Engaging and partnering with patients and families in health research

Concepts, evidence, challenges and opportunities

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April 6, 2017
Toronto, ON

Locating myself in this field

- social scientist and health policy researcher

- 20-year observer and analyst of trends in public involvement in health policy (including planning, priority setting, resource allocation and governance)

- expertise in design, implementation and evaluation of public engagement methods

- more recent focus on public and patient involvement in *health research*

Brief history and key developments in citizen and patient engagement

1970-2000: ‘the public consultation era’

- citizen involvement and representation in health care planning, health services design, priority setting and resource allocation
- early years of patient involvement in health research (e.g., setting priorities for research, guidelines development, health technology assessment)
- focus on citizens and patients as consultants or research subjects (traditional consultation methods)

Brief history (2)

2000-2010: ‘the public engagement era’

- shift from the *consultation* to the *engagement* era (use of deliberative processes, citizen councils, panels to inform decision making)
  - citizen engagement in health system reform (Romanow Commission, 2002-2004)
  - institutionalized citizen engagement bodies (2003-2006)
    - National Institute for Health and Care Excellence (NICE) Citizens Council (UK)
    - Citizens’ council for the public drug program (Ontario)

- emphasis on patient safety and quality improvement agendas (and related patient engagement activities)
  - Canadian Patient Safety Institute
  - Excellent Care for All Act (Ontario)
  - Patient and Family Advisory Councils
  - Canadian Foundation for Health Care Improvement

Brief history (3)

2010-present: ‘the patient engagement and partnership era’

- Increasing worldwide attention to involving patients in various health system domains
    - quality improvement
    - planning and policy making
    - health technology assessment
    - health research
- Shift from patients as research subjects to more active participants, collaborators and partners

Major strategic investments

- re-orienting clinical and health systems research in the U.K., U.S.A. and Canada to the needs and priorities of patients


INVOLVE

NHS
National Institute for
Health Research

Briefing notes
for researchers:
public involvement in
NHS, public health and
social care research

Supporting public involvement
in NHS, public health and
social care research

Why PCORI Was Created

Patients, their families, and clinicians face a wide range of complex and often confusing choices when it comes to addressing their health and healthcare concerns. They need trustworthy information to decide which option is best for them.

Unfortunately, traditional medical research, for all of the remarkable advances in care it produces, hasn't been able to answer many of the questions that patients and their clinicians face daily. And even when reliable information does exist, it's not always available to patients, or those who care for them, in ways they can understand or use most effectively.

PCORI was established to help address these challenges. Our job is to close the gaps in evidence needed to improve key health outcomes. To do this, we identify critical research questions, fund patient-centered comparative clinical effectiveness research, or CER, and disseminate the results in ways that the end-users of our work will find useful and valuable.

For patients, this strategy means we must provide information about which approaches to care might work best, given their particular concerns, circumstances, and preferences. For clinicians, it means we must focus on providing evidence-based information about questions they face daily in practice. For insurers, it means we must produce evidence that can help them make the best decisions on how to improve health outcomes for their members. For researchers, it means we must support for studies designed to build a badly needed base of useful evidence for improving outcomes in high-burden, high-impact conditions.

Posted: September 10, 2014

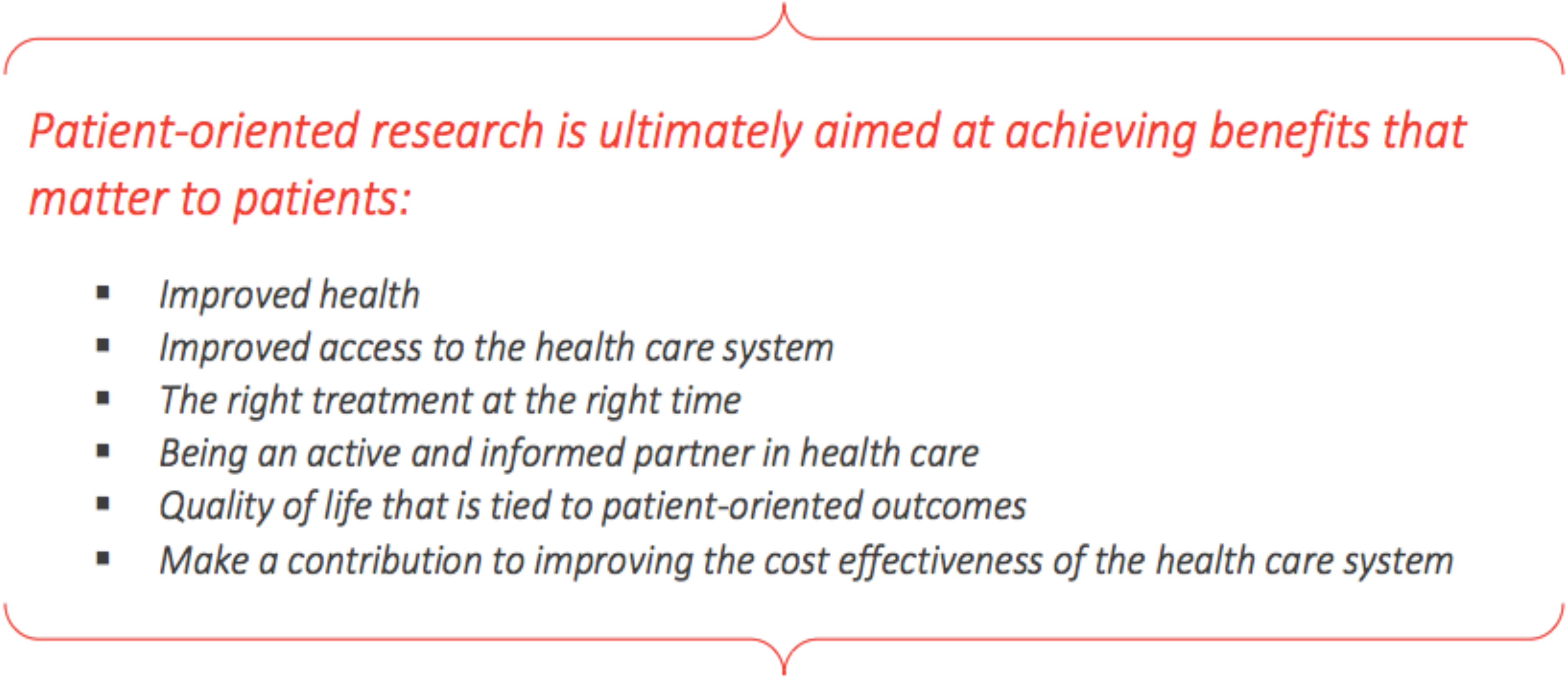

Canadian Institutes of Health Research
Instituts de recherche en santé du Canada

Canada

Strategy for Patient-Oriented Research
SPOR
Putting Patients First

Strategy for Patient-Oriented Research
Patient Engagement Framework

Canada's Strategy for Patient-Oriented Research (SPOR)



Patient-oriented research is ultimately aimed at achieving benefits that matter to patients:

- *Improved health*
- *Improved access to the health care system*
- *The right treatment at the right time*
- *Being an active and informed partner in health care*
- *Quality of life that is tied to patient-oriented outcomes*
- *Make a contribution to improving the cost effectiveness of the health care system*

Patient Engagement in SPOR

- Patient-oriented research: continuum of research that engages **patients as partners,** focuses on **patient-identified priorities** and **improves patient outcomes**. Aims to **apply the knowledge generated to improve healthcare systems and practices**

- Patient engagement: **meaningful and active collaboration** with patients in governance, priority setting, conducting research and knowledge translation

Canadian Institutes of Health Research
Instituts de recherche en santé du Canada
Canada

Strategy for Patient-Oriented Research
SPOR
Putting Patients First

Strategy for Patient-Oriented Research
Patient Engagement Framework

Johnson *et al. Research Involvement and Engagement* (2016) 2:6
DOI 10.1186/s40900-016-0020-4

Research Involvement
and Engagement

COMMENTARY

Open Access

The patient voice in research—evolution of a role

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Plain english summary: Engaging patients in research studies is becoming more common because it makes research and its results more relevant for patients. It is important to understand the best ways for patients and researchers to work together. Patients who are included as active partners in research can provide useful input on what it is like to work on a research team but very little has been written about this from the patient's perspective. As patient partners and researchers on a breast cancer study, we share our experience to develop a patient-centered project and the inclusion of patient collaborators as scientific experts. Over time, the role of the patient partner has developed to include unanticipated roles and responsibilities. We use our experience to share how the patient voice can affect the execution of a research study and to provide a model for meaningfully engaging patients in research.

PATIENT ENGAGEMENT

On the path to a science of patient input

Margaret Anderson* and K. Kimberly McCleary*

It is early days in the creation of a science of patient input. Participants are establishing rigorous methods to better integrate patient perspectives, needs, and priorities throughout biomedical and bioengineering R&D and care delivery to patients. To assess progress and unmet needs, *FasterCures* tracked more than 70 collaborative initiatives clustered in six categories that are defining and shaping this developing field. No longer is patient engagement a fanciful notion as it was at the start of our journey in 2003, and the rush of activity is welcome and vital.

In the 21st century, market research is a business imperative for most industries. In 2011—decades after Steve Jobs famously said, "A lot of times, people don't know what they want until you show it to them"—Apple started a market research group that sends anonymous surveys to invited users to find out exactly what they want from their devices. In January 2016, IBM formally launched a company-wide process to shift its culture to focus on users' needs (*1*). Health care and the research and development (R&D) of biomedical products have lagged behind other technology sectors in moving toward consumer-centered practices. Now, as a result of multiple cultural influences and pragmatic factors, the mindset of these stakeholders is changing, and the patient's role is expanding (*2*). Momentum is building to incorporate patient preferences into the biomedical R&D system so that products and services better align with patient needs, improve individual and public health, and reduce time and spending on unproductive care.

With its broad network of stakeholders—patient organizations, industry, academia, government, and funding agencies—*FasterCures* has a distinct vantage point into this landscape of new patient-centered activities; such information is crucial to the creation of a new field: The science of patient input. The goals of this new field are to develop rigorous methods so as to better integrate patient perspectives, needs, and priorities across the translational research continuum. In this Perspective, we summarize and encourage broad use of resources that are already available, and we capture a baseline assessment to benchmark growth and identify areas of unmet need. We don't want a minute wasted on duplicating efforts.

Stakeholders are fashioning of a new field of patient input, which has already begun to take flight.

CREDIT: PAPER BOAT CREATIVE/GETTY IMAGES

WHO'S ON FIRST?

Through an environmental scan, we tracked more than 70 collaborative initiatives, clustered in six categories, that are further defining and shaping patient-centered practice and policy (Tables 1 and 2). Within these 70 initiatives, nearly 40 discrete supporting entities are assembling resources, providing direction, and tracking milestones. Each entity approaches this field from a different vantage point, which is what makes the efforts so promising: It is natural—and essential—that the work required to create the field of patient input be performed through strong collaborations composed of highly interactive, diverse organizations.

FORMING SOLID PLATFORMS: FRAMEWORKS AND MODELS

Some of the first formal efforts to outline the science of patient input borrow, from software development, the use of frameworks to provide a logical structure for organizing information, identifying sources of the information, and suggesting ways it might be used and viewed by distinct parties (*3*).

Frameworks serve different purposes, with varied approaches and audiences. It is important to be familiar with these frameworks because they lay the groundwork for much of the ongoing and future work in this space. The Clinical Trials Transformation Initiative (CTTI) created perhaps the most recognizable tool, and its work has become a guidepost. CTTI is a public-private partnership supported by the U.S. Food and Drug Administration (FDA) and member pharmaceutical companies and patient organizations and has popularized a visual chevron-based framework that identifies points at which clinical trial sponsors and regulators might engage patients along the R&D continuum for pharmaceuticals (*4*). A companion framework for medical devices was developed by another public-private partnership, the Medical Device Innovation Consortium (MDIC), which built detailed considerations into an FDA Center for Devices and Radiological Health (CDRH) diagram of places in the total product life cycle of medical devices at which patient-preference information might enhance product development (*5*).

The Patient-Centered Outcomes Research Institute (PCORI) requires that all its funded investigators partner with patients from the beginning of the application process through completion of the study and dissemination of its results. To guide formation of meaningful engagements with patients, PCORI developed a Patient Engagement Rubric (*6*) and a compensation framework (*7*) that now guide applicants, reviewers, and awardees at every step. The engagement principles outlined in the rubric—reciprocal relationships, colearning, partnership, trust, transparency, and honesty—have become the essential characteristics of patient-centeredness in R&D and health-care delivery. These initiatives, like most of the others identified here, use the U.S. regulatory system as a foundation. Composed of industry and patient groups, the Patient-Focused Medicines Development partnership is leading an effort to develop a comprehensive global framework for patient engagement.

Recently, we have seen a surge in frameworks being used by a number of organizations to help define the value of certain drugs and medical products for insurance coverage decisions. Frameworks assessing the value of medicines have been put forward by the American Society for Clinical Oncology, Institute for Clinical and Economic Review, National Comprehensive Cancer Network, and others; however, most efforts to date have

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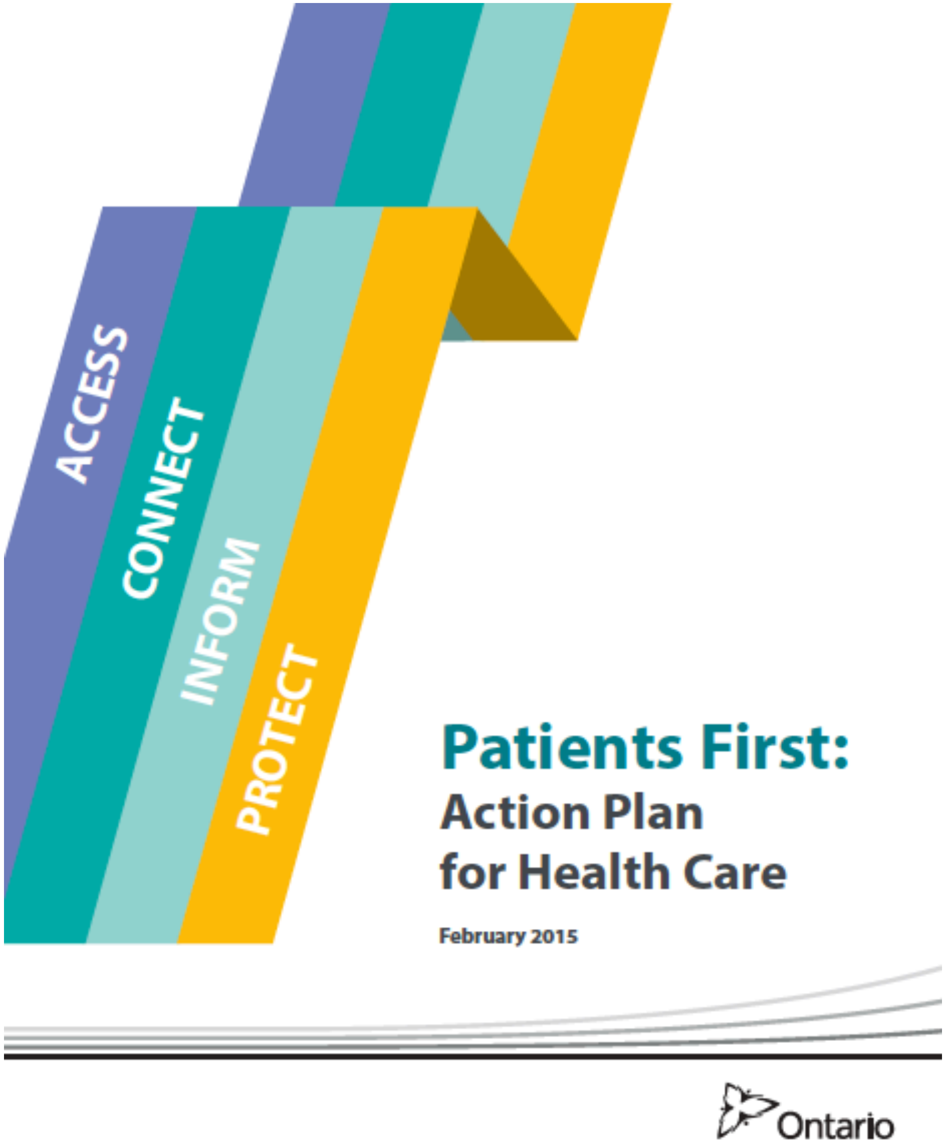
Relevant government initiatives



ACCESS
CONNECT
INFORM
PROTECT

Patients First:
Action Plan
for Health Care

February 2015

Ontario

1ST SESSION, 41ST LEGISLATURE, ONTARIO
65 ELIZABETH II, 2016

Bill 210

An Act to amend various Acts in the interest of patient-centred care

The Hon. E. Hoskins
Minister of Health and Long-Term Care

Government Bill

1st Reading	June 2, 2016
2nd Reading	
3rd Reading	
Royal Assent	

Printed by the Legislative Assembly
of Ontario

1re SESSION, 41e LÉGISLATURE, ONTARIO
65 ELIZABETH II, 2016

Projet de loi 210

Loi modifiant diverses lois dans l'intérêt des soins axés sur les patients

L'honorable E. Hoskins
Ministre de la Santé et des Soins de longue durée

Projet de loi du gouvernement

1re lecture	2 juin 2016
2e lecture	
3e lecture	
Sanction royale	

Imprimé par l'Assemblée législative
de l'Ontario

Lots of talk about engagement but what are we really talking about?

Clarifying key concepts

Who are we engaging or partnering with?

What do we mean by engagement and partnership?

What are our goals?

Different publics in…
different roles for…
different purposes

‘The public’: An evolving and contested term

- Citizens (lay person, voter, taxpayer)
- Service users (clients, patients, families)
- Communities (geographic, shared experiences)
- Advocates (family, service providers, organizations)
- Experts (clinical, scientific, lay/patient)?
- Elected officials?

Who SHOULD be engaged?
Who WANTS to be engaged?
Who IS engaged?

Who do you want to engage or partner with?

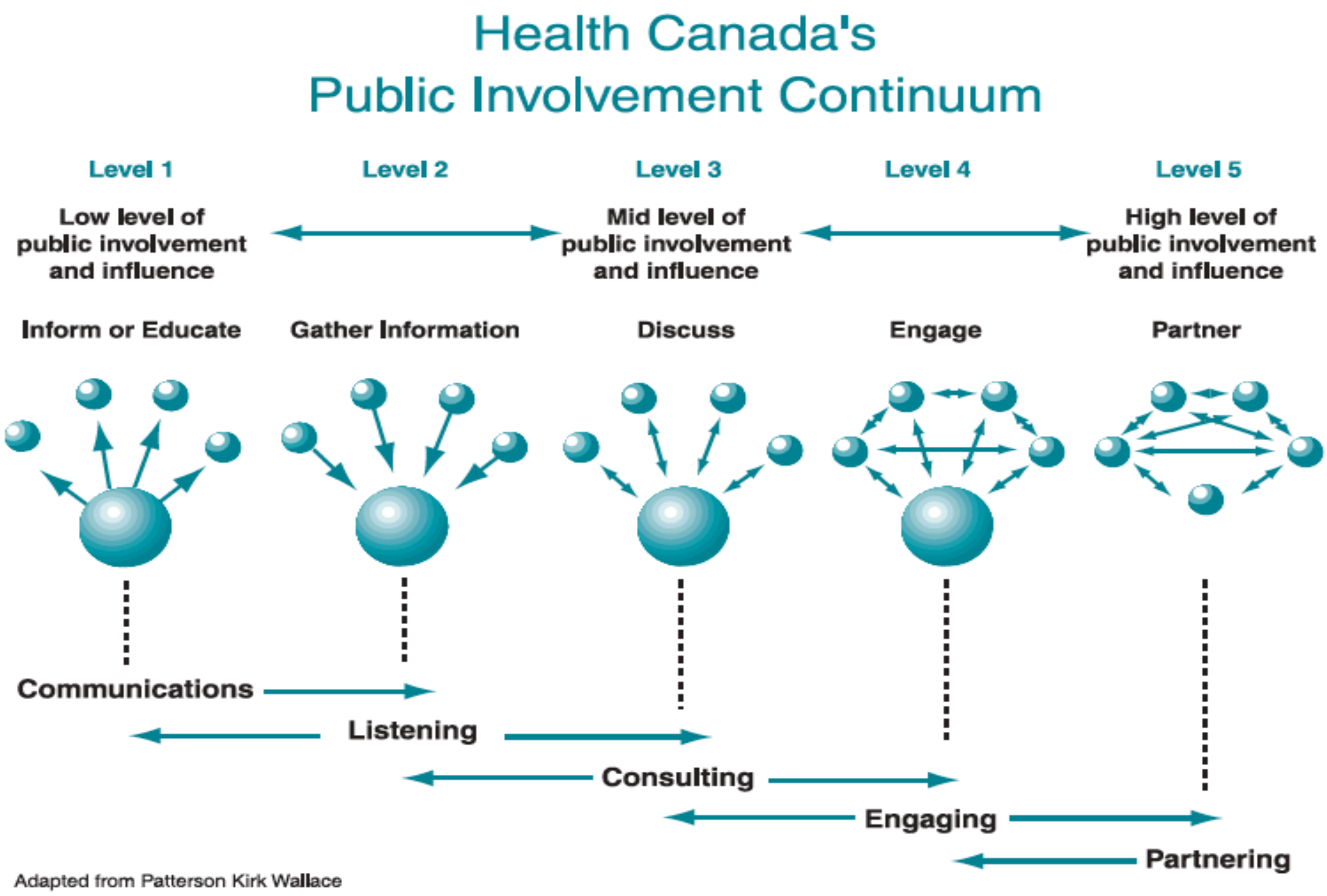
Those who are directly affected – Individuals with experiential knowledge about a particular aspect of care who can provide relevant perspectives (patients and families)

Broader publics – Individuals who can contribute broad social values but who may or may not have specific experience with the health system to draw from

Stakeholder groups – Groups with organized interests, related to funding and delivery arrangements (e.g., advocacy groups, industry, provider organizations)

(Abelson et al. 2016; Gauvin et al. 2014)

What do we mean by engagement and partnership?



Health Canada's Public Involvement Continuum

Level 1	Level 2	Level 3	Level 4	Level 5
Low level of public involvement and influence	← →	Mid level of public involvement and influence	← →	High level of public involvement and influence
Inform or Educate	Gather Information	Discuss	Engage	Partner

Communications →

← Listening →

← Consulting →

← Engaging →

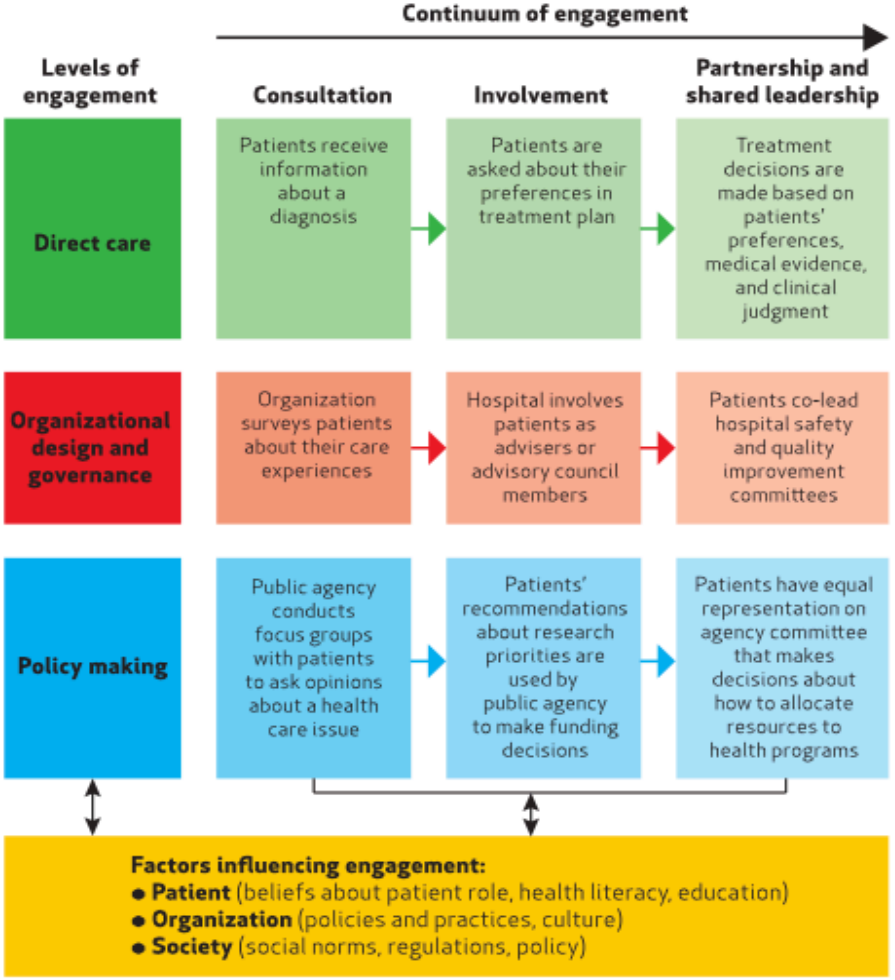
← Partnering

Adapted from Patterson Kirk Wallace

Health Canada. 2000. Policy toolkit for public involvement in decision making.

Carman K. et al. Patient And Family Engagement: A framework for understanding the elements and developing interventions and policies. *Health Affairs*, 32, no.2 (2013):223-231

A Multidimensional Framework For Patient And Family Engagement In Health And Health Care



Continuum of engagement

Levels of engagement	Consultation	Involvement	Partnership and shared leadership
Direct care	Patients receive information about a diagnosis	Patients are asked about their preferences in treatment plan	Treatment decisions are made based on patients' preferences, medical evidence, and clinical judgment
Organizational design and governance	Organization surveys patients about their care experiences	Hospital involves patients as advisers or advisory council members	Patients co-lead hospital safety and quality improvement committees
Policy making	Public agency conducts focus groups with patients to ask opinions about a health care issue	Patients' recommendations about research priorities are used by public agency to make funding decisions	Patients have equal representation on agency committee that makes decisions about how to allocate resources to health programs

Factors influencing engagement:
- **Patient** (beliefs about patient role, health literacy, education)
- **Organization** (policies and practices, culture)
- **Society** (social norms, regulations, policy)

SOURCE Authors' analysis. **NOTE** Movement to the right on the continuum of engagement denotes increasing patient participation and collaboration.

What are our goals for engagement or partnership?

Instrumental/quality goals (outcomes driven)

Better decisions, policies, programs, health research, health outcomes

Democratic/legitimacy goals (process driven)

Better decision making (e.g., more inclusive, legitimate, accountable)

Developmental goals (capacity driven)

Increased competency and capacity to contribute to individual and collective decision-making

Principle/ethics goals (rights driven)

Patient/family engagement as a right

Abelson et al. 2016. Public and Patient Involvement in HTA: Framework for Action. *IJTAH*C, 32:4.

Reviewing the Evidence

"… there is a striking imbalance between the amount of time, money and energy that governments in OECD countries invest in engaging citizens and civil society in public decision making and the amount of attention they pay to evaluating the effectiveness and impact of such efforts."

(OECD, 2005)

Key findings from reviews of patient engagement in health research

- More focus on doing than assessing
- Rich practice stories citing context-specific benefits (for specific populations, diseases/conditions, types of research)
- Weak evidence base about methods and impact
  - lack of conceptual clarity about WHO, HOW and WHY
  - small scale studies with limited follow up & few comparisons

Key findings (2)

Brett et al. 2012. Mapping the impact of patient and public involvement on health and social research: a systematic review*; Health Expectations*; 17:637-50

- **Beneficial impacts reported**
  - identification of user-relevant research topics and questions
  - improvements to recruitment, patient information materials and data collection tools
  - assistance with the interpretation of findings from user perspectives clinician and researcher perspectives)
  - strengthened dissemination and implementation of research findings

- **Challenging impacts reported**
  - perceived compromises and ethical dilemmas in the study design stage
  - tokenistic attitudes toward patient members of the team
  - recruitment challenges related to ‘hard-to-reach’ and marginalized groups)
  - time needed to support meaningful involvement of patient members (e.g., accessible material, adequate discussion time at meetings)

Findings (2)

Shen et al. 2016. How and why should we engage parents as co-researchers in health research? A scoping review of current practices. *Health Expectations*; DOI:10.1111/hex.12490

- 10 articles reviewed (low-moderate quality)
- Structural enablers: reimbursement and childcare
- Benefits cited
  - enhanced relevance of research to target population; maximize research participation; parent empowerment
- Challenges cited
  - resources required to support parent engagement
  - wide-ranging experiences
  - lack of role clarity and power differences

Evaluating PPE: the next wave?

Perspective

Evaluating patient and stakeholder engagement in research: moving from theory to practice

Journal of **Comparative Effectiveness Research**

Despite the growing demand for research that engages stakeholders, there is limited evidence in the literature to demonstrate its value – or return on investment. This gap indicates a general lack of evaluation of engagement activities. To adequately inform engagement activities, we need to further investigate the dividends of engaged research, and how to evaluate these effects. This paper synthesizes the literature on hypothesized impacts of engagement, shares what has been evaluated and identifies steps needed to reduce the gap between engagement's promises and the underlying evidence supporting its practice. This assessment provides explicit guidance for better alignment of engagement's promised benefits with evaluation efforts and identifies specific areas for development of evaluative measures and better reporting processes.

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Keywords: comparative effectiveness research • evaluation • patient engagement • patient-centered outcomes research • PCOR • review • stakeholder engagement

Key messages re evaluation

- To evaluate stakeholder partner engagement researchers should consider:
    - the need for *a priori* evaluative frameworks or criteria
    - use of predefined, validated tools
    - conducting evaluation at continuous or regular intervals through the engagement process
    - use of external evaluators where possible
    - documenting the context and process of engagement as fundamental components of the evaluation

Selected evaluation resources

- PCORI evaluation framework + researcher/patient questionnaires
- Patients Canada evaluation materials
- Public and Patient Engagement Evaluation Tool (PPEET)

PPEET

Public and Patient Engagement Evaluation Tool

- Systematic review of evaluation frameworks and tools underway (sponsored by SPOR SUPPORT units)

Engaging and partnering with patients and families – the basics

On-line resources (many and growing…)

- OCHSU and OSSU
  - Masterclass on patient-oriented research (for researchers, patient advisors, clinicians and policy makers) – last course in November 2017
  - Resource guide for research teams and networks
  - Patients Canada material

- CIHR training curriculum (available soon)

- Patient Oriented Research (POR) Curriculum For Children, Families And Clinician Scientists In Child Health (PORCCH) (NL Jones, The Hospital for Sick Children, Toronto) NEW!

Key areas

- Scope and level of engagement/partnership (*who, what, why*)
- Recruitment and selection
- Clarification and negotiation of roles
- Compensation
- Orientation and support for patient/family advisors *and* researchers

Involving patients/families as advisors

Types of activities by research stage

Setting priorities for research

- what research questions and outcomes are important to patients/families?

Proposal writing and applying for funding

- help with the development of the patient engagement/partnership strategy (relevant sections of the proposal)

Study implementation

- assistance with recruitment, training and support for research subjects

Analysis and interpretation of data

- what are patient/family members reflections on the data?

Dissemination and KT

- work within relevant networks to share the study results
- help with public-friendly versions of results

Recruitment and selection

- One of the most important but challenging areas

- Key considerations
  - who do you want to involve and how do you find them?
  - what perspectives, experiences, population or community characteristics do you want them to reflect or represent?

- Commonly used sources
  - market research firms and online panels
  - health charities and disease-specific organizations
  - health system organizations (patient and family advisory councils, patient partners and advisors)
  - other community resources

Clarifiying and negotiating roles

- As early as possible and check in periodically

- Clarify expectations for involvement in all stages of the research process

- Allow enough time for discussions about roles to show respect and commitment to meaningful involvement

Compensation

- Similar to advice about negotiating roles
  - initiate discussions early and in an open and respectful manner
- Don’t assume anything
- Expectations will vary

Resources:

Change Foundation – *Should money come into it?*

Forthcoming… CIHR SPOR and Ontario SPOR SUPPORT Unit documents

Orientation and support

- What do patients/family members need
  - a good understanding of the project, role and expectations
  - introductions to the team and various roles
  - respect for their roles and contributions

- What do researcher members need to do
  - invest early on in preparation of citizen/community members for their roles
  - peer support, mentoring, periodic check ins and clear, on-going communication

For more practical advice…

Take in the interactive panel (after the break)

THANK YOU

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