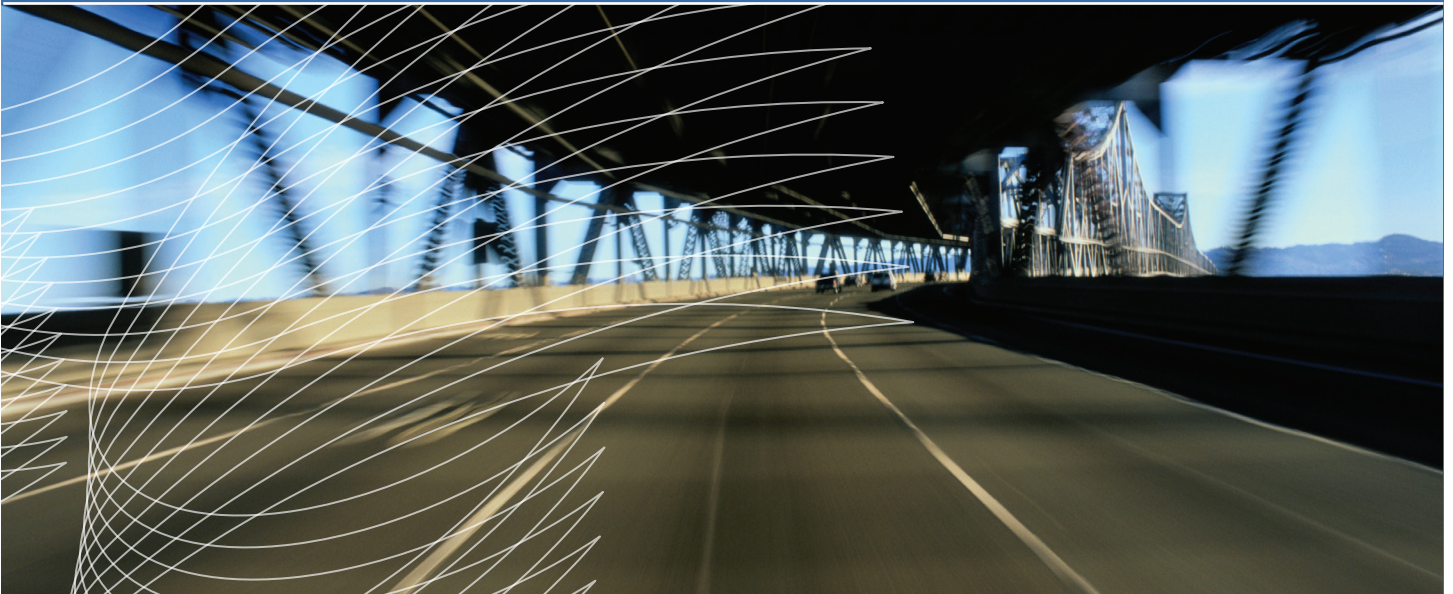


Patient and Clinician Participation in Research Agenda Setting / Lessons for Future Application

Prepared by:



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Executive Summary

Each year, the United States spends billions of dollars on research aimed at improving the health of patients and helping patients and clinicians choose the most appropriate treatments. This vast body of research studies how care is delivered and the safety, efficacy, and effectiveness of medical technologies. Research organizations are equally as varied, ranging from the academic community, to federal government agencies and third-party research organizations.

Congress is considering legislation to expand the nation's capacity to conduct comparative effectiveness research. If passed into law, this new, significant influx of funding will support improving the quantity and quality of research that informs healthcare decision-making. If such an investment is to succeed, it is imperative that the priorities of patients and practicing clinicians are included in the comparative effectiveness research agenda. As policymakers and others debate comparative effectiveness research, some organizations—including U.S. and international federal agencies, as well as patient and provider advocacy organizations—are already considering ways to ensure that the patient and practicing clinician are adequately represented when they set their research priorities.

This white paper is intended to support current and future research efforts by identifying best practices in integrating patients and practicing clinicians in the development of research agendas. These best practices can inform current efforts as well as guide policymakers who are considering new federal investments in comparative effectiveness research. To determine best practices, Avalere conducted primary and secondary research on a variety of organizations both inside and outside the United States that have developed novel programs and approaches.

Highlights of the best practices of these programs include the following:

- Organizations must have a champion with a strong vision to implement a system for patient and clinician input;
- Dedicated staff and funding are necessary to provide sustainability for long-term success;
- Organizations must have willing and motivated volunteers to understand their role and be constructive participants;
- Training of patient and clinician participants is needed to ensure practical and targeted input for decision-making; and
- Measuring program success, although difficult, is critical to establishing long-term program value. Organizations should strive to develop long-term success measures.

Organizations conducting research should recognize the importance of patient and clinician participation in research agenda setting to successfully generate research that improves healthcare decision-making. Organizations should focus on recruiting the *right people*, providing them with the *right tools* and the *right support* in order to succeed. The findings from this white paper support the need for research organizations to make the effort to include patients and clinicians in the research agenda-setting process. By implementing these best practice principles, researchers can help create information that is relevant and valuable to patient and clinician decision-making.

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Introduction

Each year, significant public and private resources are devoted to the clinical research enterprise, yet a gap has been identified between the types of research that are conducted and the types of research that yield the most relevant information for patients and caregivers.¹ For example, patients may be interested in evidence of a therapy's effect on quality of life, including convenience, side effects, and cost, as opposed to clinical or molecular endpoints.

Unfortunately, few organizations have the resources to effectively define a research agenda that more specifically, and in a systematic way, addresses specific patient and clinician needs. However, many organizations are thinking actively about ways in which they can incorporate that patient and practicing clinician perspective in research agendas. For example, the Agency for Healthcare Research and Quality (AHRQ) is one such group that addresses the gaps in knowledge of multiple decision-makers, offering an opportunity for the public to help guide future topics for research.²

In support of the efforts to include the patient and clinician perspective in research agenda setting, the purpose of this white paper is to describe innovative approaches to gaining such input. Avalere reviewed existing programs, which include those both proactively driving research priorities and those providing input into research agendas through reviews of individual research protocols and participation on advisory bodies. The goal of this study is to assist in understanding best practices for patient and clinician participation in research.

Methodology

Avalere conducted the research in two phases. The analysis first consisted of secondary research on organizations soliciting patient and practicing clinician input in identifying research priorities. Leading healthcare organizations were evaluated, including U.S. federal agencies and international organizations with formal programs to solicit patient and provider perspectives as part of their research policy development processes. Patient and provider organizations involved in research decision-making were also examined.

The second phase of the research involved primary research via interviews with selected organizations with innovative approaches to determine key attributes of programs successful in soliciting input from patients and practicing clinicians. Many public, private, and international organizations have developed processes to capture patient and clinician input. For the purposes of this paper, those organizations and programs that focus on healthcare research, priority setting, or developing research agendas were chosen. These programs support a broad concept of healthcare research, encompassing many methods of study intended to increase knowledge and understanding of various healthcare subjects. In addition, organizations that had public availability of information about their programs were chosen for this analysis. The organizations selected for further review included three U.S. federal agencies, two patient organizations, two clinician organizations, and one international organization. Avalere developed a detailed interview protocol to aid in discussions with programs identified in the secondary research phase of the project. Table 1 provides a summary.

¹Wilensky GR, Developing a Center for Comparative Effectiveness Information, *Health Affairs* 25 (2006): w572–w585.

²Slutsky JR and Clancy CM, The Agency for Healthcare Research and Quality's Effective Health Care Program: Creating a Dynamic System for Discovering and Reporting What Works in Health Care, *American Journal of Medical Quality*, Vol. 20, No. 6 (Nov/Dec 2005), 358-360.

Table 1. Summary of Organizations and Programs Reviewed for Patient and Clinician Participation in Research Agenda Setting

Organization	Description of Program Reviewed
U.S. Federal Agencies	
Food and Drug Administration (FDA) – Patient Consultant and Patient Representative programs	Engages patients in FDA and industry meetings after Phase II and in the FDA advisory committees
National Institute for Occupational Safety and Health (NIOSH) – National Occupational Research Agenda (NORA)	Develops occupational research agenda with public and private stakeholders (labor, business, professional societies, academic research, and government agencies)
National Institutes of Health (NIH) – Director’s Council of Public Representatives (COPR)	Provides advice and recommendations to the Director of NIH regarding matters related to medical research, NIH policies and programs, and public participation in agency activities
Patient Organizations	
Juvenile Diabetes Research Foundation (JDRF) – Lay Review Committee (LRC)	Reviews grant applications in conjunction with a scientific review committee to make funding decisions for type 1 diabetes research
National Breast Cancer Coalition (NBCC) – Project LEAD (Leadership, Education and Advocacy Development)	Trains patient advocates to participate in research steering committees, advisory groups, and institutional review boards
Clinician Organizations	
American Academy of Family Physicians (AAFP) – National Research Network (NRN)	Conducts “practice-based research” on primary care issues with physician partners
American Thoracic Society (ATS) – Public Advisory Roundtable (PAR)	Supports participation of public interest organizations in research grant process, standing committees, and public presentations
International Organization	
United Kingdom National Institute for Health and Clinical Excellence (NICE) – Patient and Public Involvement Programme, Citizens Council, Partners Council	Involves both patients and the general public in providing input to NICE on guidance documents, including technology appraisals, clinical practice, and public health guidelines

In reviewing the models described in Table 1, it became clear that the vocabulary used within each organization was very diverse. Participants in the process were referred to as consumers, volunteers, citizens, providers (including clinicians), and workers; the processes were referred to as programs, initiatives, and standing committees. For the purposes of this paper, we refer to all outside stakeholder involvement as patient and clinician participation programs.

To better understand the nature of these programs, we developed a qualitative framework to assess the activities of each. Table 2 displays the five dimensions of patient and clinician involvement that were applied to each organization and program reviewed.

Table 2. Five Dimensions Reviewed for Each Patient and Clinician Participation Program

Dimension	Description
Origin of Program	Impetus, goals, and evolution
Operations of Program	Staff, funding, and specific activities
Criteria for Patient and Clinician Participation	Nature of individuals involved (actual patients, advocates, caregivers, etc.)
Implementation and Use of Patient and Clinician Input	Utilization of patient and clinician input and the degree to which it influences decision-making
Strategies for Continued and Future Patient and Clinician Involvement	Methods to measure impact and create sustained involvement

This paper provides an assessment of the five dimensions of patient and clinician involvement from Table 2 in the eight programs indicated in Table 1.

Results: Trends and Best Practices

A detailed description of how each reviewed organization fit into the assessment framework can be found in the Appendix. We synthesize these research findings below using the assessment criteria we developed to understand the approach each organization takes around utilizing patient and clinician input in priority setting for research. In order to assess broad trends and best practices, for each criterion within our framework, we discuss principles learned, as well as key characteristics of a “model” program.

1. Organizations must have a champion with a strong vision to implement a system for patient and clinician input

The organizations and their programs largely fell into several categories with respect to their program origins. Certain organizations—such as NBCC and JDRF—were founded with a member or volunteer voice as an essential component of their mission, with involvement in research agenda setting an organic outgrowth of this mission. Certain of the government programs studied—such as NICE and NIOSH—were established with an emphasis on public participation. In the case of NICE, this charge was included in the organization’s legislative mandate. Other organizations—such as NIH, FDA, AAFP, and ATS—in recognition of the value other types of input bring to the research process, established patient and clinician involvement programs as a complement to their initial mission.

Vital to each program’s success has been the presence and vision of a champion to garner strong institutional support for the program’s goals. Support for each program within each organization often originated with that organization’s leadership. For example, the ATS PAR program originated at the suggestion of the ATS president, creating an institutional imperative.

Each program also required a strong volunteer advocate base to thrive; in fact, patient advocates for those with HIV/AIDS were the basis for the origin of the FDA Patient Representative Program. From this origin, the FDA program has evolved, thus illustrating the value of a strong advocate base together with a champion. In 1996, President Clinton’s Reinventing the Regulations of Cancer Drugs initiative mandated that a cancer advocate be at the table during advisory committee meetings reviewing cancer drugs. Clinton’s support triggered the expansion and formalization of the Patient Representative Program to include cancer. Since then, the Patient Representative has expanded to include several other serious diseases, and the FDA has developed a second program, the Patient Consultant Program, which focuses on bringing the patient voice into FDA and industry discussions even earlier in the research development process.

Another obstacle that must be overcome is the initial skepticism of the program's value. This especially held true for those programs for which patient or practicing clinician input and involvement appeared to break the traditional research agenda development paradigm. In the case of the NIH COPR, the Director of NIH made it a priority to incorporate the public into the research process. In these cases, many within the organization may not initially see the additional value gained by the addition of patients or practicing clinicians to an existing process, especially given the time and funding necessary to seek this input. As such, these programs often began as small, informal pilot programs to test the concept and gain gradual buy-in. Oftentimes, justification of the program's value is also vital to the success of any patient and clinician involvement.

2. Dedicated staff and funding are necessary to provide sustainability for long-term success

Efficient operations of a patient and clinician participation program are essential to embedding participant perspectives into the larger organization's decision-making and research processes. A vital operational element to the success of each program reviewed is the provision of dedicated staff to these efforts. These staff members can be responsible for various activities, such as recruiting, providing support to members to facilitate successful participation, and sometimes acting as a liaison with program participants and the broader organization.

The recruitment process varied widely among programs. Certain programs actively recruit participants via cooperation with member advocacy organizations, while others post a participant description and solicitation on the program website or in its newsletter. For example, the NBCC's Project LEAD has a formal application and selection process, usually receiving double the number of applications that are accepted. A similar surge in interest in the NIH COPR led NIH staff to develop an alternative opportunity for those not chosen as full COPR members, called the COPR Associates.

Programs also differed with respect to their use of term limits for participants: while some organizations felt that rotating in a fresh perspective is vital to the health of the organization, others stated that participants are encouraged to continue their serving as long as they are willing. Some programs found a middle ground, rotating seasoned members out of their official position, but allowing their continued involvement in a guidance and mentoring capacity.

An ongoing operational struggle for nearly every organization reviewed is the availability of funding. Continued success of these programs depends heavily on the staff and participants' ability to justify continued support, whether via appropriations (for the federal agencies) or leadership grants and fundraising (for private organizations). For instance, the FDA has often raised the idea of expanding the Patient Consultant and Patient Representative programs to additional disease states, but the uncertainty of continued, let alone additional, funding has stalled such an expansion. Many organizations feel that providing evidence of the program's value is vital to justify continued funding and operational support.

3. Organizations must have willing and motivated volunteers to understand their role and be constructive participants

Attracting patients and clinicians who will provide valuable insight based on their unique perspectives is vital to the success of a program. Therefore, attracting passionate and motivated participants is among the top goals cited by all programs. In most cases, participants need direct experience with the particular condition the program supports, either as someone directly affected (FDA, NBCC, NICE, NIOSH), as a caregiver (JDRF, NICE) or as a practicing clinician in that specialty (ATS, AAFP).

Nearly as important as the participant's direct experience is his or her willingness to become a constructive participant in the research prioritization process, which often entails a willingness to

learn the intricacies of the research process. For instance, although members of the NIH COPR represent various stakeholder groups, they all share a demonstrated interest in the work of the NIH. Understanding what is necessary to thoughtfully contribute to the research process is vital to a successful relationship with the particular program, and as such it is often incumbent on the program staff to create a clear and explicit job description when recruiting participants. For instance, NICE council participants are not expected to come up with answers, but to ask the right questions, and the ATS PAR and JDRF seek to fully integrate participants into the research or funding decision-making process.

4. Training of patient and clinician participants is needed to ensure practical and targeted input for decision-making

The programs reviewed represent a wide spectrum of patient and clinician involvement in the research prioritization process. Some organizations allow for public comment, some welcome individual patients and clinicians to serve as a member on an existing advisory body, while some created entirely new advisory bodies to solicit patient and clinician input. Common among all of these models is a desire for an explicit patient and clinician presence in the process, as these perspectives in priority setting contribute not only to the process but also the outcome.

Regardless of the particular model followed, each program recognized the importance of training participants to become successful contributors. Several organizations stressed the importance of knowing the right questions to ask. As such, nearly every organization reviewed provides upfront and/or ongoing training to patients and clinicians to learn the necessary aspects of the organization, internal processes, and roles in order to ensure clear communication among all participants. This aspect also has proven to be important to continued involvement in the decision-making process; training patients and clinicians to speak the same language as the experts adds credibility to their participation. NIOSH has a specific emphasis on teaching participants the “language” of the research process to ensure that all parties are communicating effectively.

Nearly as varied as the models of patient and clinician participation are the outcomes of that participation. The AAFP provides a unique example of a cyclical process for continued clinician involvement. Participating clinicians must not only have the desire to affect change through participation in a research network, but they must provide data based on that research, and be willing to change and adopt new practices as a result of research findings. Other potential outcomes from such programs include comments on the research prioritization, the priorities themselves, or dissemination of findings based on those priorities. Certain models use their program to educate the public, whereas others inform the internal workings of the organization. It is also important for each organization engaged in garnering patient and clinician input to understand how different perspectives can inform the decision-making process; patients, patient advocates, and caregivers may have very different viewpoints on various issues.

5. Measuring program success, although difficult, is critical to establishing long-term program value. Organizations should strive to develop long-term success measures

Every program studied had a strategy for continued and future patient and clinician involvement, primarily revolving around ensuring ongoing reciprocal benefits to the organizations and to the volunteers. Most organizations follow a process for evaluating the success of its patient and clinician involvement effort. This may involve an internal process to justify future funding or publicizing benchmarks for public review. JDRF updates its numerous members through its newsletter. Many times the incorporation of the patient perspective in NICE guidance is a topic of concern and public scrutiny. NICE often works with patient advocacy organizations to translate research findings and disseminate guidance that advocates helped to develop. Similarly, the NIH

COPR proceedings indicate a continuous interest in tracking public involvement in NIH activities, and use of COPR recommendations.

Programs often have difficulty measuring and disseminating success metrics around patient and clinician involvement, and therefore have difficulty justifying continued and additional funding and support. The need for success stories to justify future funding highlights the fact that these outcomes are difficult to measure. For example, the recommendations that serve as the output from an FDA Advisory Committee don't explicitly recognize the input made by the patient representative to the committee. This practice might not achieve tangible results, as several organizations noted that equally as valuable as the actual input given by the patient representatives is the shift in topic, tone, and language made by other representatives due to the simple fact of the patient's presence in the discussions.

Discussion: Broadly Applicable Best Practices

As discussed above, our research on patient and clinician participation in research agenda setting revealed several trends. Despite the fact that some organizations would coalesce around particular criteria, it is important to note that in general, the various categories of organizations and programs that were reviewed tended to cluster; that is, the government programs (FDA, NIOSH, NICE, NIH), patient organizations (JDRF, NBCC), and clinician groups (AAFP, ATS) had a propensity to function similarly. Often observed were similarities in structure, function, and method of garnering patient or clinician input. However, all revealed broadly applicable best practices for patient and clinician participation programs. Table 3 highlights the best practices that emerged for programs seeking patient and clinician participation in research agenda setting.

Table 3. Principles for Involving Patients and Clinicians in Research Agenda Setting

Involvement Criteria	Best Practice Principles
Origin of Program	<p>Strong desire for input often requires a champion</p> <ul style="list-style-type: none"> ▪ Institutional support and identified advocates are important for success ▪ Focused goals and specific objectives define purpose to external stakeholders ▪ For government organizations, legislative mandate may be essential to gaining initial support for such a program
Operations of Program	<p>Dedicated staff and funding are vital to the program's success</p> <ul style="list-style-type: none"> ▪ Steady funding/staff ensure sustainability in program operations ▪ Transparency in funding, recruitment, and scope of involvement in research is vital
Criteria for Patient and Clinician Participation	<p>Programs require willing and motivated volunteers</p> <ul style="list-style-type: none"> ▪ Constructive participation must be the norm ▪ Identifying the specific type of input (patient, advocate, public, caregiver, physician) helps to ensure the proper contribution
Implementation and Use of Patient and Clinician Input	<p>Training of patient and clinician participants is vital to ensuring targeted input for decision-making</p> <ul style="list-style-type: none"> ▪ Clearly defined criteria for decision-making and the role of participants helps create trust ▪ Identifying the nature of the involvement in research (e.g., priority setting, study design, selection of outcomes, and dissemination of results) is key to managing expectations
Strategies for Continued and Future Patient and Clinician Involvement	<p>Programs encounter difficulty in tangibly measuring success</p> <ul style="list-style-type: none"> ▪ Sustaining participant engagement over the long term helps to foster a core group of involved participants ▪ Creating metrics to measure the programs' impact and value will be increasingly important for demonstrating success

The results of the analysis revealed several global trends. Among the most important was attracting patients and clinicians who will provide valuable insight into their particular perspectives. For instance, participants need to understand the inherent tradeoffs in creating a prioritized research agenda—many times funding and research method limitations may prevent some concerns from being answered in a given research study. Participants, particularly advocates, have to be prepared to be constructive participants rather than dissenters in the often rigid research decision-making process to ensure consideration of their perspectives. In order to achieve the goal of garnering patient and clinician input, it is important to adequately define the role the patient and clinician will be expected to play, and then provide the appropriate tools to help them succeed in that role.

Nearly every program provides upfront and/or ongoing training to volunteers to learn the necessary aspects of the program, in order to ensure clear communication among all participants. Each organization also provides dedicated support staff to volunteers to help facilitate successful participation, which may include mediation for volunteers who feel that their voice is not being heard. An ongoing struggle for nearly every program was the adequacy of support, especially financial.

Measuring the impact of patient and clinician involvement in research decision-making emerged as the next step for further development and broad enhancement of these programs. Anecdotally, it is easy to point to examples of the beneficial impact of patient and clinician input. All organizations interviewed described the immense benefit their programs brought to the clinical research enterprise. Among these benefits were the creation of common goals for supporting research and more informed participants in this process, more valuable research that is relevant to patient and clinician decision-making, and increasing researcher and decision-maker awareness of the real-world implications of their findings.

However, capturing how patient and clinical participation contributes to the improvement of the research is the next step in understanding how patient and clinician input ultimately leads to improving patient outcomes. This qualitative or quantitative evaluation must measure the degree of involvement and the influence of that involvement on a decision-making or research agenda setting. Doing so will help to validate the importance and value of including the patient and clinician perspectives in these endeavors. When organizations are considering the creation of patient involvement programs they should also be considering ways to capture the return on investment and impact of their contributions.

Despite the challenges in measuring the success of a patient or clinician participation program, as the discussion of informed medical decision-making evolves, ensuring that the patients' and practicing clinicians' perspectives are recognized in the agenda-setting process is important for the ultimate success of new investments in research. The key to continued and future success and growth of these programs involving patients and clinicians in research agenda setting is making participants full partners in the decision-making process. As others consider integrating the patient and clinician voice into research decisions, ensuring the recruitment of the *right people*, providing them with the *right tools* and *right support* are critical to achieving maximum success.

Appendix: Detailed Description of Programs Reviewed

Table A1. Food and Drug Administration (FDA) Patient Representative and Patient Consultant Programs

<p>1. Origin of Program</p>	<ul style="list-style-type: none"> ▪ Patient Representative Program grew out of AIDS advocates challenging FDA to involve advocates in the FDA Advisory Committee process during the review of AIDS-related drugs. Involvement of advocates is now seen as a valuable part of the review process ▪ Cancer advocates soon asked for the same involvement, so in 1995, the first cancer advocates participated in the Oncologic Drugs Advisory Committee ▪ In 1996, President Clinton's Reinventing the Regulations of Cancer Drugs initiative mandated that a cancer advocate be at the table at advisory committee meetings where cancer drugs were being reviewed ▪ In December 1996, FDA requested public comment on a proposal to recruit cancer patient advocates to serve as patient representatives. ▪ After comments were received and considered, the recruitment initiative was begun as a pilot and today is a full-fledged program recruiting patient representatives to serve across many serious disease areas. ▪ Participation at Advisory Committee meetings occurs at the end of the drug review and approval process. In 2001, the Patient Consultant Program was started to engage advocates earlier in the process (end of Phase II meetings) ▪ Some opposition occurred in the beginning, due to skepticism that patients could not be objective, but now the programs have gained wide acceptance and are valued by advocates, FDA, and industry
<p>2. Operations of Program</p>	<ul style="list-style-type: none"> ▪ Limited resources for both programs ▪ Developed selection criteria, evaluation form, recruitment letter; public comment on these items ▪ Patient Representatives and Patient Consultants are cleared as special government employees (SGEs), mirroring clinicians/scientists on advisory committee member clearance ▪ Recruitment: <ul style="list-style-type: none"> ▪ Flyer sent out with criteria in resume format ▪ Recruit on an as-needed basis
<p>3. Criteria for Patient/Clinician Participation</p>	<ul style="list-style-type: none"> ▪ Select patients who are active in their advocacy community and want to become students of the drug review and approval process ▪ Recruit patients who are willing to learn about the organization and process, dedicate their time, actively participate in the process, and take what they have learned back to their patient community ▪ No term limits, prefer long-term involvement

Table A1 continued on next page

4. Implementation and Use of Patient and Clinician Input

- Training of participants involves monthly phone lectures by FDA staff on various topics. Highly interactive, with open question and answer opportunity. In addition, hold an annual Patient Consultant Workshop
- After September 11, 2001, many U.S. citizens were reluctant to travel. FDA wanted a way to initiate the program sooner rather than later to assure that the momentum of the new program was not lost. The solution was telephone lectures. The telephone lectures are now the backbone of the program
- Open question period helps bring group together
- One-on-one orientations with patient representatives and consultants to get them grounded in “FDA 101”
- Participants encouraged to call with any questions
- Staff debrief with advocates after committee meetings
- Contribute to FDA Product Review in the areas of trial design, entry criteria, endpoints, drug toxicity issues, quality of life issues, study recruitment, informed consent, expanded access, and product labeling
- Patient Reps and consultants point out unclear language in informed consent forms
- Assist in clarity of labels
- Assist with understanding quality-of-life issues
- Patient Reps and consultants read and comment on guidance
- On advisory committee, Patient Representatives have a vote as do all members of the committee
- For effective involvement advocates need to understand the drug review and approval process.
- Patients are considered full members of the process

5. Strategies for Continued and Future Patient/Clinician Involvement

- Include more disease areas
- Try to secure more resources and include more patient consultants
- Consideration is now being given to combining the Patient Representative and Patient Consultant Programs

Table A2. National Institute for Occupational Safety and Health (NIOSH)-National Occupational Research Agenda (NORA)

1. Origin of Program	<ul style="list-style-type: none"> ▪ NIOSH and its public and private partners brought together approximately 500 organizations and individuals outside of NIOSH to participate in the development of the NORA ▪ Originally developed in 1996 to provide a framework to guide research for NIOSH and other researchers ▪ Second decade of program focusing on a sector-based structure to better move research to practice within workplaces, organized around industry sectors ▪ Due to the nature of the NIOSH, workers have always been involved in the process ▪ Sometimes encountered difficulty in getting organizations to partner in research, for example if researchers need access to their injury data
2. Operations of Program	<ul style="list-style-type: none"> ▪ Approximately 500 organizations and individuals participate ▪ NIOSH Office of the Director responsible for leadership ▪ Three individuals involved in management of the sectors, and two individuals involved in each sector: one coordinator and one assistant coordinator ▪ Recruit via online newsletter, website, and council member outreach
3. Criteria for Patient/Clinician Participation	<ul style="list-style-type: none"> ▪ Focus on openness and inclusiveness via the broadest possible public participation ▪ Initial priority list selected by a working group of senior scientists both inside and outside NIOSH, which were then vetted by researchers, stakeholders, and health professionals ▪ Agency representatives from 31 federal agencies or programs ▪ Participation in NORA is broad, including stakeholders from universities, large and small businesses, professional societies, government agencies, and worker organizations
4. Implementation and Use of Patient and Clinician Input	<ul style="list-style-type: none"> ▪ Uses a consensus-building process to determine final research priorities based on input from working groups, written comments, oral comments made at public and town meetings, and other comments made during deliberations throughout the process ▪ Involvement ranges from providing input electronically to volunteering for a Sector Council ▪ Participation includes two face-to-face meetings per year, plus periodic teleconferences ▪ Develop priorities that guide research in both the public and private sectors, with decision-makers working in all areas of the occupational safety and health field using the research agenda ▪ Diverse parties collaborate to identify the most critical issues in workplace safety and health. Partners then work together to develop goals and objectives for addressing these issues ▪ Some members rotate onto councils and some participants work on “special projects” if there isn’t room on the councils
5. Strategies for Continued and Future Patient/Clinician Involvement	<ul style="list-style-type: none"> ▪ Although research is often performed, with no legislative authority it is hard to get results implemented into the workplace ▪ External partners have to be involved to assist with implementation ▪ Difficult to measure success because NIOSH is not involved in implementation of guidelines and reforms

Table A3. NIH Director's Council of Public Representatives (COPR)

1. Origin of Program	<ul style="list-style-type: none"> ▪ Authorized in the Public Health Service Act ▪ Established by former NIH Director Harold Varmus, MD, following the 1998 release of the report "Scientific Opportunities and Public Needs" by the IOM, which called for stronger, more formal mechanisms for public input in the NIH decision-making and priority-setting processes
2. Operations of Program	<ul style="list-style-type: none"> ▪ Committee is governed by the provisions of the Federal Advisory Committee Act ▪ COPR consists of up to 21 members appointed by the NIH Director ▪ Estimated annual costs for operating the COPR, including compensation and travel expenses for members but excluding staff support, is \$222,351 <ul style="list-style-type: none"> » Estimate of annual person-years of staff support required is 1.30 at an approximate annual cost of \$135,248 ▪ COPR members are chosen through a nationwide application process to serve up to three-year terms on the Council (were originally selected via a nomination process) ▪ As NIH receives many applications, they established the COPR Associates Program to maintain contact with these qualified individuals
3. Criteria for Patient/Clinician Participation	<ul style="list-style-type: none"> ▪ Members represent the various stakeholders interested in NIH and may include patients, family members of patients, healthcare professionals, scientists, health and science communicators, and educators
4. Implementation and Use of Patient and Clinician Input	<ul style="list-style-type: none"> ▪ As needed, the COPR may seek advice from special consultants, assemble ad hoc working groups, and convene conferences, workshops, or other activities ▪ COPR members participate as reviewers and judges for consumer-oriented products produced by NIH communications offices and entered into the NIH Plain Language Awards Program ▪ COPR Associates comment on NIH draft strategic plans, guidelines, websites, and other documents and participate in NIH panels, workgroups, and meetings ▪ Provides advice and recommendations to, and consults with, the Director of NIH regarding matters related to medical research, NIH policies and programs, and public participation in agency activities ▪ Meetings held approximately two times a year, and are open to the public <ul style="list-style-type: none"> » Agenda topics initiated by COPR members and the NIH Director ▪ In addition, COPR members participate in work groups to further examine issues of public interest or to respond to charges and requests from the NIH Director ▪ The COPR role is advisory; however, COPR advice carries considerable weight, and members are valued for their credibility and expertise ▪ Recently incorporated a consensus-building cycle
5. Strategies for Continued and Future Patient/Clinician Involvement	<ul style="list-style-type: none"> ▪ COPR Members take part in outreach activities, such as speaking to community groups about the NIH and its programs and serving on NIH working groups and review panels ▪ Unless renewed by appropriate action prior to its expiration, the Charter of the COPR will expire November 3, 2008

Table A4. Juvenile Diabetes Research Foundation (JDRF) Lay Review Committee (LRC)

1. Origin of Program	<ul style="list-style-type: none"> ▪ JDRF was founded in 1970 by parents of children with type 1 diabetes, with the mission to find a cure for type 1 diabetes through the support of research ▪ JDRF has grown and now contains chapters in every U.S. state and world affiliates ▪ Funded \$140 million in diabetes research in 2007 ▪ Entire history of JDRF has been volunteer-driven; LRC seen as “heart and soul” of the organization
2. Operations of Program	<ul style="list-style-type: none"> ▪ Over 100 staff in New York broken into different areas (management, research, communications, etc) ▪ Hubs in New York and Washington, DC, 90 chapters around the country (most small) ▪ JDRF research consists of five major topic areas, each with its own scientific staff ▪ Every decision is in partnership with the lay members
3. Criteria for Patient/Clinician Participation	<ul style="list-style-type: none"> ▪ JDRF’s LRC is comprised of specially trained volunteers who often, but not always, have a personal connection to diabetes ▪ Members recruited through regional chapters via a nominating committee, nominees attend and observe LRC activities, then nominations are finalized
4. Implementation and Use of Patient and Clinician Input	<ul style="list-style-type: none"> ▪ Organized a partnership program whereby seasoned lay volunteers mentor newer volunteers ▪ Members have term limits, but many <i>emeritus</i> members stay on as ad hoc reviewers ▪ JDRF’s grants process includes the LRC in the review of applications for research grants, and in the annual review of progress of grant awards ▪ All JDRF grant applications are subject to a dual review system by the Medical Science Review Committee and by the LRC, both of which evaluate the scientific merit of each application, the qualifications of the investigator, and the relationship of the proposed research to the cause, cure, treatment, and/or prevention of diabetes and its complications ▪ Lay volunteers sit on MSRC meetings; scientists appreciate the feedback and questions ▪ The LRC places particular importance on research that may be rapidly translated into clinical applications and research deemed to have the greatest impact ▪ The LRC takes rank ordered list of grants (ordered via NIH scoring by the MSRC) and works with JDRF staff to make final funding decisions (staff puts each application in the context of the current JDRF research portfolio, and how to fill the gaps) ▪ LRC selects grants for funding according to their priorities; thus, a lower scoring grant could receive funding because of the impact it may have
5. Strategies for Continued and Future Patient/Clinician Involvement	<ul style="list-style-type: none"> ▪ Success is measured through monthly evaluations of goals, yearly and three-year goals and milestones, vetted by the International Board ▪ Dissemination via website, <i>Countdown</i> magazine, newsletters, online support team, etc.

Table A5. National Breast Cancer Coalition (NBCC) Project LEAD (Leadership, Education, and Advocacy Development)

<p>1. Origin of Program</p>	<ul style="list-style-type: none"> ▪ NBCC started in 1991; soon conceived of and spearheaded a new model of consumer involvement and innovative research at the Department of Defense (DOD) peer-reviewed breast cancer research program, attracting more than 18,000 proposals since its inception ▪ Basic Science Project LEAD launched in 1995 ▪ The goals for the Basic Science Project LEAD have remained the same and the curriculum is subject to ongoing review and development. Two additional courses have been developed: Clinical Trials Project LEAD and Quality Care Project LEAD
<p>2. Operations of Program</p>	<ul style="list-style-type: none"> ▪ Project LEAD is supported by a grant from the AVON Foundation through the AVON Breast Cancer Crusade, and from NBCCF operational funds ▪ Program budget - \$1,177,108 ▪ Membership for NBCC is \$35 ▪ Course held four times a year; different cities each year ▪ Renowned scientists from academic and research institutions serve on the Project LEAD faculty ▪ No charge for the course apart from travel and hotel accommodations; scholarships available for those with serious financial need ▪ Staff <ul style="list-style-type: none"> » Annette Bar-Cohen is director of programs » Project LEAD coordinator » Quality Care Analyst » Two facilitators (one does science and clinical trials, other Quality Care) ▪ There is an application and selection process for membership; NBCC usually receives double the number of applications than can be accepted
<p>3. Criteria for Patient/Clinician Participation</p>	<ul style="list-style-type: none"> ▪ Current individual NBCC members are eligible to apply to Project LEAD ▪ Applicants are not required to have a science background ▪ Requirements for acceptance to the course include the following: <ul style="list-style-type: none"> » Experience with breast cancer advocacy in one's community » Interest in learning the language and concepts of science » A clear personal connection to breast cancer » A commitment to continue learning about scientific concepts ▪ Willingness to prepare for, and participate in, entire four-day course

Table A5 continued on next page

4. Implementation and Use of Patient and Clinician Input

- Education:
 - » Through lectures, group study, critical appraisal of scientific articles and research proposals, Project LEAD graduates learn:
 - Basic science
 - Basic epidemiology
 - Leadership and advocacy development skills
- Goals for Project LEAD participants:
 - » Discover how to critically appraise scientific literature
 - » Acquire study skills necessary to remain educated on scientific aspects of breast cancer
 - » Study how breast cancer research decisions are made
 - » Become familiar with consumer advocacy opportunities
 - » Gain confidence to contribute by asking questions and finding common ground with scientists
- Send out news alerts to network of advocates and activists
- Clinical Trials Project LEAD (advanced course) open to LEAD graduates
 - » Graduates collaborate in clinical trials and are included in trial design, oversight, and outreach
 - » Graduates ensure the right research gets done correctly and quickly
 - » They ensure that policies encourage access to trials
 - » Develop materials from trials to educate community
- Quality Care Project LEAD – trains advocates to:
 - » Improve the quality of breast cancer care through systems change
 - » Assure that NBCC’s quality care core values translate into meaningful measures of quality
- In clinical trial partnerships, NBCC provides advocate input on study design and implementation; input to design of outreach materials; and grassroots assistance with patient accrual and dissemination of information
- Partners are expected to: provide NBCC with opportunities for meaningful input, review, and membership on all relevant committees; keep NBCC updated on progress and status to the extent feasible; and publish primary study results in peer-reviewed journals regardless of outcome or FDA approval
- Input is valued at the same level as the clinicians and researchers
- Many review grants for DOD
- Expected to work with NBCC and other LEAD graduates to encourage and strengthen consumer participation in breast cancer research
- Since June 1995, over 1,300 advocates have attended Project LEAD. Graduates have:
 - » Served on program steering committees at the state or local level
 - » Served on advisory boards at the state or local level
 - » Served on study sections within the U.S. DOD Breast Cancer Research Program, California Breast Cancer Research Program, and other state research programs
 - » Served on IRBs at hospitals or universities
- Created patient resources, educational tools, and outreach materials for their communities

5. Strategies for Continued and Future Patient/Clinician Involvement

- Evaluate courses, instructors, and identify strong people within the courses
- Undertook extensive evaluation of as many graduates as possible to see what they have done since and put information in a compendium
- Continuing education track for LEAD graduates once/year—dealing with deeper science issues
- LEAD graduates have opportunity to participate in Journal Club, to stay abreast of developments in breast cancer research and continue refining ability to critically appraise both epidemiology and basic science research studies
- Very active email list; NBCC will launch a continuing education website to complement and continue Project LEAD training

Table A6. American Academy of Family Physicians (AAFP) National Research Network (NRN)

<p>1. Origin of Program</p>	<ul style="list-style-type: none"> ▪ In August 1999, the AAFP established the National Network for Family Practice and Primary Care Research (now called the AAFP NRN). From 1999 to today, the growth of practices involved with the NRN has been steady as physicians are empowered through research to help their communities and world ▪ Many NRN clinicians were recruited from the former Ambulatory Sentinel Practice Network (ASPN), a North American primary care practice-based research network established in 1978. ASPN ceased operations in 1999, however, the legacy of ASPN's success lives on in the AAFP NRN ▪ The Board of Directors of the AAFP established the AAFP NRN to preserve the momentum of ASPN in studying the phenomenon of primary care. The major contributors to the development and long-standing success of ASPN have rallied their support behind the AAFP NRN and can provide the benefit of ASPN's 20+ years of experience in practice-based research ▪ The board-approved mission of the NRN is to conduct, support, and promote primary care research in practice-based settings that addresses questions of importance to the discipline of Family Medicine and improves healthcare and the health status of patients, their families, and communities ▪ 14 research networks now in place
<p>2. Operations of Program</p>	<ul style="list-style-type: none"> ▪ The NRN is part of the AAFP Scientific Activities Division, directed by Herbert F. Young, MD, MA, FFAFP, and has 12 staff devoted to the program ▪ To date, the NRN has received nearly \$7,500,000 in new multi-year funding ▪ Formally affiliated with 14 regional family medicine Practice Based Research Networks (PBRNs) ▪ Budget and Supporters <ul style="list-style-type: none"> » \$300,000 from the Robert Wood Johnson Foundation's Prescription for Health initiative » \$50,000 from the U.S. Department of Health and Human Services' Office of Research Integrity (through the Association of American Medical Colleges) » \$531,221 from the Agency for Healthcare Research and Quality (AHRQ), through a subcontract with Olmsted Medical Center » Two awards totaling \$135,576 from the AHRQ-funded PBRN Resource Center at Indiana University Purdue University at Indianapolis » \$67,000 from John Mullaly, MD, a family physician in Port Huron, Michigan » \$322,814 from Opt-E-Scrip, Inc » \$1,977,296 from the PepsiCo Foundation for a joint NRN-Americans In Motion fitness and health education intervention project ▪ Infrastructure needs support—difficult to find funds for that and is a concern of the Academy
<p>3. Criteria for Patient/Clinician Participation</p>	<ul style="list-style-type: none"> ▪ Primary care clinicians willing to be involved and proceed with projects ▪ Must supply data, continue membership, and participate in NRN events at least every two years ▪ To be in NRN, should have at least 5 practices and 15 clinicians, but most have 10 practices and 30-35 clinicians
<p>4. Implementation and Use of Patient and Clinician Input</p>	<ul style="list-style-type: none"> ▪ Advisory Group meetings ▪ Scientific Review Committees ▪ Annual Convocation ▪ Participating clinicians carry out research agenda set by NRN ▪ Providing better patient care ▪ Improving their office systems ▪ Obtaining new equipment ▪ Expanding reimbursement
<p>5. Strategies for Continued and Future Patient/Clinician Involvement</p>	<ul style="list-style-type: none"> ▪ Measuring success via how many practices they "touch," and whether practices actually change ▪ Future goals include working more with electronic systems, improving quality of data, answer more specific research questions, and translating population-based research into individual patient care

Table A7. American Thoracic Society (ATS) Public Advisory Roundtable (PAR)

<p>1. Origin of Program</p>	<ul style="list-style-type: none"> ▪ PAR started in 2001 by suggestion of ATS President. To provide “Patient arms” to the society, and advocacy. ▪ It is not a program, but a standing committee of society members. ▪ It is now an official charter of ATS, but functions as a committee with more flexibility and autonomy ▪ Founded by representatives from public interest organizations; while they represent their organization on PAR, they also represent themselves on the committee ▪ In the beginning, PAR was a vague concept and unclear how useful/doable it was. Now that it is five-years-old it is more formal
<p>2. Operations of Program</p>	<ul style="list-style-type: none"> ▪ Council of Public Representatives (CPR) is an extension of PAR and allows groups that are not part of PAR to work on issues ▪ PAR functions as an internal committee with full access to staff and leadership ▪ Try to keep 15 organizations on the roundtable—there is a commitment to rotate off so that more patient organizations have the opportunity to serve on PAR ▪ No set terms, terms can vary (consider rotations annually). More important that all diseases are covered from patient perspective ▪ ATS does not perform research but gives grants (partners help fund) ▪ PAR representative on at least half of the standing committees (try to have a “voice” wherever there is a patient issue) ▪ Monthly ATS conference calls with PAR ▪ PAR chair serves on board of ATS (new bylaws less than three-years-old) ▪ Since PAR is an internal committee, they are fully integrated with ATS ▪ PAR committees are more informal, but the appointments on the standing ATS committees are done once a year. PAR provides recommendations to the ATS President as to which committees should include a PAR member and they suggest the person to serve on that committee. On an ad-hoc basis PAR members are appointed on committees the same way ATS members are ▪ PAR works formally to help ATS with all patient issues
<p>3. Criteria for Patient/Clinician Participation</p>	<ul style="list-style-type: none"> ▪ Not-for-profit health related organizations, patient, or family member/caregiver who clearly represents individuals with respiratory diseases, sleep-related conditions, or critical illness ▪ Direct connection with patients or connection to respiratory diseases, sleep-related conditions, or critical illnesses ▪ Support the mission and vision of ATS PAR ▪ Able to put personal interests aside and work for the good of the entire group ▪ Active involvement on an ATS PAR Action team or taskforce
<p>4. Implementation and Use of Patient and Clinician Input</p>	<ul style="list-style-type: none"> ▪ There are over 25 ATS committees, approximately half of each have a PAR representative ▪ New initiatives are introduced as needed, may be as informal as an email from the President of ATS. ▪ Scientific meetings with patient speakers ▪ Brochures on conditions and lay summary versions of ATS documents ▪ Do not physically involve patients in the research ▪ Approach patient groups if they would match grants for a way to get them more involved and raise contribute more money to research
<p>5. Strategies for Continued and Future Patient/Clinician Involvement</p>	<ul style="list-style-type: none"> ▪ Since it is not a program, there are no success measures but the working committee does have goals ▪ System for tracking past awardees is in place and ATS staff are working on expanding these offerings

Table A8. National Institute for Health and Clinical Excellence (NICE) Patient and Public Involvement Programme, Citizens Council, and Partners Council

<p>1. Origin of Program</p>	<ul style="list-style-type: none"> ▪ Since NICE is funded by the public and its guidance has an impact on patients, it seemed natural to include their input ▪ Recognition that patients bring a unique perspective to the discussion and that perspective should be addressed ▪ NICE has a designated team (the Patient and Public Involvement Programme), which helps NICE identify and develop opportunities for patient and public involvement
<p>2. Operations of Program</p>	<ul style="list-style-type: none"> ▪ Open advertising for all vacancies with job descriptions and specifications specific to group or project people are being recruited to ▪ At least two lay people on all committees and development groups that produce clinical or public health guidance ▪ Individuals are nominated to the Partners Council
<p>3. Criteria for Patient/ Participation</p>	<ul style="list-style-type: none"> ▪ Patient and public members of advisory bodies: <ul style="list-style-type: none"> » Not necessary to have a scientific background » Expected to be familiar with a condition (e.g., personal experience, experience as a caregiver, and/or patient group members or staff with and without the condition who have an understanding of the views of the people their group represents) » Bring an individual perspective, not an organizational view » Participants are not expected to come up with answers, but to ask the right questions ▪ Citizens Council <ul style="list-style-type: none"> » Members bring the perspective of the general public » Unlike the above, Council members should not be activist patients and are not selected because they have a specific condition or area of interest » Council members use deliberative processes, and hear from expert witnesses (including expert patients) to provide a public view on the societal or ethical issue they are asked about ▪ Partners Council focuses on corporate governance rather than guidance production.

Table A8 continued on next page

4. Implementation and Use of Patient and Clinician Input

- Dedicated training and support offered to all successful applicants
- All NICE guidance produced in versions written specifically for patients, caregivers, and the public
- The PPIP: A dedicated team within NICE develops and supports opportunities for patient, caregiver, and public involvement. The PPIP:
 - » Provides advice on developing and supporting opportunities for patient, caregiver, and public involvement
 - » Identifies organizations that represent patient, caregiver, or public interests that may have an interest in specific guidance topics on NICE's work program
 - » Runs workshops for organizations that represent patient, caregiver, or public interests that want to know more about how NICE guidance is produced and how to contribute to its development
 - » Provides information, training, and support to individual patients, caregiver, and members of the public. Support ranges from informal telephone advice to formal training workshops
 - » Comments from a lay perspective on draft guidance issued by NICE
 - » Evaluates patient/caregiver/public involvement in NICE activities.
- Patient membership of advisory bodies
 - » Patient, caregiver, and public members contribute directly to the production of the guidance
- Four main ways for patients and public to influence processes:
 - » Patient/public membership of NICE advisory bodies (at least two lay people on all committees and development groups that produce clinical or public health guidance)
 - » Consultation - Organizations representing the views of patients, caregivers, service users, and the public can suggest topics for future development and register an interest in topics under development. These groups can then comment on NICE processes and for individual guidance topics they can comment on the draft scope (in writing or in person at dedicated workshops), submit evidence for consideration by the advisory body, and comment on draft recommendations. Individual patients, caregivers, and members of the public can suggest future topics and comment on draft recommendations via the website
 - » The Citizens Council brings the views of the public to NICE decision-making. Consisting of 30 members, the Council meets twice a year and provides advice in response to a specific questions of importance to NICE
 - » The Partners Council Individuals are nominated to provide NICE with the views of a range of organizations that have an interest in our work (mainly corporate governance issues)

5. Strategies for Continued and Future Patient/Involvement

- Patient involvement can sometimes add time to the process, so NICE must continue to show the benefits of having patient involvement
- NICE will build on existing work and develop new opportunities in the following ways:
 - » Share lessons learned from involving patients, caregiver, and the public across different parts of NICE's work program
 - » Develop processes and methods to evaluate the successes and limitations of patient, caregiver, and public involvement work at NICE
 - » NICE commissioned the Open University to undertake an evaluation of the Citizens Council
 - » NICE commissioned an independent researcher to undertake an evaluation of patient and caregiver membership of guideline development groups (to be repeated next year)
 - » NICE commissioned and acted on an evaluation of its patient information
 - » A project is currently underway to identify how to disseminate patient/public versions of guidance to "hard-to-reach" groups

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