The National Working Group on Evidence-Based Health Care

The Role of the Patient/Consumer in Establishing a Dynamic Clinical Research Continuum:

Models of Patient/Consumer Inclusion

August 2008

The Working Group

The National Working Group on Evidence-Based Health Care (the Working Group) is a collaboration of patient/consumer organizations, professional societies, providers, researchers, and other interested stakeholders. Since January 2006, it has sought to empower patients and consumers by involving them in designing and prioritizing research, as well as reviewing evidence and contributing to its translation, dissemination, implementation, and evaluation. The Working Group supports including diverse stakeholders' perspectives in the conversations taking place in the public and private sector on generating more evidence to make healthcare decisions, developing best practices and care guidelines, and creating incentives for value-based healthcare.

Evidence-Based Health Care

Evidence-based healthcare (EBH) is the concept of determining a patient's treatment by balancing scientific evidence, practitioner judgment, and patient experience and preference. Over the past few years, policymakers have also viewed EBH as a solution to rising healthcare costs, poor quality, and safety concerns. To expand the adoption of EBH, policymakers and others have created programs to redefine research priorities, generate new medical information, support the creation of clinical guidelines, and measure quality and pay for value. Policy discussions to create a centralized research entity to produce more information on the comparative risks and benefits of competing treatment strategies embody the zeal for more EBH.

Principles for Patient/Consumer Participation in the Research Continuum

The National Working Group on Evidence-Based Health Care (the Working Group) strives to ensure inclusion of the patient/consumer perspective in EBH. As part of this effort, in summer 2007 the Working Group released a policy paper, titled "Rebalancing EBH: The Central Role of Patients and Consumers." The Working Group's paper describes a *Call to Action* to ensure patient/consumer involvement in EBH and articulates principles for patient/consumer inclusion.

Governance and Accountability

- Ensure that advisory and active voting roles within government entities, technology assessment institutions, and industry and payer organizations are held by diverse patients/consumers and patient representative organizations.
- Promote and ensure inclusion of patients/consumers on Institutional Review Boards and other research oversight mechanisms, such as FDA review panels.
- Ensure that patients and consumers are on peer review panels to review all draft research findings, not just offer public comments.
- Provide a more continuous process for soliciting patient/consumer input on all processes related to the funding and conduct of research, rather than Internet-only, two-week comment periods.
- Ensure equal time for public comment by patients/consumers and/or representative organizations in public forums, such as hearings.

Research Prioritization

- Involve patients/consumers in identifying further unanswered questions or research gaps that can provide a "feedback loop" to the research development phase.
- Promote patient and consumer participation in pre-clinical research focus groups that identify targets for new research, including identification of evidence gaps, and clinical or quality of life endpoints.
- Invite and incorporate patient/consumer input in defining and prioritizing post-market research needs and methods.
- Engage patients/consumers in defining health services research agendas, as well as defining methods to evaluate the impact of system changes brought about by the application of evidence into practice.
- Develop research studies.
- Involve patients/consumers and representative organizations in comparative research in defining key questions and research methodologies.
- Require clinical research to measure consumer-focused endpoints, such as quality of life measures and functionality.
- Include patients/consumers on pre-clinical and post-market review panels within government regulatory bodies to ensure critical assessment of research and pinpoint goals and outcomes of importance to real-world patients.
- Provide incentives for research organizations to demonstrate methods for including patient/consumer perspectives.

Research Study Development

- Involve patients/consumers and representative organizations in comparative research in defining key questions and research methodologies.
- Require clinical research to measure consumer-focused endpoints, such as quality of life measures and functionality.
- Include patients/consumers on pre-clinical and post-market review panels within government regulatory bodies to ensure critical assessment of research and pinpoint goals and outcomes of importance to real-world patients.
- Provide incentives for research organizations to demonstrate methods for including patient/consumer perspectives.

Translation and Dissemination

- Engage patients/consumers to test key findings derived from evidence development, and include those perspectives in the translation of research.
- Use patients/consumers to translate research to a lay audience, review draft translational materials, and create strategies for communicating the information.
- Involve patients/consumers in identifying key questions and/or action steps consumers can use to implement evidence-based findings into their healthcare decision-making. Conduct patient/consumer focus groups to draw input on barriers to implementing research into consumer practice/action (e.g., what are different ways patients/consumers will incorporate research information into personal discussions and clinical interactions).
- Involve patients/consumers in developing training materials, media, or other educational products for targeted audiences.
- Use patients/consumers to identify messaging gaps, health literacy needs, or areas for further research.
- Seek input from patients/consumers on culturally appropriate messages and approaches to translating research for their particular cohort (disease, ethnicity, or age groups).
- Include patients/consumers in identifying methods, channels, tools, and networks to disseminate research effectively and efficiently.

Implementation

- Ensure transparency of evidence-based decision-making processes and criteria, such as in determining coverage and payment.
- Include voting patients/consumers and representative organizations on advisory panels that inform coverage and reimbursement policy and the development of quality measures and pay-for-performance systems.
- Institute active monitoring, research, and analysis to track patient health outcomes and cost offsets and identify any access-to-care issues that result from these policy changes.
- Emphasize distinct evaluation of subpopulations, including chronic illness, cultural, and socioeconomic cohorts.
- Include patients/consumers in stakeholder dialogue with decision-making entities (public and private) to identify key lessons from evidence development activities, identify targets for policy change, map intended and unintended consequences, and formulate evaluation plans to monitor impact based on outcomes that are defined with patients/consumers.

Continuing the momentum ignited by the *Call to Action*, the Working Group, in March 2008, convened more than 80 patient/consumer advocates, decision-makers, regulators, and other stakeholders to participate in the *Advancing the Evidence of Experience: Practical Issues for Patient/Consumer Inclusion Forum*. The forum examined patient/consumer inclusion in the research continuum—designing, prioritizing, conducting, translating/disseminating, and implementing research. In addition, the forum facilitated the sharing of strategies and tactics to incorporate the patient/consumer perspective into research.[1]

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[1] A summary of the forum is available at www.evidencebasedhealthcare.org.

Patient Advocacy as a Driving Force on Regulation and Innovation in Healthcare Technology: The Central Role of the FDA in EBH

Creating new healthcare innovations is vital to supporting a patient-centered healthcare approach. An engine of new healthcare innovation is government-funded research through the National Institutes of Health (NIH). This knowledge is often brought to market by medical product industries, which are regulated by the Food and Drug Administration (FDA). However, the voice of the patient/consumer is often missing in the process of regulating innovation and research, but the process is evolving. For instance, the FDA is increasingly including patients/consumers in its deliberations. The FDA Patient Consultant and Patient Representative Programs, administered by the Office of Special Health Initiatives (OSHI), are examples of significant patient/consumer participation in EBH. By advising the FDA and industry sponsors on the patient/consumer perspective, these programs have had a tangible impact on the research conducted by industry while developing new therapies that make up the evidence generated for healthcare decision-making.

The FDA convenes advisory committees of medical and scientific experts to help make decisions about new drug and medical device approvals. To balance the medical and scientific expert perspectives, FDA Advisory Committees include both industry and consumer/patient representatives. The consumers speak for broad public interests, including those of patients who are the direct consumers of the specific regulated medicines that are under review. However, patients with an illness often are willing to take more risks to achieve benefits when compared to the average consumer. The OSHI recruits and trains patient representative to participate as either voting or non-voting members of Advisory Committees to ensure the patient perspective is part of panel deliberations.

Further upstream, the FDA helps to incorporate the patient perspective into the development of new therapies, recognizing that patients are not only the ultimate consumers of medicines, but they also provide valuable insights in evaluating the safety and efficacy of therapies. Patient consultants are recruited and trained by OSHI, in collaboration with patient advocacy organizations, to advise the FDA during pre-approval reviews of clinical trial protocols and interim results from early phase studies. Patient consultants are empowered to contribute to FDA product review in the areas of trial design, study recruitment, informed consent, defining clinical endpoints, and quality of life issues. The patient consultant program has pilot projects in oncology and neurology.

The research and innovations regulated by the FDA are central to EBH. The FDA Patient Consultant and Patient Representative Programs are models for engaging patients/consumers in the conduct of research and EBH.

In addition to the *Call to Action* and forum, the Working Group identified organizations that engage patients/consumers in conducting research. These organizations highlight a variety of paradigms and best practices for gaining the patient/consumer perspective in research. In an effort to inform policymakers and organizations conducting research on including patients/consumers in EBH, this paper highlights organizations engaging patients/consumers in research.[2]

[2] The terminology used by the organizations to refer to patients/consumers varies. Various stakeholders define the terms patient, consumer, patient advocates, and public differently. The descriptions and analysis below use the same terminology as the organizations when describing their activities.

Examples from Five Organizations Involving Patients/Consumers in Research

The organizations below represent a wide variety of models from large, publicly funded health technology assessment groups to small, independent, disease-specific research programs. To obtain diversity, the organizations were selected based on the extent of their engagement in the research continuum; structure (government agencies, membership organizations, and disease-specific research groups, etc.); and breadth of approaches to patient/consumer involvement that can be replicated at both the national and local levels. The organizations are both domestic and international.

Agency for Healthcare Research and Quality Effective Health Care Program

The Agency for Healthcare Research and Quality (AHRQ), a federal agency under the U.S. Department of Health and Human Services, is tasked with conducting research on healthcare quality, costs, outcomes, and patient safety. The Medicare Prescription Drug, Improvement, and Modernization Act (MMA) of 2003 authorized AHRQ to conduct comparative effectiveness research on healthcare items and services, establishing the Effective Health Care Program (EHCP). The Stakeholder Group, an advisory body, was established to support the EHCP by providing input on evidence gaps, key research questions, and evidence implementation. The Stakeholder Group guides the program in an effort to improve the quality and value of its research products. www.ahrq.gov/

Specialized Program of Research Excellence

Specialized Program of Research Excellence (SPORE) is a disease-specific, translational cancer research program supported by the National Cancer Institute. The primary goal of SPORE is to conduct research around translating laboratory discoveries to patient and population research settings. One of the SPORE grantees, University of California, San Francisco (UCSF) Breast Oncology Program, initiated a patient involvement pilot in 1992, now referred to as the Breast SPORE Advocacy Core (BSAC). The BSAC members assist the UCSF researchers on the Institutional Review Board, research meetings, clinical trial designs, and enrollment. http://spores.nci.nih.gov/

Medical Research Council

The Medical Research Council (MRC) is a publicly funded research organization in the United Kingdom that provides research funding to individual scientists, universities, and hospitals. The Public Panel, a MRC advisory body, matches individual patients/consumers with relevant expertise to current MRC activities, facilitating patient/consumer participation in MRC research activities. www.mrc.ac.uk/index.htm

National Health Service's HTA Programme

The National Institute for Health Research, the research arm of the United Kingdom's publicly funded National Health Service (NHS), established the Health Technology Assessment (HTA) Programme in 1993 to evaluate the appropriateness of technologies for coverage decision-makers like the National Institute for Health and Clinical Excellence (NICE). It is important to note that NHS established the INVOLVE program to promote public involvement in NHS research and public health activities. INVOLVE is an advisory group on public involvement in research and development in the Department of Health. Specifically, the group focuses on involving patients/consumers when research is prioritized, conducted, translated, and implemented by the NHS. www.ncchta.org/

Consumer's Health Forum and Medical Services Advisory Committee

The Consumer's Health Forum (CHF) of Australia is an independent, member-based organization for health consumers. The group advises policymakers of the consumer perspective on various health policy issues. CHF nominates consumer representatives to advise and inform the activities of the Medical Services Advisory Committee (MSAC), among other organizations. MSAC is a HTA committee for the Australian government focused on assessing new medical services and technologies. The MSAC provides information about the strength of evidence supporting the use of new medical interventions in terms of their safety, cost-effectiveness, and clinical-effectiveness. The MSAC also provides recommendations to the Minister of Health on which interventions should be paid for using public funds. www.msac.gov.au/

Highlights from Organizations Engaging Patients/Consumers in Research

Organizations conducting research incorporate patients/consumers into the research continuum in a variety of ways. Highlights of the organizations' patient/consumer engagement approaches are organized below using the Working Group principles as a framework.

Governance and Accountability: Consumers/Patients Have a Seat at the Table

Patients/consumers are often represented directly with seats on the governing bodies of the five model organizations. For example, the AHRQ EHCP Stakeholder Group maintains two consumer/patient representatives as a part of the 17-member governing body, which guides program improvement. Recognizing the need for patient/consumer representation, the MRC's Public Panel serves to link "suitable lay people" with some expertise in medical research to MRC project-specific panels.[3] Patient/consumer representatives selected from the Public Panel may participate in the research review process, as well as the allocation of research funds.

Stakeholders have questioned the ability of patients/consumers to have their voices heard when they are one of many engaged stakeholders. To account for this, research organizations may consider creating a separate patient/consumer advisory body, whose purpose would be to advise the organization throughout the research process.

Notwithstanding the type of representation model, it is important for patients/consumers to continue to pursue positions on governing boards and lobby for equal representation. This will help ensure that the patient/consumer perspective is a part of influencing each organization's activities.

Research Prioritization: Patients/Consumers Directly and Indirectly Suggest Research Topics

The organizations explored avenues to integrate the patient/consumer perspective in the prioritization of research in several ways. Public comment periods, during which patient/consumer input is solicited, are the most common method organizations use to engage patients/consumers in their research activities.

For instance, the NHS HTA Programme utilizes a web-based form to allow both the public and pre-determined service users (members of the public who use NHS services) to suggest areas for research and assessment. Topics suggested are then sent to the relevant review panels. Each panel has service users who comment on research questions and a research proposal that defines the plan for conducting the research.

MRC uses a more direct model of engagement, which conducts "public consultations" with surveys and public meetings to understand areas where the public would like to see more research. Finally, the most direct method identified is the UCSF SPORE, where patient advocates assist the Institutional Review Board and participate in the approval of research and study designs.

Patient/consumer engagement in priority setting activities is vital to the research enterprise generating information that patients/consumers care about with regard to, for instance, outcomes and quality of life. It will be important for patients/consumers to seek direct engagement opportunities, if they want to have the greatest influence over research topics.

Research Study Development: Patient/Consumer Participation in Study Design Demands Institutional Resources

Organizations that allocate resources to support patients/consumers in their research engagement efforts, through education tools and other resources, tend to have more comprehensive patient/consumer engagement. In the past, MSAC and CHF have collaborated to ensure that consumer engagement in research is valuable and productive for consumers

[3] The MRC defines suitable lay people as members of voluntary organizations, a patient or caregiver with a particular illness, condition, or experience, or a member of the public with an interest in medical research.

and researchers. The two groups undertook the Consumer Representatives Resources Project, which provided support systems and information to consumer representatives to ensure that they had the right tools to engage. One of their activities included a workshop to identify the necessary tools, skills, and knowledge required for patient/consumer representatives to participate in the research enterprise and to discuss best practices.

In the **HTA Programme**, service users, which include patients/consumers, act as peer referees on the HTA Commissioning Board, which is charged with evaluating proposed research designs. To support the service users on the HTA Commissioning Board, the **NHS** created a Support Unit to supply information and guidance to the service users, researchers, and research sponsors.

Research organizations should be accountable for providing patients/consumers with the appropriate resources to engage in study design activities successfully. Study design is complex and requires specialized knowledge. An asymmetry of knowledge should not prevent patients/consumers from fully engaging in the process.

Translation and Dissemination: Key Messages Should Be Developed with Patient/Consumer Input

Patient/consumer involvement in translation and dissemination often depends on the products of the organization conducting research. If the organization is producing educational tools for patients/consumers, then patients/consumers tend to be more involved. If the organization is translating information for researchers or physicians, then the organization will often seek the perspectives of appropriate professionals. Further, the organization conducting research may not be responsible for translating and disseminating information, as another organization may be performing those activities.[4]

The **UCSF SPORE** uses patient advocates to help design patient-oriented tools and engage in educational efforts for both patient and scientific audiences. In contrast, the **HTA Programme**'s customers, such as **NICE**, conduct the translation and dissemination of the evidence generated by the Programme. However, NICE does incorporate the public into the translation and dissemination process through the Patient and Public Involvement Program to ensure public input into translation and dissemination.

The patient/consumer perspective should be incorporated regardless of the end user of the information, as it will help ensure that evidence application to inform policy and clinical decisions maintains the patient/consumer perspective.

Implementation: Applying Evidence Considering the Patient/Consumer Perspective Is an Area for Growth

Given the activities of the aforementioned organizations conducting research, evidence implementation may require organizations to enhance their efforts to incorporate patients/consumers. Evidence implementation is vital, as it stands to have a significant impact on patient/consumer access to treatments and participation in treatment decisions.

In 2007, **AHRQ** designated 1 of its 14 Centers for Education & Research on Therapeutics (CERTs), the Houston CERT, to focus on "risk and health communication; patient, consumer and professional education; health decision-making and decision-support; and therapeutic adherence." Projects focus on using social marketing techniques, testing informed decision-making tools, and creating aids for low-literacy patients.[5]

Patient/consumer inclusion in evidence implementation should be an area of focus and research moving forward as the implementation phase stands to have the most direct impact on the delivery of care to patients/consumers.

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[4] It is important to note that some of the variation in patient/consumer engagement can be attributed to differences in each organization's mission. In other words, groups with the same overall purpose are likely to employ similar methods to engage patients/consumers.

[5] The University of Texas MD Anderson Cancer Center and Baylor College of Medicine collaborate as the Houston CERT.

Conclusion

According to the Institute of Medicine (IOM), the "Clinical Research Enterprise is…a very complex enterprise made up of many stakeholders—the doctors, the patients, the public, the academic health centers, the industry entities—who do not necessarily function in a seamless fashion." The IOM suggests that patient/consumer participation in research extends the "role of the public in clinical research beyond participation in trials." Research organizations, patient and consumer groups, regulatory agencies, and payers try to engage patients/consumers in the research continuum by using a variety of approaches, and strive to identify additional means to include patients/consumers.[6]

This paper provides a snapshot of domestic and international activities to involve patients/consumers throughout the research enterprise. Identifying best practices for involving patients/consumers is essential, given discussions about increasing the U.S. capacity for comparative effectiveness research and the potential for a new centralized entity to conduct the research. Whether policymakers decide to establish a new entity, the need for patient and consumer involvement in the research continuum will remain. The concepts outlined in this paper should inform the efforts to include patients and consumers in the entire research enterprise.

[6] In 2000, the Institute of Medicine convened a workshop titled Exploring Challenges, Progress, and New Models for Engaging the Public in the Clinical Research Enterprise, a seminal event supporting patient/consumer engagement in research. The report states that research should actively involve members of the public in the research process by incorporating public views in the prioritization, review, and translation and dissemination of research. As public participation will foster trust in the clinical research enterprise, increase research participation, address issues of the most importance to communities, and aid the translation of research results into practice. More information is at http://books.nap.edu/catalog.php?record_id=10757.

Next Steps

Establish Common Language for Evidence-Based Healthcare

A common language around EBH has yet to be clearly defined, thwarting effective communication among policymakers, patient/consumer advocates, patients/consumers, and other stakeholders around EBH. The use of one term over the other in policy documents and legislation may inadvertently exclude patients/consumers from participating in the research continuum. Specifically, when reviewing the five example models, organizations often used the terms patient, consumer, and advocate interchangeably. This issue also arose at the Working Group's patient/consumer inclusion forum. In the context of the increased demands to fill evidence gaps, like comparative effectiveness research, it is important to ensure that terminology allows for broad "patient/consumer" participation.

Ensure Substantial Patient/Consumer Involvement Throughout the Research Continuum

In line with the Working Group principles, garnering patient/consumer input throughout the clinical research process is vital to generating, conducting, translating, and implementing evidence that is useful to all stakeholders. In order for patients/consumers and their representatives to engage in the research enterprise meaningfully, the appropriate tools and supports are required. Research organizations or patient/consumer groups can develop these tools. The Project LEAD® science training course created by the National Breast Cancer Coalition helps to train advocates to influence the research process. The Bill of Rights for Parkinson's Clinical Trial Participants by The Parkinson Pipeline Project defines criteria for patient-centered clinical research and patient protection beyond informed consent in an effort to develop greater trust and collaboration among volunteer patient research subjects, study sponsors, and scientists conducting the studies.[7] While many of the models reviewed address this concern, more could be done to facilitate and support patient/consumer engagement. Stakeholders should continue to encourage patient/consumer participation in the implementation phase of research. Although the reviewed

organizations place a premium on patient/consumer involvement, more can be done to ensure that patients/consumers truly influence research activities lead to better policy and care decision-making.

Conduct Further Research to Evaluate Impact of Patient/Consumer Engagement

Organizations conducting research should evaluate their patient/consumer involvement programs. In June 2006, the NHS conducted research to evaluate the influence of patients/consumers on the HTA Programme. The evaluation assessed whether public involvement influences identifying evidence gaps and commissioning and publishing research to fill them. The evaluation led to creation of a plan to improve public involvement in the HTA Programme. Research organizations should be encouraged to conduct similar evaluations to identify best practices and areas for improvement.

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[7] More information on Project LEAD® is at http://www.stopbreastcancer.org/index.php?option=com_content&task=view&id=395&Itemid=138. More information on the Bill of Rights for Parkinson's Clinical Trial Participants is at http://www.pdpipeline.org/advocacy/rights.htm.

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