

Patient-Oriented Research and Patient engagement in clinical trials: an introduction

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Hospital**
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About us

- Stuart Nicholls

- Social scientist and health services researcher
- PhD in Social Statistics
- Strategy for Patient Oriented Research (SPOR) Program facilitator (Ottawa Methods Centre)

- Alicia Hilderley

- Patient at TOH Cancer Centre
- Research partner
- Clinical trial participant
- Postdoctoral research fellow (PhD in Rehabilitation Sciences)

Learning objectives

At the end of this session, you should:

- Be able to describe patient-oriented research (POR) and patient engagement (PE)
- Understand the foundational principles behind POR and PE
- Be able to examine clinical trials with respect to their degree of pragmatism and patient-centredness

Patient-Oriented Research

A decorative graphic on the right side of the slide. It consists of several concentric circles of varying radii, centered around the middle of the slide. Scattered around these circles are numerous small, light blue plus signs (+). The circles and plus signs are semi-transparent, allowing the dark blue background to show through.

Patient engagement is here



Strategy for Patient-Oriented Research

SPOR

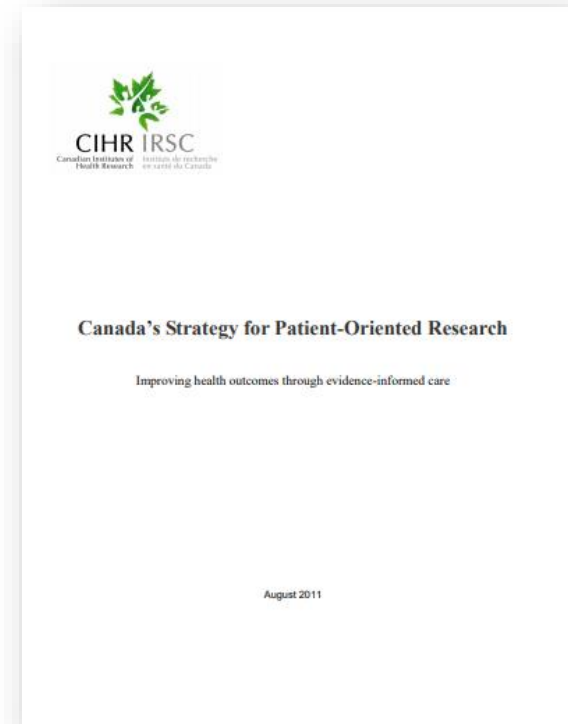
Putting Patients First 

INVOLVE



Why patient-oriented research?

- Two major concerns:
 - Investment in research does not always reflect priorities important to Canadians
 - Limited accountability and evaluation of the relevance/importance of research programs



Strategy for Patient-Oriented Research

- Created a framework in 2014
- Defines POR and PE
- Set out guiding principles



Patient-Oriented Research

“A continuum of research that engages patients as partners, focusses on **patient-identified priorities** and improves patient outcomes. This research, conducted by multidisciplinary teams in partnership with relevant stakeholders, aims to apply the knowledge generated to improve the healthcare systems and practices” *Canadian Institutes of Health Research*

<https://cihr-irsc.gc.ca/e/48413.html>

Strategy for Patient-Oriented Research

SPOR

Putting Patients First 

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Strategy for Patient-Oriented Research

SPOR

Putting Patients First 

Patient engagement in SPOR

- “A central tenet of SPOR is to put patients first—meaning that the patients themselves (and their families) are at the centre of the discussion on health. **The idea is for patients, researchers, health care providers and decision makers to actively collaborate to build a sustainable, accessible, and equitable health care system to bring about positive changes in the health of people living in Canada.** Engaging patients is thus an integral component in the development and implementation of all elements of SPOR [...].”

<https://cihr-irsc.gc.ca/e/45851.html>

Who counts as a patient?

- “An overarching term inclusive of individuals with personal experience of a health issue and informal caregivers, including family and friends.”

SPOR Patient Engagement Framework, 2014

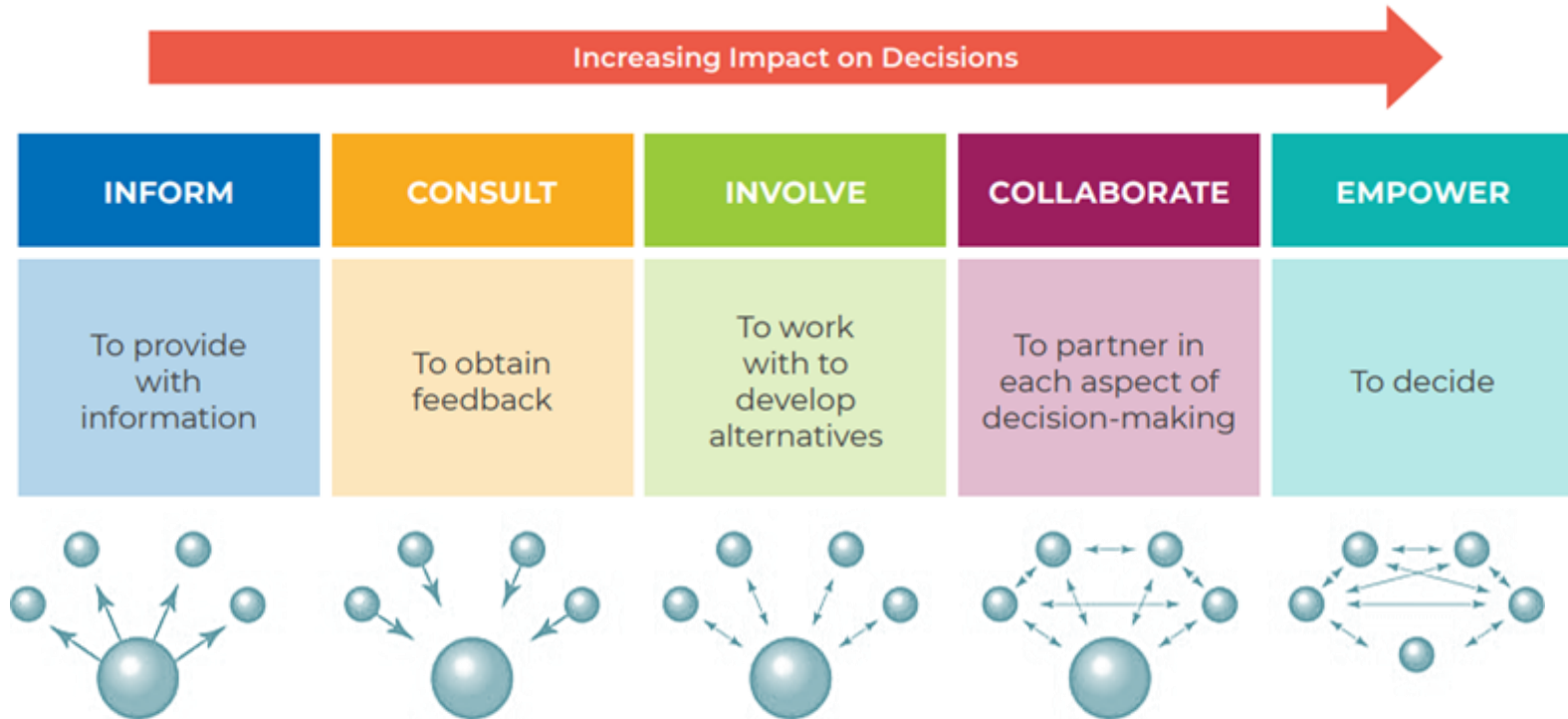
- Depending on the question, other stakeholders might be
 - Service providers
 - Policy makers
 - Members of the public



Guiding principles

- **Inclusiveness:** Patient engagement in research integrates a diversity of patient perspectives and research is reflective of their contribution – i.e., patients are bringing their lives into this.
- **Support:** Adequate support and flexibility are provided to patient participants to ensure that they can contribute fully to discussions and decisions. This implies creating safe environments that promote honest interactions, cultural competence, training, and education. Support also implies financial compensation for their involvement.
- **Mutual Respect:** Researchers, practitioners and patients acknowledge and value each other's expertise and experiential knowledge.
- **Co-Build:** Patients, researchers and practitioners work together from the beginning to identify problems and gaps, set priorities for research and work together to produce and implement solutions.

Frameworks of engagement



A patient partner perspective

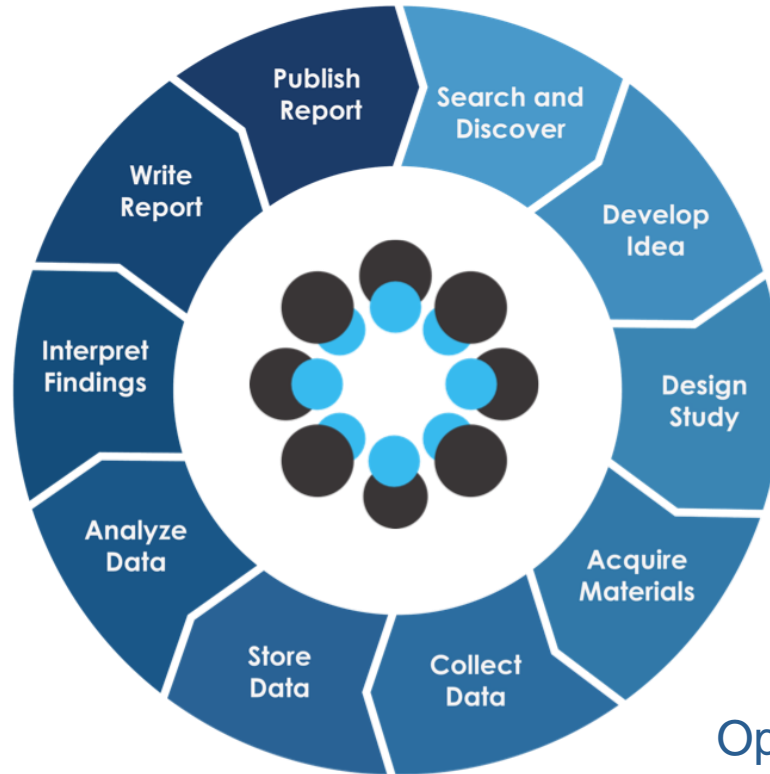
A decorative graphic on the right side of the slide. It consists of several concentric circles of varying line weights. Scattered around and on these circles are numerous small, light blue plus signs (+). The overall effect is a subtle, geometric pattern that suggests a network or a system.

Alicia slides here/Alicia talks here

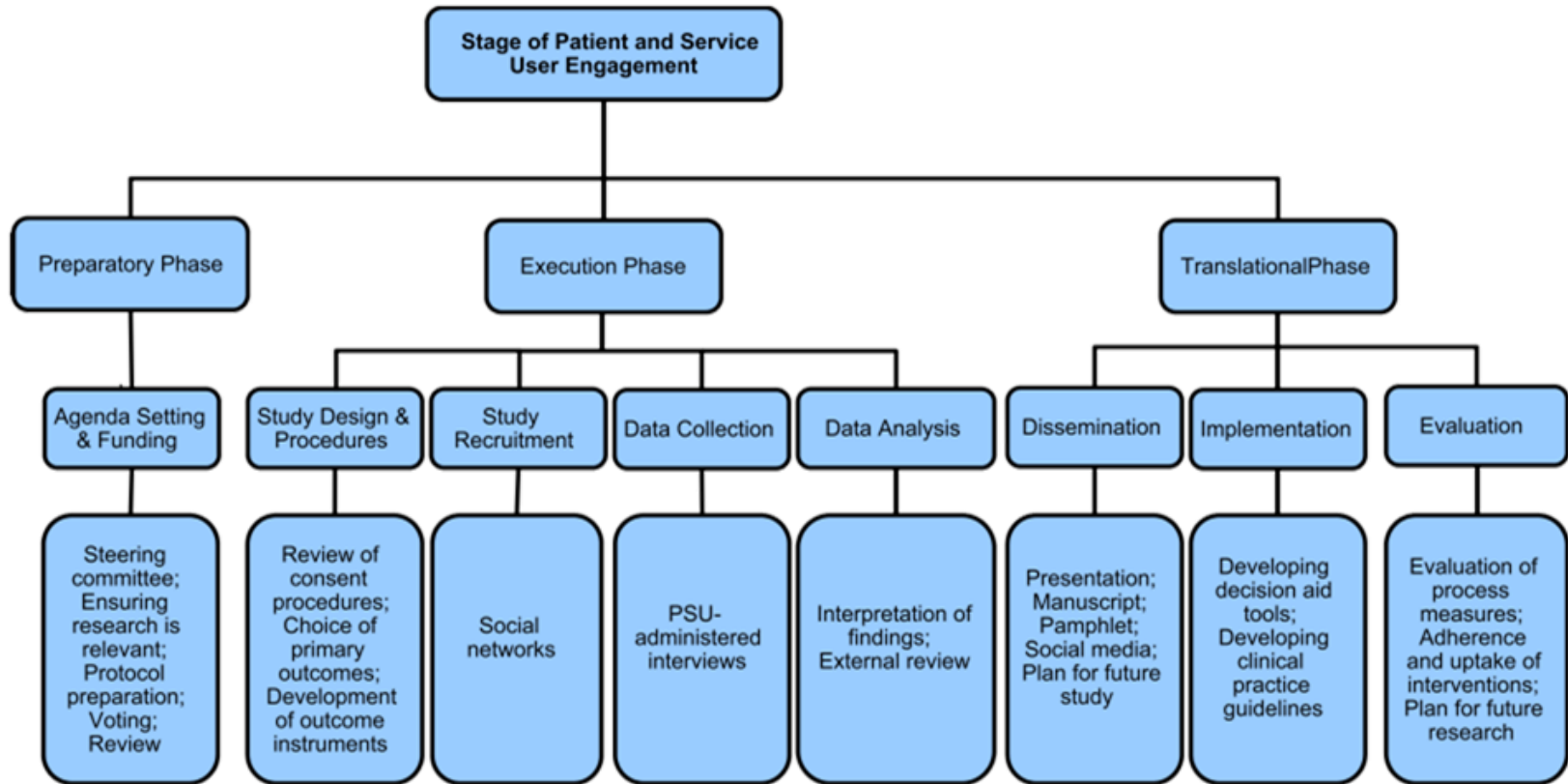
How to engage patients in research?

The background of the slide is a solid dark blue. On the right side, there is a decorative graphic consisting of several concentric circles of varying radii. Scattered around these circles are numerous small, light blue plus signs (+). The overall aesthetic is clean and professional, typical of a presentation slide.

The research cycle



Open Science Framework



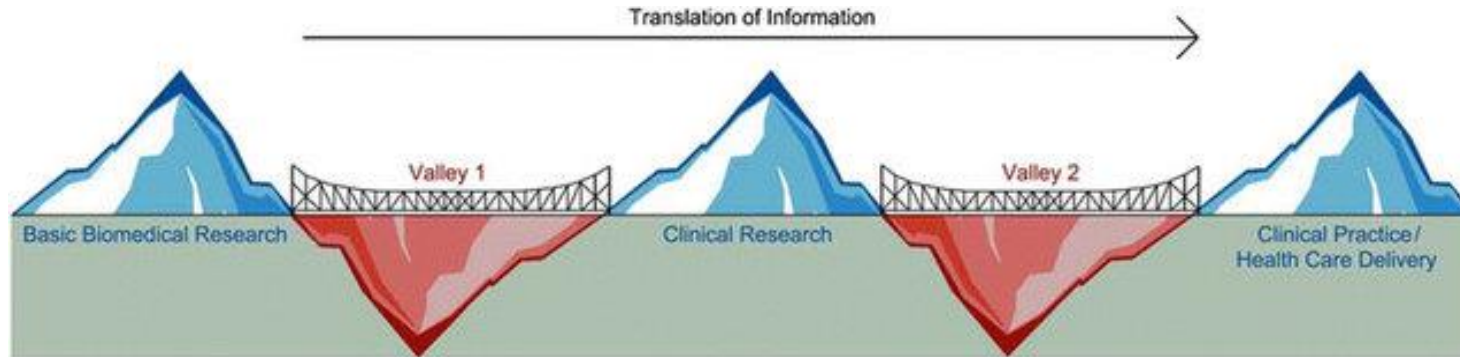
Shippee et al., (2015). Patient and service user engagement in research: a systematic review and synthesized framework. *Health Expect*, 18(5), 1151-1166. doi:10.1111/hex.12090

Patient-oriented clinical trials

- “A significant disconnect seems to continue to exist with respect to the prevalent focus of clinical trials on drugs as opposed to other forms of intervention.”
- “The concern is that clinical trials focused on particular medical endpoints and not on the patient experience with drugs and devices might result in an efficacious drug being designed that brings with it a quality of life or personal cost too great to warrant the use of the drug.” <https://cihr-irsc.gc.ca/e/44000.html>

Patient-oriented clinical trials

- Clinical trials can be slow and expensive
- Implementation in practice can significantly lag behind trials*
- Are the results of ‘traditional’ trials applicable to everyday practice? §



* Morris ZS, Wooding S, Grant J. *Journal of the Royal Society of Medicine*. 2011;104:510-20.

§ Feinstein AR, Horwitz RI. *American Journal of Medicine*. 1997;103:529-35.

Examples of patient engagement in clinical trials

- 2777 articles at full text review
- 23 trials: <0.001% of trials conducted in that period (371,159)
- # patients engaged: 2-24
- The majority of trials engaged patients in the development or refinement of the study intervention.

Fergusson et al. *Research Involvement and Engagement* (2018) 4:17
<https://doi.org/10.1186/s40900-018-0099-x>

Research Involvement
and Engagement

RESEARCH ARTICLE

Open Access



The prevalence of patient engagement in published trials: a systematic review

Dean Fergusson^{1,2*}, Zarah Monfaredi³, Kusala Pussegoda¹, Chantelle Garritty¹, Anne Lyddiatt², Beverley Shea¹, Lisa Duffett³, Mona Ghannad⁴, Joshua Montroy¹, M. Hassan Murad⁵, Misty Pratt¹, Tamara Rader⁶, Risa Shorr⁷ and Fatemeh Yazdi¹

Plain English summary

With the growing movement to engage patients in research, questions are being asked about who is engaging patients and how they are being engaged. Internationally, research groups are supporting and funding patient-oriented research studies that engage patients in the identification of research priorities and the design, conduct and uptake of research. As we move forward, we need to know what meaningful patient engagement looks like, how it benefits research and clinical practice, and what are the barriers to patient engagement? We conducted a review of the published literature looking for trials that report engaging patients in the research. We included both randomized controlled trials and non-randomized comparative trials. We looked at these trials for important study characteristics, including how patients were engaged, to better understand the practices used in trials. Importantly, we also discuss the number of trials reporting patient engagement practices relative to all published trials. We found that very few trials report any patient engagement activities even though it is widely supported by many major funding organizations. The findings of our work will advance patient-oriented research by showing how patients can be engaged and by stressing that patient engagement practices need to be better reported.

Abstract

Background: Patient-Oriented Research (POR) is research informed by patients and is centred on what is of importance to them. A fundamental component of POR is that patients are included as an integral part of the research process from conception to dissemination and implementation, and by extension, across the research continuum from basic research to pragmatic trials [J Comp Eff Res 2012; 1:181–94, JAMA 2012; 307:1587–8]. Since POR's inception, questions have been raised as to how best to achieve this goal. We conducted a systematic review of randomized controlled trials and non-randomized comparative trials that report engaging patients in their research. Our main goal was to describe the characteristics of published trials engaging patients in research, and to identify the extent of patient engagement activities reported in these trials.

Methods: The MEDLINE®, EMBASE®, Cinahl, PsycINFO, Cochrane Methodology Registry, and Pubmed were searched from May 2011 to June 16th, 2016. Title, abstract and full text screening of all reports were conducted independently by two reviewers. Data were extracted from included trials by one reviewer and verified by a second. All trials that report patient engagement for the purposes of research were included.

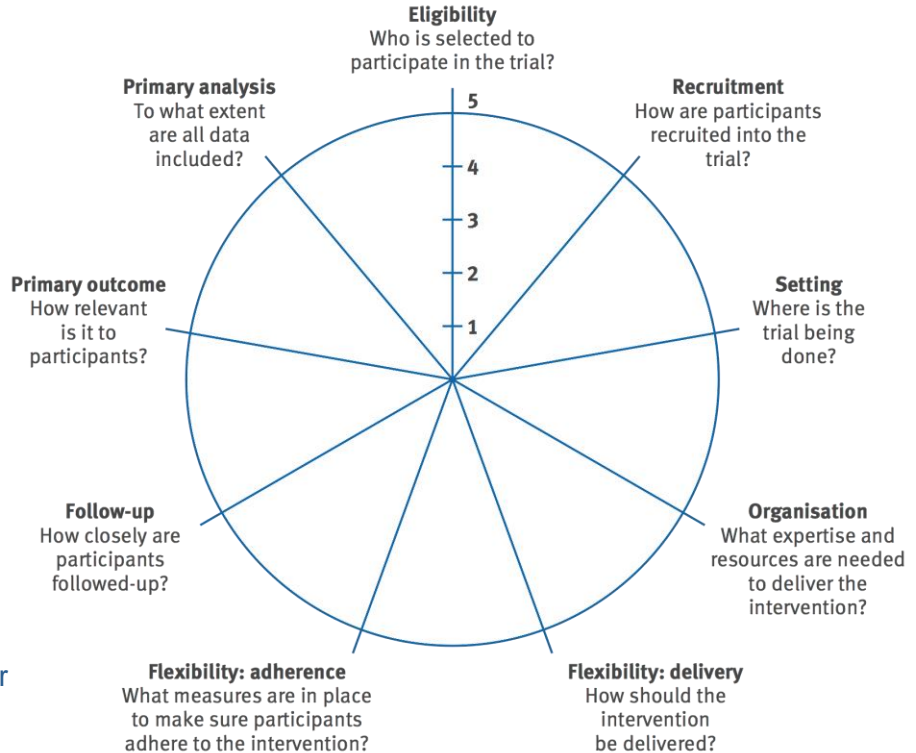
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Pragmatic trials

- **Randomized controlled trials (RCTs)** are a commonly used experimental research design for generating robust evidence regarding benefits and harms of health interventions.
- **Pragmatic RCTs** *are intended to have their results be applicable to clinical or health policy decisions* and should thus mimic as closely as possible the users, settings and circumstances in which it is thought the interventions under evaluation will be used
- **Explanatory RCTs** *aim to generate an understanding of the mechanism of action for the intervention.* Their design should focus on tightly controlling aspects of delivery and the study environment.

PRagmatic Explanatory Continuum Indicator Summary (PRECIS-2)

- 1. Very explanatory
- 2. Rather explanatory
- 3. Equally pragmatic/explanatory
- 4. Rather pragmatic
- 5. Very pragmatic



Loudon K, et al The PRECIS-2 tool: designing trials that are fit for purpose. *BMJ*. 2015;350:h2147

	Explanatory attitude	Pragmatic attitude
Question	Efficacy: can the intervention work?	Effectiveness: does the intervention work when used in normal practice?
Setting	Well resourced, 'ideal' setting	Normal practice
Participants	Highly selected; poorly adherent participants and those with conditions which might dilute the effect are often excluded	Little or no selection beyond the clinical indication of interest
Intervention	Strictly enforced and adherence is monitored closely	Applied flexibly as it would be in normal practice
Outcomes	Often short-term surrogates, or process measures	Directly relevant to participants, funders, communities and healthcare practitioners
Relevance to practice	Indirect: little effort is made to match the design of the trial to the decision making needs of those in the usual setting in which the intervention will be implemented	Direct: the trial is designed to meet the needs of those making decisions about treatment options in the setting in which the intervention will be implemented

Summary

- Patient-Oriented Research is a continuum of research that engages patients as partners, focusses on **patient-identified priorities** and improves patient outcomes.
- Patient engagement involves collaboration with patients, families, and caregivers in this process.
- Key principles are: Inclusiveness, Support, Mutual Respect, & Co-building

Summary

- Patient engagement can occur across the research lifecycle
- The PRECIS-2 framework can help think about the patient-oriented and pragmatic nature of a clinical trial design

Office for Patient Engagement in Research Activities (OPERA)

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AA DISPLAY OPTIONS

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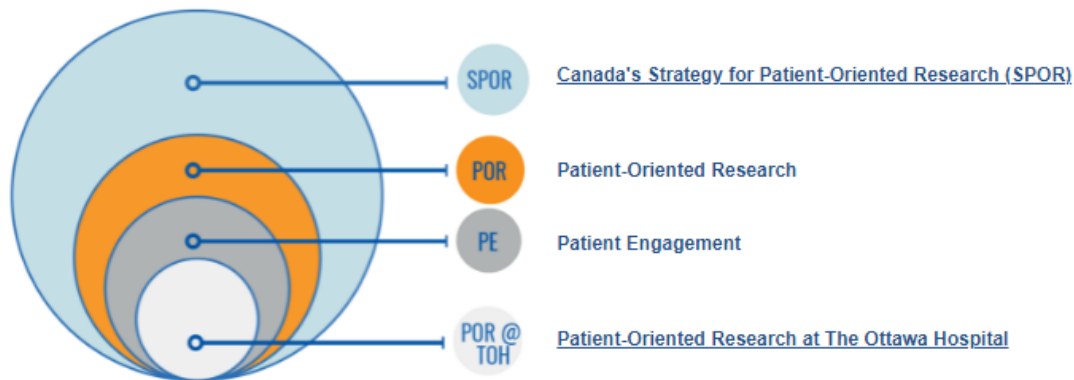
THE TEAM

SERVICE REQUEST

ASK A QUESTION

Patient-Oriented Research and Patient Engagement

Patient-oriented research (POR) is about putting patients first – research that is informed by patients and focuses on what is important to patients. It produces information for decision makers and health care providers that will improve health care practices, therapies and policies. It ensures that new and innovative diagnostic and therapeutic approaches are applied where they are needed (CIHR 2014).



<http://www.ohri.ca/opera>

Help and SUPPORT

SUPPORT Units

Most SUPPORT Units have their own websites, which are listed below. Please visit the website of your SUPPORT Unit of interest in order to learn more about their activities and offerings.

Alberta

Tim Murphy
tim.murphy@albertainnovates.ca

British Columbia

Minnie Downey
mdowney@bcsupportunit.ca

Manitoba

Carly Leggett
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Maritimes

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Marina.Hamilton@nshealth.ca

Newfoundland and Labrador

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Dr. Stephanie Irlbacher-Fox
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Nunavut

Amy Caughey
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Ontario

Dr. Vasanthi Srinivasan
VasanthiSrinivasan@ossu.ca

Quebec (French only)

Dr. Antoine Groulx
antoine.groulx@usherbrooke.ca

Saskatchewan

Dr. Malcolm King
Malcolm.king@usask.ca

Yukon

Dr. Liris Smith
lsmith@yukonu.ca

<https://cihr-irsc.gc.ca/e/45859.html>

Thank you

Questions?

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Methods of Engagement

Instructions: Patient engagement is about meaningful engagement of patients/public in the research process (not just as subjects of research). This table provides methodological options that map across to the research stages outlined previously

	<div>More patient engagement ← → Less patient engagement</div>									
Identifying & prioritizing the research question	8	11		12	14	15	6	9	10	
Research design stage	21	1	2	14	18	20		9	10	
Development of grant proposal	21	2	7				1	9	10	
Preparation for study implementation	21	1	18				1	9	10	
Data collection	3	5	13	1	20	1	9	10	19	
Data analysis	3	13	1	20			9	10	16	
Dissemination of finding	8	21	1	20		9	10	16	19	
Evaluation of the study	17	21	2			4	9	10	19	

1 Advisory group

2 Charettes

3 Citizen Juries

4 Comment forms

5 Deliberative polling

7 Dialogue technique

8 Digital Storytelling

9 Discussion groups

10 Interviews

11 James Lind Alliance

13 Participatory decision-making

14 Patient journey mapping

15 Patient observation of existing services

16 Public meeting/town hall

17 Reality check

19 Surveys/questionnaires

20 Workshops

21 World café

Key

○ Consultation/data collection

● Collaboration