Patient Engagement in Health Outcomes Research: A Snapshot from the ISPOR Community

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KEY POINTS . . .

Current reported practice of patient involvement in the outcomes research process highlights a lack of bi-directional collaboration, with a potential for missed opportunities.

Findings highlight the lack of consistent methodology for patient engagement and the opportunities to develop a framework to systematically operationalize patient engagement in outcomes research.

The working group is endeavoring to generate recommendations on how to determine the best way to involve patients and their representatives in all stages of research and ensure the effective participation of patients' organizations in the creation and development of tools to measure outcomes.

Introduction

The goal of the ISPOR Patient Centered Special Interest Group is to determine how best to involve patients and their representatives in all stages of decision making, for the purpose of improving health care delivery and outcomes. Within the Special Interest Group, the aim of the Patient Engagement in Research Working Group is to define "patient engagement," and to recommend a framework for successful engagement of patients, health care consumers, and their representatives in the realm of outcomes research and decision making. Toward these goals, a targeted literature review and informal survey of ISPOR members were conducted to generate preliminary data.

The goal of this article is to share the results of this preliminary work, to stimulate further discussions among ISPOR membership and broader stakeholder communities about patient engagement in outcomes research, and to encourage the engagement of patients in outcomes research initiatives.

Targeted Literature Review: Existing Frameworks for Patient Engagement in Research

The working group conducted a targeted literature review to identify existing theoretical frameworks and definitions of patient engagement in health research. Though the review was not all-encompassing, both published and gray literature were examined, representing perspectives ranging from academia, industry, and government, to organizations representing patients. A number of fundamental, consistent conceptual themes of patient engagement were identified [1–5]. Several frameworks described varying levels or intensities of patient engagement in health care and health research [3, 6]. In reviewing these frameworks, the working group identified three frameworks: 1) Carman et al.; 2) Domecq et al.; and 3) Guise et al., which they found to be helpful in describing patient involvement through the flow of information; the stages of research in which patient engagement occurs; and prioritizing stakeholder engagement in research. The model proposed by Carman and colleagues is bi-dimensional and describes a continuum of engagement characterized by the flow of information between the patient and provider (consultation, involvement, partnership and shared leadership) involvement in direct care, organizational design and governance, and policy making [3]. This hierarchical approach to levels of engagement is also reflected in frameworks created by patient advocacy organizations [6].

Stakeholder engagement has also been examined by "stage" of research (from early development through to dissemination), which can be broadly grouped as preparation, execution and translation [7]. Reviews by Domecq and Guise have examined current practices for engaging stakeholders in research, with Guise particularly focusing on the role of patient engagement in research prioritization [7-8]. Results revealed that patient engagement was reported most often in the early stages of research, particularly in the agenda setting, study design, and recruitment stages. The most consistently and frequently reported engagement activities were interviews, focus groups, and surveys. This suggests that current practice is primarily a one-way communication (consultation) with patients providing information and receiving minimal feedback from the researcher. This lack of bi-directional engagement in current practice is supported by a review of the literature by Domecq and colleagues, who found more collaborative forms of engagement such as deliberation and organizational participation were less frequent across all stages of research [7].

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Finally, the working group's review also identified a number of key gaps in evidence, including lack of consistency in the structure of engagement and in its reporting. There is insufficient empirical evidence measuring the impact and success of engagement; no standard method for doing so exists, though a checklist for reporting results of engagement has been proposed [8]. The literature review concluded that there is little structured design or analytical methodology existing around patient engagement.

Survey of ISPOR Membership: Current Perspectives on Patient Engagement in Research

An informal survey on patient engagement was conducted with attendees at two ISPOR conferences: the 19th Annual International Meeting (Montreal, QC, Canada) and the 17th Annual European Congress (Amsterdam, The Netherlands). A total of 39 semi-structured interviews were conducted with a convenience sample (21 in Montreal, 18 in Amsterdam). Working group members used an interview guide developed for the study. The questionnaire included 29 questions, soliciting both closed and open-ended responses, reflecting the following dimensions:

- Definition of patient engagement
- Personal experience with patient engagement, including engagement in:
    - o Data collection
    - o Study design
    - o Interpretation and dissemination of results
    - o Ethics and/or regulatory approval process
- Awareness of patient engagement initiatives at current organization, including:
    - o Engagement objectives and methods
    - o Responsible function(s)
    - o Respondent's own views about existing patient engagement activities
    - o Barriers to engagement
    - o Opportunities and value of engagement
- Views on future directions of patient engagement in organization

Demographic and research setting descriptors with anonymized responses were also collected. Results were analyzed in terms of levels of patient engagement, and by the phases of implementation along the continuum of health outcomes research activities, based on Carman's framework [3]. These results have been presented at two ISPOR conferences [9-10].

Survey participants represented perspectives from industry (56.4%), health research (17.9%), academia (17.9%), managed care (5.1%), government (2.6%), and clinical practice (2.6%). This distribution was generally representative of the overall ISPOR membership, with some perspectives under-represented (government, health care communications). These results are shown in Table 1. It should be noted that certain fields which may have a significant role in patient engagement, e.g. clinical practice, are not well represented in this sample (nor in the ISPOR membership); this should be taken into account when interpreting and extrapolating from the survey results.

Table 1. Primary work areas of ISPOR informal survey respondents

Primary Work Areas	PERWG Survey (N=39)	ISPOR Membership (N=7443)
Industry	22 (56.4%)	3385 (45.5%)
Health Research	7 (17.9%)	1871 (25.1%)
Health Care Communications	0 (0.0%)	334 (4.5%)
Academia	7 (17.9%)	1101 (14.8%)
Managed Care	2 (5.1%)	116 (1.6%)
Government	1 (2.6%)	502 (6.7%)
Clinical Practice	1 (2.6%)	134 (1.8%)

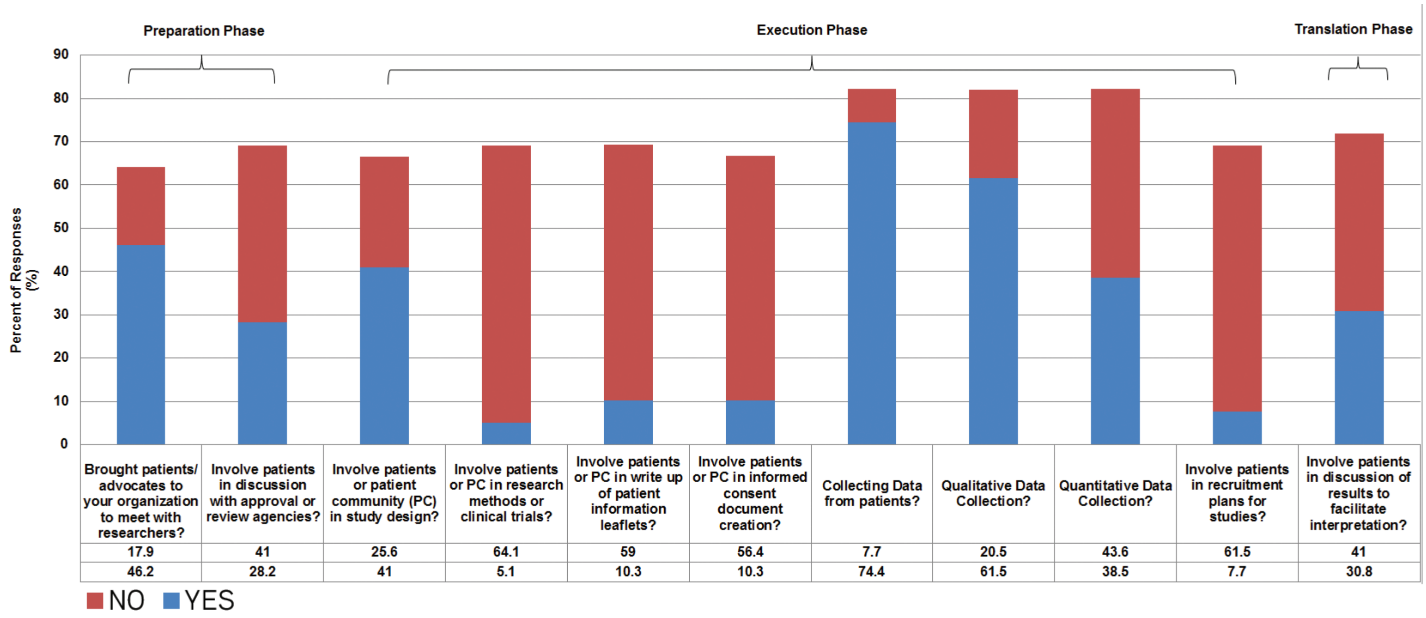
Abbreviation: PERWG indicates Patient Engagement in Research Working Group

Survey results showed that 84% of participants were aware of some form of patient engagement activities within their organization (data not shown). Participants had limited knowledge about the exact nature of the types of engagement, or hesitated to define patient engagement, with multiple individuals noting that 'this was handled by other groups' within their organization (e.g. patient advocacy/communications groups). When asked to describe patient engagement activities and initiatives, respondents mentioned examples ranging across levels of intensity. Less intensive forms of engagement included 'passive involvement' of patients as research participants in clinical trials, discussing medical needs, consulting on the focus of research, and sharing experiences in clinical trials to discuss ways to enhance recruitment and retention. More intensive forms included patients and patient advocates being co-investigators, actively driving and shaping the research agenda as partners, and leading and funding clinical trials and research. Similar to findings from the literature review, most of the engagement examples mentioned by participants reflected one-way communication from patient to researcher in research organized by the researcher [7-8]. Figure 1 shows results of the survey stratified by phase of research for patient engagement proposed by Domecq et al. [7].

Results of the survey aligned with findings from the literature review, particularly with regard to the lack of consistent methodology for patient engagement; no respondents mentioned a specific framework or a standard checklist. However, there was a very strong interest in moving forward and finding ways to systematically operationalize patient engagement in outcomes research.

To put results in context, examples of patient engagement in research mentioned in survey responses are summarized below on a continuum of patient engagement (adapted from Carman), with engagement ranging from consultation (mostly one-way communication) at the basic level, involvement as a middle ground, and partnership and shared leadership being the most intense form of engagement [3]. An example in the consultation level was, "just listening to patient concerns, the impact of the condition, how they're coping, and keeping them interested in the clinical trial." At the involvement level, "part of adherence means patients are involved in their care and are willing participants in finding the best treatment." When asked about personal experience with patient engagement in research, a majority of respondents (66.7%) noted they had engaged patients in their research. However, when probed for specifics, responses focused on patients as research participants, and pointed towards patient involvement in early phases of research (particularly in early clinical research in drug development). One example of patient involvement in early stage research involved "interviews with psoriasis patients to

Figure 1: Survey results on personal experience and patient engagement phases (N=39)



	Brought patients/ advocates to your organization to meet with researchers?	Involve patients in discussion with approval or review agencies?	Involve patients or patient community (PC) in study design?	Involve patients or PC in research methods or clinical trials?	Involve patients or PC in write up of patient information leaflets?	Involve patients or PC in informed consent document creation?	Collecting Data from patients?	Qualitative Data Collection?	Quantitative Data Collection?	Involve patients in recruitment plans for studies?	Involve patients in discussion of results to facilitate interpretation?
🟥 NO	17.9	41	25.6	64.1	59	56.4	7.7	20.5	43.6	61.5	41
🟦 YES	46.2	28.2	41	5.1	10.3	10.3	74.4	61.5	38.5	7.7	30.8

identify unmet needs." In this case, patient involvement was particularly impactful as it highlighted incorrect assumptions on the part of researchers about which endpoints were most important to patients (initial assumptions were based on general opinion or literature), leading to a change in study endpoints.

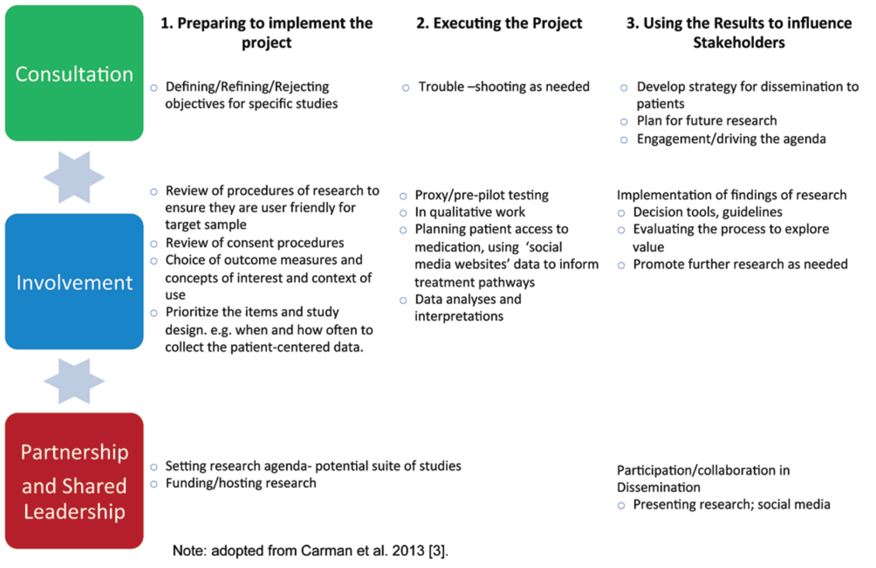
In spite of the lack of clarity in how to operationalize patient engagement in health outcomes research, examples were identified that illustrate opportunities worthy of consideration by the research community. Figure 2 summarizes these potential opportunities in context of Carman's framework.

Future Perspectives for the ISPOR Patient Engagement in Research Working Group

The working group's goal in executing the targeted literature review and informal survey was to better understand the landscape of patient engagement, particularly with regard to current activity and perspectives for the ISPOR member's activities involving patient engagement. Though the review was narrowly focused and the survey sample was small, the findings highlight how we can begin to provide some answers for stakeholders. There are opportunities for engaging, consulting, and involving patients within many different stages of health outcomes research. Within project preparation, involvement may include discussing project objectives with patients or consumers. Gaining this perspective early can be useful in shaping which aspects to emphasize, or helping with priority setting. In project execution, this may include involving patients in the study design process to ensure and optimal approach. Patient input into study design can include consideration of lower participant burden, collection of data within focus groups, development of interview guides for concept elicitation, and pilot testing of instruments which measure patient experience. Improvements in execution time and energy can be enabled by sharing:

- Details of cognitive interviews with patient advocates
- Patient's pre-read of protocols prior to project initiation
- Research agenda setting
- Determining funding priorities
- Leading or sponsoring research activities

Figure 2.: Identified opportunities for patient engagement in outcomes research



	1. Preparing to implement the project	2. Executing the Project	3. Using the Results to influence Stakeholders
Consultation	○ Defining/Refining/Rejecting objectives for specific studies	○ Trouble –shooting as needed	○ Develop strategy for dissemination to patients ○ Plan for future research ○ Engagement/driving the agenda
Involvement	○ Review of procedures of research to ensure they are user friendly for target sample ○ Review of consent procedures ○ Choice of outcome measures and concepts of interest and context of use ○ Prioritize the items and study design. e.g. when and how often to collect the patient-centered data.	○ Proxy/pre-pilot testing ○ In qualitative work ○ Planning patient access to medication, using 'social media websites' data to inform treatment pathways ○ Data analyses and interpretations	Implementation of findings of research ○ Decision tools, guidelines ○ Evaluating the process to explore value ○ Promote further research as needed
Partnership and Shared Leadership	○ Setting research agenda- potential suite of studies ○ Funding/hosting research		Participation/collaboration in Dissemination ○ Presenting research; social media

Note: adopted from Carman et al. 2013 [3].

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Patient and researcher alignment toward shared goals provide opportunities to increase project efficiency.

The ISPOR Patient Engagement in Research Working Group recognizes that patient engagement in the context of outcomes research exists along a continuum; starting from activities which involve low levels of intensity (consultation) and moving toward higher levels of intensity (partnership and shared leadership). Timing of engagement is also a factor, with experience suggesting that early engagement may lead to more successful collaborations. When undertaken in the right spirit, this can bring important new insights and in many ways can be regarded as the ultimate "reality check" on the project in hand, which should repay its (relatively small) cost many times over in increased efficiency and can even prove critical to the success of a project. However, if it is skimped or undertaken in a spirit of tokenism the reverse will often be the result.

> It's an exciting time for outcomes researchers, at the threshold of more meaningful engagement with patients, both as partners and co-leaders in research.

Currently, the working group is conducting a systematic literature review to define "patient engagement" for the ISPOR community and its varied stakeholders. The group is endeavoring to generate recommendations on how to determine the best way to involve patients and their representatives in all stages of research (from providing opinions to making decisions), and to ensure the effective participation of patients' organizations in the creation and development of tools to measure outcomes.

Call to Action

Across different settings researchers face challenges to bringing the patient's perspective into drug development and health outcomes research. There is great value however, in understanding this transformational change toward greater patient engagement. It's an exciting time for outcomes researchers, at the threshold of more meaningful engagement with patients, both as partners and co-leaders in research. The working group welcomes ideas and suggestions from the ISPOR community as it works towards developing best practices to promote consistency, and advance the field of outcomes research. The need for further development of a framework and definition of terms within ISPOR to facilitate patient successful engagement has been identified as critical in the groups' preliminary work. Fostering stakeholders' development of necessary skills and continuous awareness of emerging good practices for patient engagement within the outcomes research community are some of the efforts that can supplement current efforts ongoing within ISPOR. Through collaborative efforts we can demonstrate the value inherent in patient engagement and define new methods to enable its measurement. Much work remains and help from all interested stakeholders is needed to improve clarity in capturing stakeholder perspectives and to identify gaps and future needs for research in this area.

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Additional information:

The following article is based on a workshop, "Patient Engagement in Outcomes Research: Current Status, Questions, Beliefs, and Future Perspectives", presented at the ISPOR 17th Annual European Congress in Amsterdam, The Netherlands. The Patient Centered Special Interest Group was formed after the first ISPOR Patient Representative Roundtable met in Dublin and requested a multi-stakeholder group including patient representatives be developed to address issues to engage patients in the research process.

To view the presentation, go to: http://www.ispor.org/sig/PatientCentered/WorkshopPresentation-2014Amsterdam.pdf

To learn more about the ISPOR Patient Centered Special Interest Group, go to: http://www.ispor.org/sigs/PatientCentered/PC_EngagementInResearch.aspx