research process since they, especially patients, ultimately take the risk either during the trial or later with the approved therapy.[4,5]




Unlike with more common diseases, PAC involvement and engagement in clinical research is entrenched in rare diseases.[6] In the rare disease arena, PACs have taken on a more active role as partners with HCPs, getting involved in research and clinical trial design, funding, and implementation. Often, PACs have more knowledge in their rare disease than do HCPs, providing an expertise different from and complementary to that of HCPs because of their unique and intimate knowledge of the natural history, the spectrum of manifestations, and overall burden of the disease.[7,8] In addition, the European Medicines Agency and the FDA encourage early involvement of PACs in clinical research on rare diseases.[9]

The new paradigm empowers PACs and enhances the quality and relevance of the research by including outcomes and analyses that are meaningful to PACs. It is important to note that PAC involvement from the outset places them on the same standing as HCPs and statisticians (ie, providing intellectual input based on expertise and perspectives that are unique to them and that the other stakeholders cannot provide).

It is also important to mention that patients who qualify as authors in this new paradigm may also choose to participate in the clinical trial under special circumstances, such as having a rare disease. Therefore, PACs are fully engaged in the clinical trial and consequently, fully qualify as authors per ICMJE guidelines.

## Unanticipated benefits of PAC involvement

Involvement of PACs from the beginning of the clinical research provides additional benefits to all stakeholders. These include easier patient recruitment and higher patient confidence in the trial when they know that a PAC is involved in the development of the protocol, potentially leading to faster completion of the trial. This is particularly important in rare diseases where the limited number of available patients can delay trial completion, and/or obtaining statistically significant or clinically meaningful outcomes. Assuming the intervention being tested is effective and well tolerated, PAC involvement from the outset and in advising regulators on patient-relevant outcomes and the human aspect of their condition can lead to securing faster approval and community uptake of the novel therapy.

> *"Patients, advocates, and caregivers are Key Opinion Leaders in their rare disease space in their own right and also can be the key to unlocking nuanced insights and a compelling publication."*
>
> Dakota Fisher-Vance
> Young Adult Cancer Connection, Co-Founder and Patient

## Key takeaways

- PAC involvement in all aspects of clinical research is encouraged by regulatory agencies, especially in rare diseases
- Some funding agencies require PAC involvement for research that they support
- PAC involvement is beneficial to the meaningful outcomes of the clinical research and commercial success of the product, especially in rare diseases

To expand your rarelQ, please contact Dan Donovan at **ddonovan@rareLifesolutions.com**.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