EDITORIAL

Making public and patient involvement in clinical trials more than aspirational

Holly E. Hinson, MD, MCR, Roy H. Hamilton, MD, MS, and David B. Matchar, MD

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Correspondence
Dr. Matchar
david.matchar@
duke-nus.edu.sg

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There is growing evidence that including patients and public participants in the planning and execution of clinical trials can improve the applicability of research questions to real patient concerns and may more meaningfully affect patients' quality of life.[1] In contrast to targeting metrics that may be far removed from patient experience, patient-centered outcomes research seeks to "provide patients and the public information they can use to make decisions that reflect their desired health outcomes."[2] The pathway to this laudable goal is not always straightforward, however.

In this issue of *Neurology: Clinical Practice*, Tallantyre et al.[3] have attempted to create a roadmap for the implementation of patient and public involvement (PPI) in international clinical trials in neurology using a clinical trial in multiple sclerosis (MS) as a test case. The authors made a commendable effort to engage the relevant stakeholders by choosing a research question highly meaningful to patients with MS, namely, to compare an escalation strategy vs an early aggressive strategy to treat MS in the early days after diagnosis. The study team took care to integrate stakeholders into study governance early and sought guidance often, which adheres to some of the best available evidence for stakeholder engagement.[4]

However, there may have been some missed opportunities in the approach outlined by the authors. As the authors note, the stakeholders appear to have been a recruited by "word of mouth" and/or from existing PPI volunteers, thus "…introduced recruitment bias towards. [certain] contributors…." This type of bias can shape the character of input toward more affluent groups of patients who often enjoy easier access to clinicians and researchers and who also have the resources to participate in in-person focus groups, potentially disenfranchising low-income individuals who cannot afford to miss a day on the job and/or who do not have access to reliable transportation. Moreover, word-of-mouth recruitment may promote fairly homogeneous samples regarding race, ethnicity, age, sexual orientation, and gender identity. As a result, investigators may be missing out on critical viewpoints, especially on how to recruit in historically underrepresented groups. Many common neurologic illnesses disproportionately affect underrepresented populations, underscoring the importance of their participation as stakeholders in clinical research.[4] Moreover, open, engaged, and transparent collaboration with underrepresented communities—such as racial and ethnic minorities—can help to overcome negative attitudes toward participation in biomedical research that derive from both the historical precedent of exploitation of these groups in research and ongoing socioeconomic and health care system inequities.[5] To that end, it would have been helpful for the authors to note the demographic characteristics of the participants in their PPI program and to reflect on how some of these characteristics may influence the valuable input that they and other investigators receive about their trials. Other disciplines have formulated strategies for diverse stakeholder engagement, including underrepresented populations.[6] Such strategies may include internet/video conferencing to those who are unable to travel, nonbusiness-hour interactions, and social media solicitations for feedback. These alternative methods could complement the standard focus groups to reach out to underrepresented

Oregon Health Sciences (HEH); University of Pennsylvania (RHH); and Duke University (DBM).

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populations. For example, investigators may choose to meet stakeholders in their usual places of gathering, such as community centers and places of worship, as opposed to requiring travel.

Efforts at PPI have additional challenges that deserve attention and innovative solutions. A critical goal of engagement is for the researchers to see the research activity from the perspectives of stakeholders and to use existing and possibly new strategies to accommodate those views. Investigators should avoid co-opting patients; in this case, one wonders whether brain volume loss was truly of interest to patients separate from the impact of MS on the overt manifestations of the condition or is it really a measure of (legitimate scientific) interest to the investigators. Gaining this sort of insight requires going beyond informal or semistructured focus groups, using methods such as group model building,[5] in which the participants are encouraged to think about causal relationships and intended and unintended consequences of particular research design strategies, especially those relationships that could have untoward effects on underrepresented populations. These sort of exercises can also suggest new design options, such as computerized adaptive tests that accommodate outcomes that are salient to each subject[6] and the sequential multiple assignment randomized trial design[7] that specifically addresses the evaluation of treatment sequences while maximizing the likelihood that a study subject will receive a treatment that is effective for them.

Although the optimal strategy for PPI is yet to be defined for clinical trials in neurology, this effort is a commendable step in the right direction to achieve the goals stated in the Patient-Centered Outcomes Research Institute mission statement for trials "focusing on outcomes that people notice and care about such as survival, function, symptoms, and health-related quality of life." We all want to assure that this mission is achieved in substance—not merely as a charming sentiment. Care should be taken to ensure that researchers use the opportunity of PPI not only to hear what stakeholders say but also to develop a deep understanding of what they truly want and to meet them on common ground.

Author contributions

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