EDITORIAL

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# Seeking What Matters
## Patients as Research Partners

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In their excellent and timely article 'Patient and public involvement in patient-reported outcome measures: evolution not revolution,'[1] Sophie Staniszewska and colleagues make important points about the contribution of patients to the development of patient-reported outcome measures (PROMs), seeing "patients as research partners in the research process, not just as those individuals who are consulted or as subjects, from whom data are sourced, to ensure the acceptability, relevance, and quality of research."

As an advocate accustomed to collaborative relationships with researchers, this seems self-evident. And if any area of research design lends itself to patient involvement, it is certainly PROMs!

### 1. A Different Kind of Expertise

Many researchers are still simply unfamiliar with patients as collaborators, as opposed to research participants – or 'subjects,' a term that is still sometimes used. It's not clear to these researchers what role patients might play, or what value they might add – that is, until they experience it for themselves. It's difficult to describe or quantify the richness and dimension patient experiences can bring to the table.

In my work as a research advocate, I often refer to patients as 'experts.' With this characterization, I hope to directly confront an objection we often encounter, that patients do not have the requisite understanding of the issues under study to allow them to serve as research partners.

Before going further, I should point out that by referring to patients as 'experts,' it is not my intention to imply that they are the *only* experts on a given research question, or that they know *better* than professionals who conduct research studies. What I do believe is that patients are, in fact, experts when it comes to the subjective experience of their disease or condition, and that they can bring values to the conversation that may otherwise remain unaddressed. To the extent that they are able to articulate their experiences, and those of other patients with whom they share that common bond, they can serve as effective collaborators, adding to the quality of research studies.

The characterization of patients as experts questions an older paradigm in which knowledge resides primarily within a hierarchy of trained medical professionals, in which the patient's role is largely passive. From that mindset, concerns are often raised about an advocate's lack of medical training, undue reliance on personal experiences, failure to understand the scientific method, and the like.

A more useful way to think about this might be in terms of differing spheres of knowledge. It's my belief that a broader base of expertise, derived from a variety of realms and perspectives, can lead to much better research designs, tools and outcomes. In the many committees and research collaborations with which I've been involved over 20 years of advocacy, I've learned from academic theorists, scientists, statisticians, physicians, epidemiologists, regulators, policymakers, and public health experts, and I believe that they have learned from me, in my role of offering a patient perspective. At the very least, bringing patient

voices into the conversation enlivens and deepens any research discussion. Often, it can do much more.

Since I work on behalf of women with metastatic breast cancer, I am always acutely aware of the importance of patient voices being heard. Until very recently, the voices of women (and men) suffering from the advanced form of the disease that leads to almost all breast cancer deaths, almost never broke the surface of the pink tide of breast cancer awareness, with its emphasis on screening and early detection. Wherever patient voices are submerged, misunderstandings and myths prevail. Without research to guide understanding, the fears driving the avoidance persist. In such under-researched conditions, patient perspectives in the conduct and content of research are vital if researchers – and society – are ever going to get it right.

## 2. The Path of Advocacy

The path of healthcare advocacy usually begins with a devastating illness or condition, our own, or that of someone we love. Fear, grief, and helplessness are transformed through learning into action. As we become 'experienced' patients, moving past our initial coping with diagnosis, symptoms and treatments, many of us are motivated to reach out to others who are coping with our condition, to give back as we've been given to. In an effort to make a broader impact, some of us then begin a lengthy process of self-education so as to understand the medical aspects of our disease and science behind the condition and its treatments more fully. If research becomes a particular interest, we then undertake training to learn about scientific methodology and evidence-based healthcare, research design, basic statistics, and epidemiology.

What begins as difficult personal experience is eventually transformed into an avocation and a mission to be of help to others. Often, we discover in our advocacy a chance to pursue undeveloped interests and skills. But we always begin with the authenticity of our own experience. It is our foundation, although along the way we develop other expertise that helps us participate more fully with researchers. Of course, this occurs along a continuum of learning, where more seasoned advocates can bring a more sophisticated understanding to the research enterprise.

As we enter into the world of research, we may risk losing touch with our roots – those raw personal experiences of patients struggling to cope not only with their conditions, but with the healthcare system and the choices they must make. Resisting the impulse to become 'junior' doctors or scientists, we must constantly ground ourselves in patient experience. For me, reading and responding on a daily basis to women living with metastatic breast cancer in online patient communities offers that grounding.

In the US, where I live and work, patient and research advocates have worked very hard to form a bridge between the larger patient population and the medical, research, and regulatory establishment. We not only translate emerging science for our constituents, but we also strive to bring their concerns and voices back to the research community.

## 3. Roles That Patients and Advocates Can Play

In the case of PROMs, an advanced understanding of the theoretical constructs, design and validation of PROMs instruments is hardly required. That is expertise that others bring to the table. A motivated and vocal patient, especially one who is in contact with other patients and aware of the variety of experiences of disease and its treatments, can be an excellent research partner. Among the many issues such patients can help to resolve are:

- do the PROMs fully capture patient experiences?
- is what really matters to patients being asked? If not, what is missing?
- is there an important dimension of life or role functioning that is not touched upon?
- are patients being asked what they actually think and feel about their experiences, in the context of their lives, beyond simply reporting symptoms and side effects?
- is the language used clear and concise? Is it specific enough? Are the words used simple

and devoid of jargon? Can questions be easily understood by patients with lower educational levels? Are there good translations of the instrument for non-English-speaking patients?
- is the length of the PROM appropriate?
- does the timing and the setting for PROM administration make sense for patients?
- are there built-in opportunities for feedback from patients?

This is hardly a comprehensive list of questions, of course, but I hope it clearly demonstrates some areas of patient expertise. An iterative process whereby an instrument is piloted with other patients and revised accordingly will still be important, but doesn't obviate the need for collaboration with a patient or advocate. It seems reasonable to think that good feedback throughout the process of PROM development would make the process of piloting and testing prior to launch more efficient.

If a PROM is incorporated into a drug development trial, and funded by a pharmaceutical company, trained advocates who retain their independence of commercial interests will be able to raise additional questions concerning whether the PROMs being designed are sufficient to adequately capture adverse effects of treatment. In such cases, awareness of subtle framing and wording of questions can be crucial so as not to minimize the experience of treatment side effects, an issue bound to become more problematic as PROMs are fully integrated into drug development. Although this has rarely been scrutinized, as with every other member of a research group, patients and advocates should be free of conflicts of interest.

## 4. Measuring What Matters

When I became a research advocate, and was first reading clinical trials results and attending scientific meetings, it was like entering another disturbing reality. This was an era when high-dose chemotherapy with bone marrow transplants still inspired hope. More was better. Two and even three different chemotherapies at a time were deemed superior, and dose 'intensity' was the paradigm dominating treatment.

In those years, this approach was frequently applied to metastatic breast cancer, a setting where no drug, or combination of drugs, is curative, treatment is continuous, and being able to live as well as possible for each remaining day is crucial to women and their families. I knew directly from the women themselves how debilitating these drug combinations could be. For much of their precious time they were simply too ill to function, only recovering in time for the next chemo cycle. Yes, the drugs sometimes appeared to slow down disease progression a few weeks or months – but at a heavy price of nausea, pain, fevers, and crushing fatigue.

Yet when the results of these combined chemotherapy trials were reported, I soon noticed, even the grade 3 and 4 toxicities of these powerful drugs would typically be summarized in abstracts as 'expected,' 'manageable,' and (my personal favorite) 'tolerable.' To whom was this tolerable? I wondered. How would they know what patients actually felt if they didn't ask? Did tumor shrinkage really measure what mattered to women, especially if extended survival could not be proven? Was anyone asking patients about their quality of life, and what mattered to *them*?

Thankfully, in the years since, treatment has come to favor sequential monotherapy, the administration of one chemotherapy agent at a time, and supportive care has improved significantly. But I can't help feeling that failing to measure patient experiences contributed to the suffering of that time.

The wider use of PROMs is a clear signal of a new age of research where patient experience matters, and where the drug industry is being called upon to demonstrate clinical benefit in measurable ways that are meaningful to the patients themselves. Involving patients and advocates in the conduct of that research, and especially in the design of the instruments that will measure patient experiences, is critical.

"Compared with the progress made in [patient and public involvement] in other areas of health research," writes Staniszewska et al., "there has been little exploration of the potential for [patient and public involvement] in contributing to enhanced

 

quality, relevance, and acceptability of PROMs." The time has come for this to change.

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## Reference

1. Staniszewska S, Haywood KL, Brett J, et al. Patient and public involvement in patient-reported outcome measures: evolution not revolution. Patient 2012; 5 (2): 79-87

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