Advancing the science of patient engagement through evaluation

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Background

- long history of ‘doing research’ with lay individuals (in public health and social sciences research)
- more recent phenomenon in clinical and health services research communities (esp. in Canada and the U.S.)
- steady growth in the last 10-15 years due to targeted investments in UK, US and Canada
PATIENT ENGAGEMENT

On the path to a science of patient input

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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 its 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 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.

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:
FRAMES 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 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 its 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 give applicants, reviewers, and awardees at every step. The engagement principles outlined in the rubric—reciprocal relationships, co-learning, 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-Centered Outcomes Institute and 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
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.
Early learnings from a young field

- More focus on doing than assessing

- Rich practice stories citing context-specific benefits (for specific populations, diseases/conditions, types of research)

- Growing attempts to systematically collect and report on experiences to date (mostly descriptive)

- Weak evidence base about methods and impact of engagement on health research

- Difficult to assess “what works” and with “what impacts”
  - lack of conceptual clarity about WHO, HOW and WHY
  - small scale studies with limited follow up & few comparisons
Lots of talk about engagement and partnership but what are we really talking about?
‘Practical’ resources to support patient and family engagement in healthcare decisions: a scoping review

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Abstract

Background: Extensive literature exists on public involvement or engagement, but what actual tools or guides exist that are practical, tested and easy to use specifically for initiating and implementing patient and family engagement, is uncertain. No comprehensive review and synthesis of general international published or grey literature on this specific topic was found. A systematic scoping review of published and grey literature is, therefore, appropriate for searching through the vast general engagement literature to identify ‘patient/family engagement’ tools and guides applicable in health organization decision-making, such as within Alberta Health Services in Alberta, Canada. This latter organization requested this search and review to inform the contents of a patient engagement resource kit for patients, providers and leaders.

Methods: Search terms related to ‘patient engagement’, tools, guides, education and infrastructure or resources, were applied to published literature databases and grey literature search engines. Grey literature also included United States, Australia and Europe where most known public engagement practices exist, and Canada as the location for this study. Inclusion and exclusion criteria were set, and include: English documents referencing ‘patient engagement’ with specific criteria, and published between 1995 and 2011. For document analysis and synthesis, document analysis worksheets were used by three reviewers for the selected 224 published and 193 grey literature documents. Inter-rater reliability was ensured for the final reviews and syntheses of 76 published and 193 grey documents.

Results: Seven key themes emerged from the literature synthesis analysis, and were identified for patient, provider and/or leader groups. Articles/items within each theme were clustered under main topic areas of ‘tools’, ‘education’ and ‘infrastructure’. The synthesis and findings in the literature include 15 different terms and definitions for ‘patient engagement’, 17 different engagement models, numerous barriers and benefits, and 34 toolkits for various patient engagement and evaluation initiatives.

Conclusions: Patient engagement is very complex. This scoping review for patient/family engagement tools and guides is a good start for a resource inventory and can guide the content development of a patient engagement resource kit to be used by patients/families, healthcare providers and administrators.

Keywords: Patient engagement, Scoping literature review, Resource Kit, Engagement tools, Education, Infrastructure, Evaluation
Scoping review results

- 15 different terms and definitions for patient engagement
- 17 engagement models
- 34 toolkits for patient engagement & evaluation initiatives

Clarifying key concepts

What do we mean by patients and the public?

What do we mean by engagement & partnership?

What are our goals in the patient engagement and partnership enterprise?

Different publics in... different roles for... different purposes
Clarifying our goals

• Different goals and priorities for different constituencies
  – meeting funding accountability requirements
  – advancing PE methods
  – contributions to knowledge about effective PE (what works within which contexts)
  – demonstrating impact of PE on various outcomes
    ▪ quality of research
    ▪ applicability of research
    ▪ translation, dissemination and uptake of research
    ▪ legitimacy and credibility of research
    ▪ participants’ knowledge, trust and empowerment
Evaluating patient and stakeholder engagement in research: moving from theory to practice

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.

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

• 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 using external evaluators
  – documenting the context and process of engagement as fundamental components of the evaluation
Work in progress...

Patient and Public Engagement Evaluation Toolkit Project

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Project objectives

• To develop an evaluation toolkit for the Canadian research community:

  – to ensure the **integrity** between engagement principles and practice (are we just talking the talk or walking the walk?)
  – to **learn** from current practices (what are best practices and key success factors?)
  – to measure the **impacts** of engagement (what difference does it make?)
  – to demonstrate **accountability** for public investments (are we getting value for money?)
1. **Working definitions and key concepts** applicable to patient and public engagement evaluation

2. **Evaluation principles, goals and frameworks**

3. **Description of existing evaluation instruments** (e.g., self-administered questionnaires, interview guides)

4. **Recommendations for most appropriate use**
Potential uses

1. Formative evaluation, feedback, and improvement of patient and public engagement practices; and

2. Summative evaluations to assess the impacts of engagement on:
   • research partners (e.g., patients, researchers, clinicians, decision-makers, citizens)
   • changes in research processes (e.g., research priorities, study questions), and
   • health system transformation (e.g., use of research for governance and clinical improvement)
Selected sources

- **Public Involvement Impact Assessment Framework (PiiAF)**
  (University of Lancaster, Exeter & Liverpool, UK) – 2014
  (http://piiaf.org.uk)

  (http://www.pcori.org/research-results/evaluating-our-work/how-we-evaluate-key-aspects-our-work)

- **Public and Patient Engagement Evaluation Tool** (McMaster University) - 2015
  (http://fhs.mcmaster.ca/publicandpatientengagement/ppeet.html)
THANK YOU

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