

## POLICY

# The study is open: Participants are now recruiting investigators

Recent events inspire optimism that a new age is dawning, one in which lay people have an active role in advancing biomedical research and health care delivery. Two ongoing experiments will deeply involve the public in these endeavors: the U.S. Precision Medicine Initiative (PMI) and the National Patient-Centered Clinical Research Network (PCORnet). PCORnet has already launched 20 patient-powered research networks designed to be led and animated by people who have an affinity with one another because of either shared disease, geography, experience, or identity (1). When U.S. President Barack Obama announced the PMI, he stated emphatically that people would be, not patients or even participants, but rather, partners in clinical research (2, 3). In the hours and days that followed, Francis Collins, director of the U.S. National Institutes of Health (NIH), reiterated this view, using words such as “participant” and “partner” when referring to people involved in clinical research (1). As a veteran citizen scientist and patient advocate (4), I was moved to tears to hear such proclamations from people other than my passionate fellow advocates. However, PCORnet’s efforts and PMI’s endeavor to enroll a million people—called All of US—will spur the advancements we seek only if we, the people, take advantage of these unprecedented opportunities and act with boldness to overcome myriad misaligned incentives, business interests, and general inertia against change.

## BUILDING THE WE: TRUE PARTICIPATION

Thousands of individuals affected by common and rare conditions indicate that they do not wish to be referred to as patients. “Patient” describes a person sitting on the exam table in a flimsy johnnie—the epitome of information and power asymmetry. This is not how the millions of people living with chronic or genetic diseases view themselves. We are also not subjects—that is, “a person or thing being studied.” Words matter, and using the word “participant” recognizes the actual engagement necessary to revolutionize clinical research and the resulting health interventions. Some investigators think that the term “participant” is a misnomer and should not be used. If that is so, and people with illnesses are not participants or partners in clinical research, then it is time to change that.

Sometimes investigators convey with enthusiasm that advocates are “invited to the table.” However, an invitation to the table implies that it is owned or managed by

the investigators and not by all stakeholders. Participants want not only to be invited to the table but also to design and host the meal with other stakeholders. There is a great deal of “us and them” language in biomedical research. Investigators point to “those patients,” and activists complain about “those investigators.” Clinicians are often left out of the process completely. When these roles are considered dichotomous and separate instead of part of a continuum, it is difficult to create authentic partnerships.

Participants have a place throughout the research continuum, including the proposal and prioritization of research questions, study design, engagement of study participants and their recruitment and retention, conduct of research and data analysis, and implementation and dissemination of results and, often, individuals’ own data. However, if we intend to engage a large and diverse array of people in clinical research, participation has to be made as frictionless as possible by creating mechanisms in communities where people live, work, and play, with community representatives leading the way. In addition, the research conducted must have relevance to the engaged parties by addressing questions that arise from communities of participants. If a study is built on the needs of individuals, families, and communities, then the results of research must be transparent and tangible—traits that run counter to the current culture. Researchers often do not return even the published results to the participants, let alone a lay summary or other accessible communication. If an effective intervention results from a clinical study, the process can take more than 10 years for the new intervention to be integrated into clinical practice (5). The new U.S. national efforts, particularly PCORnet, with its intention to establish a system that learns from the real-world experience of clinicians and patients to improve care through comparative effectiveness research, present an opportunity to change this.

Consumers hope that the improvements accelerated by PMI and PCORnet will go beyond diagnostics, therapies, and treatments to include more efficient systems. For example, if an ancillary benefit of improvement of health information exchange is that it is easier to transfer one’s child’s immunization record to their school or camp or to connect one’s electronic health record to a pharmacy clinic to get a flu vaccine, then one might be more incentivized to participate in these national efforts. Proximal benefits of big data in health and biomedical research must accrue and be felt by individuals, as they are in other consumer scenarios (such as the traffic app Waze and the music app Spotify).



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In the past, these sorts of connections and systems could be created only through the hard work of community organizers and advocates, often lauded as heroes in the ecosystem ancillary to the traditional biomedical enterprise. The networked information age allows for crowdsourcing and information liquidity, enabling everyone to play a part. There is no need for the “heroes” to take on this burden alone. Affinity groups, whether related to our family needs, hobbies, or health needs, have a much lighter form of leadership than the community and advocacy structures of old. This allows growth of unprecedented size and speed compared to the last few decades, which can be applied to the participation of people in national research efforts.

To be successful, All of US and PCORnet must first catalyze a culture change, which will be the bedrock on which their efforts stand. Trusted investigators who are members of the communities of interest are much more likely to garner greater participation throughout all phases of research. Engagement with participants in these national efforts must be built on trust relationships rather than on transactions such as consent, particularly when it is distilled to the sterile interaction of signing a form. There are many aspects to a relational model that are not inherent in the current health care system. For example, the clinician–patient encounter is encumbered by limited time and reimbursement for procedures, rather than the one-on-one encounter. Privacy is often thrown up as a roadblock to deeper engagement. But privacy and confidentiality are easily protected in trustworthy institutions, processes, and projects. When this trust is not present, potential participants in research studies are aware of that.

It is important that All of US and PCORnet not default to accessing data without engaging individuals. Researchers have claimed that authentic engagement is too expensive and that a blanket consent will suffice and provide the research enterprise with all of the data that are needed. But consent is not meant to stand alone. The biomedical research enterprise has extracted consent from the Fair Information Practice Principles and used it separately because it is a transactional element that seems to simply assure authorities (institutional review boards, journal editors, and regulators) that a right process was followed. But investigators’ inability to use information for uses not specified in the consent form creates a practice of making the consent form as broad as possible. In an age where we can state our preferences for every post and photo on Facebook or customize a dashboard for our online experience of banking, it is already possible to use the same granular and dynamic preference-setting process to share health information. There is great inertia in effecting change in this regard. The biomedical research enterprise, which relies on participants to advance their innovative therapies, should play a central role in the move toward the concepts inherent in “engagement.”

Even when well-intentioned investigators craft clinical research proposals with an authentic desire to engage participants in a meaningful way, the budget for these studies does not usually reflect commitment to engagement. Resources for engagement often are reduced dramatically, because decisions about funding are still made largely by the actors engaged in the “science” part of a project, not the engagement experts; thus, a great deal is expected of the engagement process for very little money. This is a misguided conceptualization of clinical research for two main reasons: (i) Engagement, which is often reduced to recruitment and retention, is essential. Every meeting on clinical research includes a discussion of the ~3 to 4% participation rate in clinical trials. This will not change unless resources are de-

ployed. It is unfair and unethical to ask all of the people working in engagement to do it for free or very little funding compared to the other aspects and specialists involved in the project. (ii) Engagement done well is based on data from several scientific disciplines, methods, and processes that have been validated experimentally by sociologists, anthropologists, and behavioral scientists. Remarkably, these professionals are almost never part of clinical research projects.

### THE PARTICIPANT WILL SEE YOU NOW

When health systems, national programs, or investigators use phrases such as “let people participate,” it explicitly indicates that they believe that the power lies with them, not with the participants. However, no one has the right to give people power; they already rightfully belong at the center of biomedical research. All of US and PCORnet are building the airplane as we are flying it, a situation with which those who need solutions to health problems are accustomed. This plane does not have seatbelts or oxygen masks yet. We have to work quickly and in new ways that will accelerate learning and allow emergence of new structures and processes.

The PMI’s All of US has begun well, stating that participants will be involved in all phases of the development of a million-person cohort. Most of the planning meetings held by the NIH and the White House have included community leaders, advocates, citizen scientists, and ordinary citizens. The working group that crafted the final report, essentially a road map for All of US, included individuals in these stakeholder groups, as does the Cohort Advisory Panel and executive committee—all voluntary service, at this time. Awards have been made to build various aspects of All of US. It will be telling to see the proportion of the budget spent on engagement, especially on the part called Direct Volunteers, in which ordinary folks will be engaged and invited to enroll in All of US. Hopefully, the plan is robust; the leaders have a proven track record for animating large numbers of people, and there are substantial resources committed to engagement.

PCORnet has taken a somewhat different route, because it did not have the same urgency as All of US to prep to launch before President Obama leaves office. There was time to plan governance and implementation that includes participants as partners over a longer period. Whether adequate resources have been assigned to engagement activities for these demonstrations remains to be seen. The three committees of PCORnet—Engagement, Data, and Research—work together to continually assess authentic engagement. PCORnet strives to be transparent and will be sharing lessons learned in a Commons.

It is important to give a great deal of thought to who is in participant stakeholder groups. In All of US, participants will include presumably healthy people, and so representatives can be drawn from the general population. PCORnet participants are defined as those with a lived experience of a condition, such as affected individuals, their families, and caregivers. We must remain attentive to the commodification and professionalism of advocacy and leadership within communities and organizations that preclude involvement of individuals with no lived experience of the condition or issue. These do not make useful surrogates for those who experience a condition on a day-to-day basis. Those of us who represent others must constantly reflect on our motivations and level of awareness. The people’s needs must come, first and foremost, before any others. Concurrent with this is

the importance of orientation and training to the complexity of biomedical research, quality improvement in practice, and health care delivery.

It is time for participants to invent the Uber or Lyft equivalent for solving our need for better health care. Neither of these revolutionary services was invented by the status quo but, rather, by visionary people who circumvented what existed and disrupted it. “Every system is perfectly designed to get the results it gets” (6). It remains to be seen whether we can truly create the change that we seek without a disruptive revolution from the outside. It is my hope that we can take a new tack and use the power of grassroots leadership and disruption, crowdsourcing, and citizen science to capitalize on the best of the current system’s expertise and passion. This will not be easy. We will make mistakes. But if, as a guiding principle, we relentlessly place participants and their interests at the center, the rest will follow.

–Sharon F. Terry

## REFERENCES AND NOTES

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