

Trials and tribulations:

Busting Myths in Patient Engagement

June 18, 2024



ACT Accelerating  Clinical Trials
AEC Accélérer les Essais Cliniques

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A note on terminology



Patient Engagement



Patient and Public Involvement



Patient and Public Involvement
and Engagement



Consumer Engagement

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

Alternatives to ‘Patient’

- **Person with lived experience (PWLE or PLEx)**
- **People with lived or living experience (PWLLE)**

What is patient engagement in clinical trials?

“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 [...]” (Canada)



Partners



Participants

Motivation for patient engagement within 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.”



Canada's Strategy for Patient-Oriented Research

Improving health outcomes through evidence-informed care

August 2011

Canadian Institutes of Health Research. (2011). "Canada's Strategy for Patient-Oriented Research.", <http://www.cihr-irsc.gc.ca/e/44000.html>.

Canadian infrastructure to support patient engagement in clinical trials



Réseau de recherche sur les données de santé du Canada
Health Data Research Network Canada

Despite this, there is still limited patient engagement reported in clinical trials

JAMA Network | Open



Original Investigation | Medical Journals and Publishing

Patient and Family Representation in Randomized Clinical Trials Published in 3 Medical and Surgical Journals: A Systematic Review

Nissim Benizri, MD; Sophie Hallot, BSc; Karen Burns, MD, MSc; Michael Goldfarb, MD, MSc

Abstract

IMPORTANCE Patient and family engagement in research may improve the design, conduct, and dissemination of clinical research, but little is known about whether these stakeholder groups are involved in the design and conduct of randomized clinical trials.

OBJECTIVE To characterize the involvement and role of patient and family representatives in the design and conduct of randomized clinical trials by reviewing randomized clinical trials from 3 peer-reviewed medical and surgical journals with high impact factors.

EVIDENCE REVIEW In this systematic review, the first 50 consecutive randomized clinical trials published on or after January 1, 2021, until September 30, 2021, from each of 3 medical and surgical journals with high impact factors were reviewed for patient or family involvement in trial design

and/or conduct of design and extracted data analyses (PF)

FINDINGS In their study of a single journal studies stake

CONCLUSIONS family involvement high impact

the focus was mainly on the execution phase of research design. There is a need to increase stakeholder involvement in the research design, conduct, and translation of randomized clinical trials.

Key Points

Question Are patient or family representative groups involved in the design or conduct of randomized clinical trials published in 3 medical and surgical journals with high impact factors?

Findings In this systematic review of consecutive randomized clinical trials from 3 journals, 7 of 150 trials (5%) included patient partners or community representative groups. Stakeholder involvement was mainly in the

7/150 trials (5%) reported any patient engagement

JAMA Network Open. 2022;5(9):e2230858. doi:10.1001/jamanetworkopen.2022.30858

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^{1,8*}, Zarah Monfaredi¹, Kusala Pussegoda¹, Chantelle Garrity¹, 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

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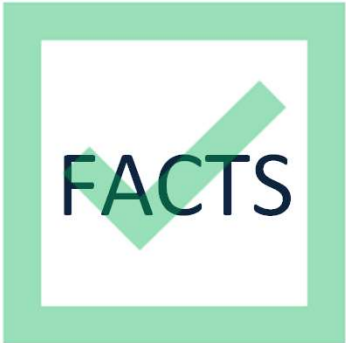
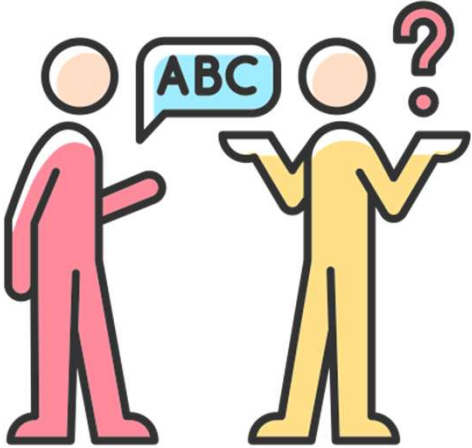
“With only 23 trials identified in this report, we estimate that far less than 1% (23/371,159) of clinical trials engage patients meaningfully and actively”

report patient engagement for the purposes of research were included.

(Continued on next page)

Understanding (and busting) the myths may help improve patient engagement in clinical trials

The Myths



Myth #1 : Clinical trials are too complicated for patients to be meaningfully engaged as partners

By Maureen Smith and Stuart Nicholls



It's too difficult to explain clinical trials & research methodology

FALSE



Youth are vulnerable and could be harmed

TRUE

Using a trauma-informed approach can mitigate this



Caregiver engagement includes youth perspectives

FALSE



Ethical implications when engaging with minors

TRUE

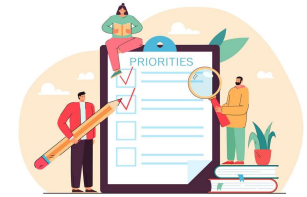
Special considerations must be in place

Example: Core Outcome Sets for clinical trials

Core Outcome Sets: an agreed standardised set of outcomes that should be measured and reported in all clinical trials of a specific condition.

Why engage with patient partners in their development?

- Advise on how to present materials (e.g., definitions of outcomes), how to set up patient-friendly Delphi surveys, and recruitment and participation in consensus meetings to include patient perspectives.
- Biggest impact of patient participation: inclusion of life impact outcomes: 86% (239) of the 278 COS involving patients have at least one life impact outcome, compared to 62% (193) of the 311 COS where patients were not included.
 - This impact is observed across all individual domains within the life impact area, including functioning and quality of life domains.
- Input of public and patients does not seem to impact the inclusion of adverse events, mortality, or physiological outcomes
- Resource use outcomes are included more often.



Outcomes may not align with **patients' priorities**



ELSEVIER



Journal of Clinical Epidemiology 158 (2023) 127–133

Journal of
Clinical
Epidemiology

REVIEW ARTICLE

Patient participation impacts outcome domain selection in core outcome sets for research: an updated systematic review

Susanna Dodd^{a,*}, Sarah L. Gorst^a, Amber Young^{b,c}, Samuel W. Lucas^a, Paula R. Williamson^a

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^bBristol Centre for Surgical Research, Population Health Sciences, Bristol Medical School, University of Bristol, Bristol, UK

^cChildren's Burns Research Centre Bristol, University Hospitals Bristol and Weston NHS Foundation Trust, Bristol, UK

Accepted 25 March 2023; Published online 11 April 2023

S. Dodd et al. / Journal of Clinical Epidemiology 158 (2023) 127e133

Question: who is better placed to understand the impacts, burdens and realities of a condition than patients, families, and caregivers?

Research Involvement and Engagement

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Research | [Open Access](#) | [Published: 07 September 2022](#)

The impact of patient engagement on trials and trialists in Ontario, Canada: An interview study with IMPACT awardees

[Stuart G. Nicholls](#)  [Grace Fox](#) [Zarah Monfaredi](#) [Evelyn Poole](#) [Chantelle Garrity](#) [Alies Maybee](#) [Justin Presseau](#) [Beverley Shea](#) & [Dean A. Fergusson](#)

Research Involvement and Engagement 8 Article number: 50 (2022) | [Cite this article](#)

1252 Accesses | 11 Altmetric | [Metrics](#)



RESEARCH ARTICLE

What Difference Does Patient and Public Involvement Make and What Are Its Pathways to Impact? Qualitative Study of Patients and Researchers from a Cohort of Randomised Clinical Trials



[Louise Dudley](#)¹, [Carrol Gamble](#)¹, [Jennifer Preston](#)², [Deborah Buck](#)³, [The EPIC Patient Advisory Group](#)¹, [Bec Hanley](#)⁴, [Paula Williamson](#)¹, [Bridget Young](#)¹

¹ Department of Biostatistics, University of Liverpool, Liverpool, United Kingdom, ² NIHR Clinical Research Network: Children, Coordinating Centre, University of Liverpool, Department of Women's and Children's Health, Liverpool, United Kingdom, ³ TwoCan Associates, Brighton, United Kingdom, ⁴ Department of Psychological Sciences, University of Liverpool, Liverpool, United Kingdom

South et al. *Trials* (2016) 17:376
DOI 10.1186/s13063-016-1488-9

Trials

RESEARCH

Open Access



Models and impact of patient and public involvement in studies carried out by the Medical Research Council Clinical Trials Unit at University College London: findings from ten case studies

[Annabelle South](#)^{1*}, [Bec Hanley](#)¹, [Mitzy Gafos](#)¹, [Ben Cromarty](#)^{1,2}, [Richard Stephens](#)^{1,3}, [Kate Sturgeon](#)¹, [Karen Scott](#)¹, [William J. Cragg](#)¹, [Conor D. Tweed](#)¹, [Jacqueline Teera](#)¹ and [Claire L. Vale](#)¹

Open access

Original research

BMJ Open Patient and public involvement in numerical aspects of trials: a mixed methods theory-informed survey of trialists' current practices, barriers and facilitators

[Beatriz Goulao](#)  [Camille Poisson](#), [Katie Gillies](#) 

Open access

Original research

BMJ Open Patient and public involvement in numerical aspects of trials: a mixed methods theory-informed survey of trialists' current practices, barriers and facilitators

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RESEARCH

 OPEN ACCESS

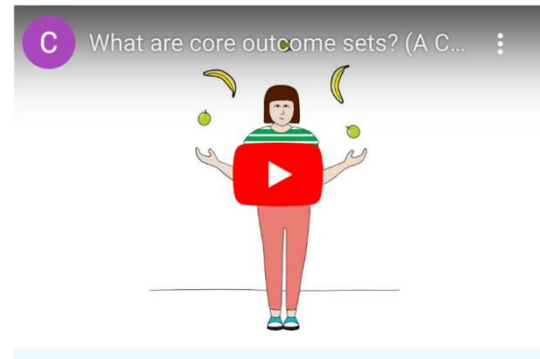
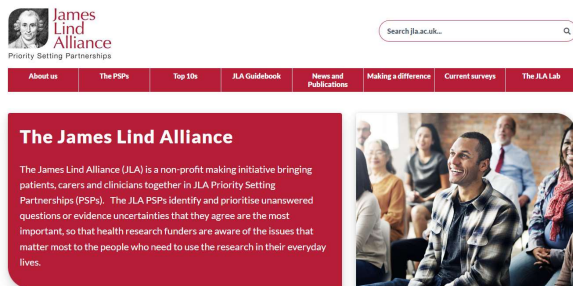
Impact of patient and public involvement on enrolment and retention in clinical trials: systematic review and meta-analysis

 Check for updates

[Joanna C Crocker](#)^{1,2}, [Ignacio Ricci-Cabello](#)^{3,4,5}, [Adwoa Parker](#)⁶, [Jennifer A Hirst](#)⁷, [Alan Chant](#)², [Sophie Petit-Zeman](#)², [David Evans](#)⁸, [Sian Rees](#)⁹

“On average, PPI interventions modestly but significantly increased the odds of participant enrolment.”
Crocker, J. C., I. Ricci-Cabello, A. Parker, J. A. Hirst, A. Chant, S. Petit-Zeman, D. Evans and S. Rees (2018). "Impact of patient and public involvement on enrolment and retention in clinical trials: systematic review and meta-analysis." *BMJ* **363**: k4738.

Take home message: Working with patients and families can improve your trial design & many tools exist to help



Formalised priority setting processes to identify important research questions

e.g. James Lind Alliance Priority Setting Partnership

<https://www.jla.nihr.ac.uk>

Methods and resources for sets of agreed important outcomes

e.g. COMET Public & Patient Involvement Toolkit

<https://comet-ppi-toolkit.liverpool.ac.uk>

Tools to engage in discussions about barriers and facilitators

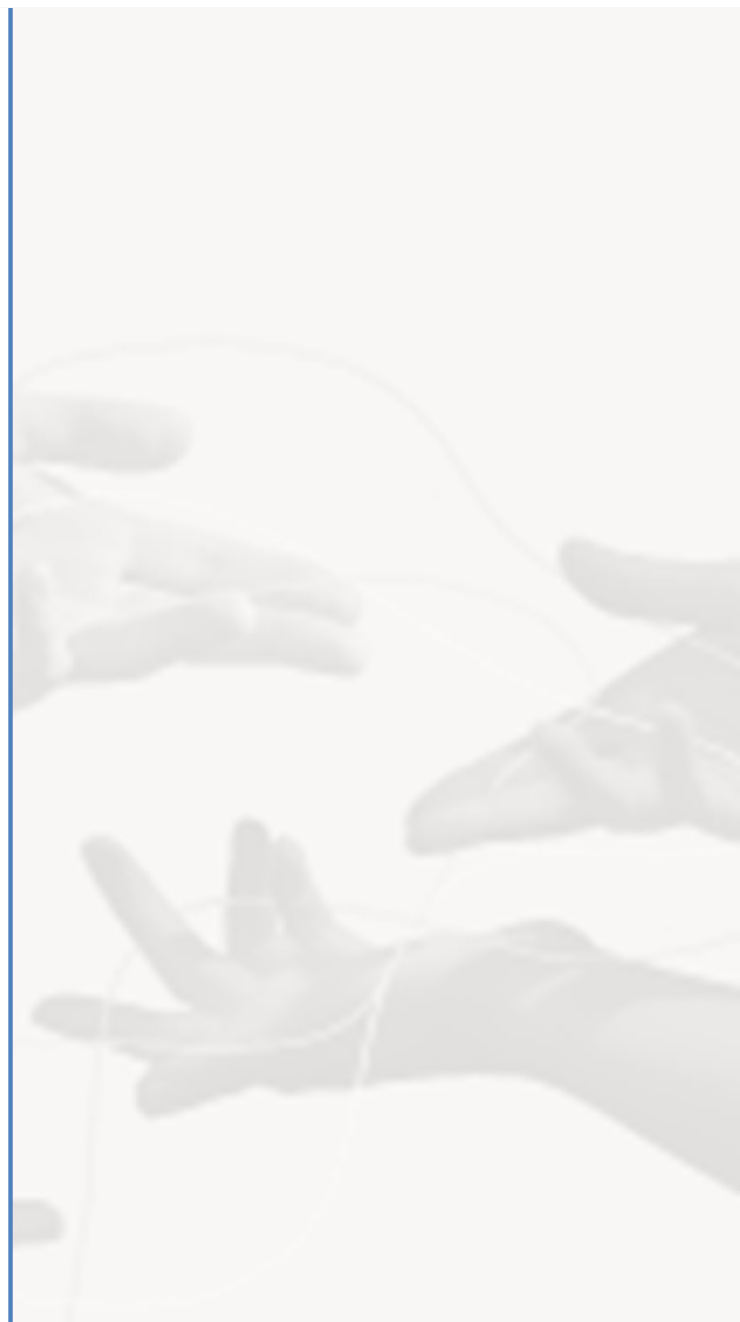
e.g. Perceived Research Burden Assessment (PeRBA) tool

Lingler, J. H., K. Schmidt, A. Gentry, L. Hu and L. Terhorst (2014). "Perceived Research Burden Assessment (PeRBA): Instrument Development and Psychometric Evaluation." *Journal of Empirical Research on Human Research Ethics* 9(4): 46-49.

Myth #2

Only Patients Need Training

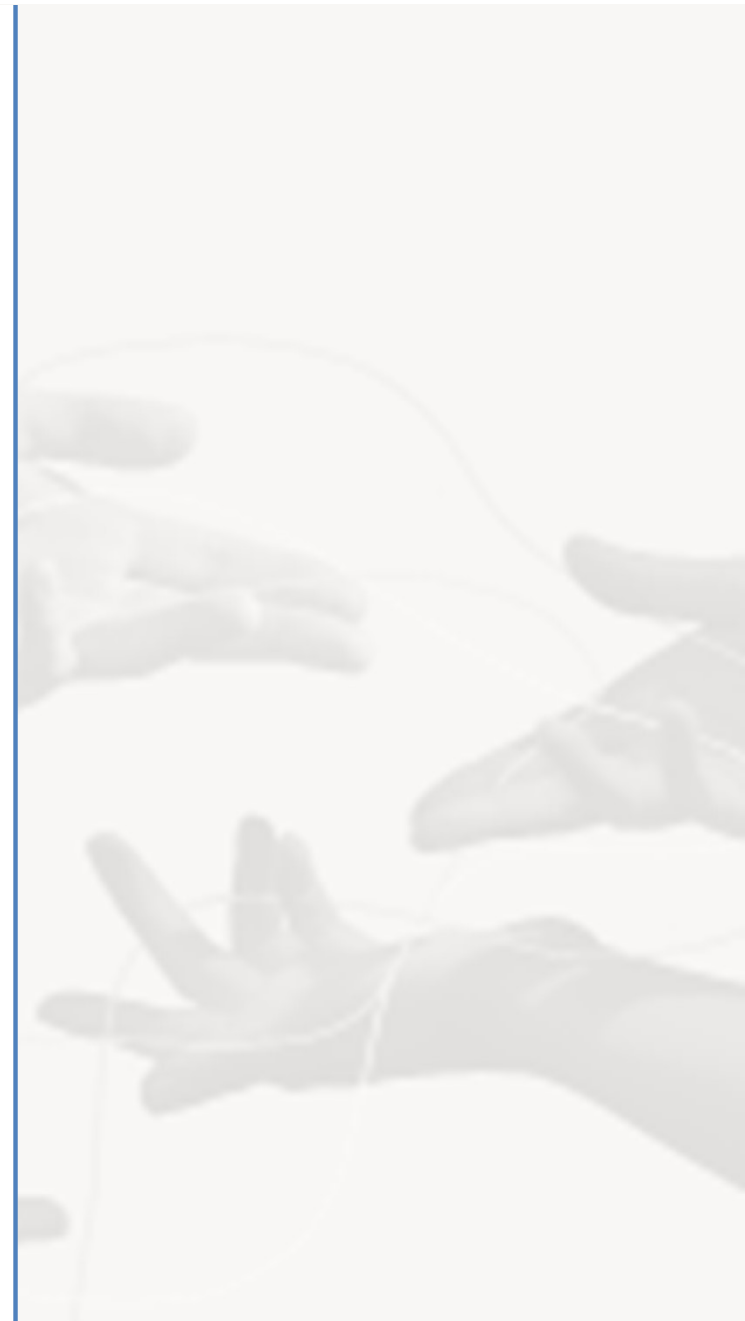
By Arlene Desjarlais and Alicia Murdoch



Busting the myth

While it is true that people with lived experience are usually not people with medical or research backgrounds that doesn't mean that they are the only ones who need training to do patient engagement in clinical trials.

Researchers have been trained on the methodology of research such as how to develop a research question and select trial outcomes, not how to engage patient partners in the work.



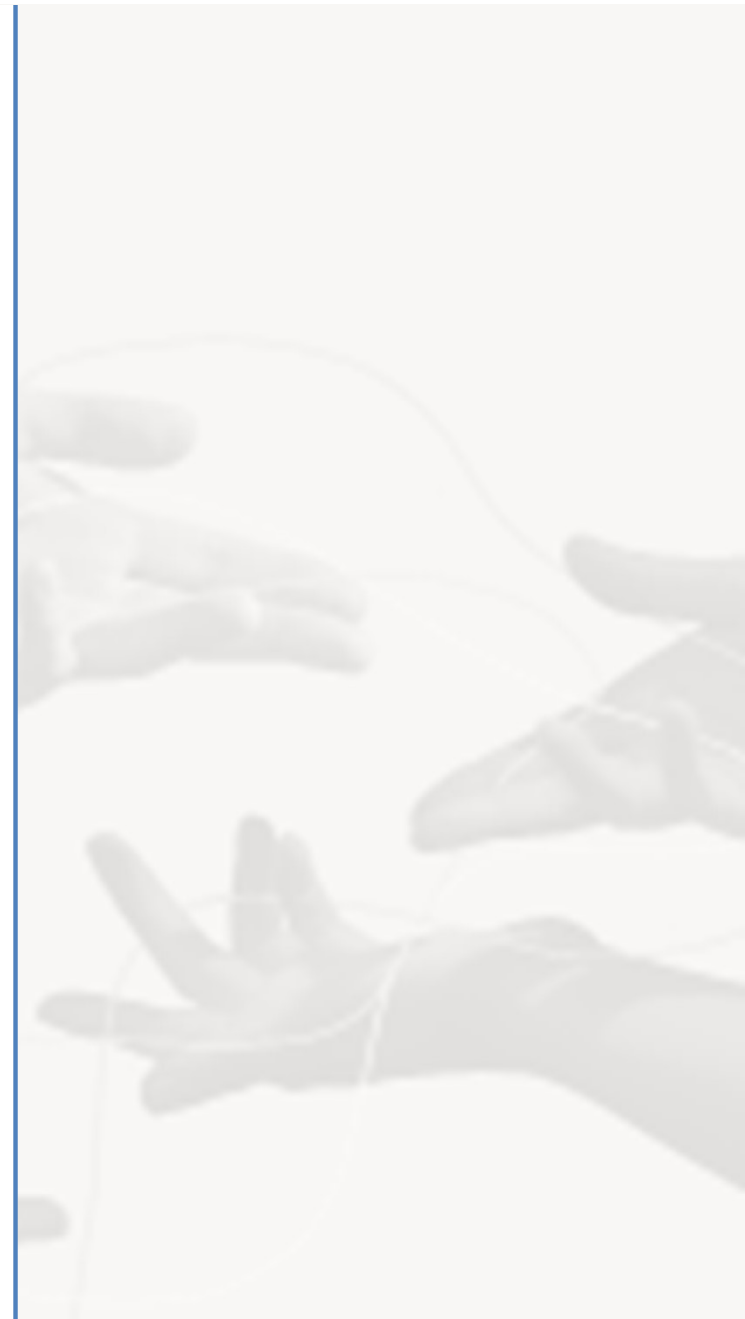
Real-life examples

Positive

Changes made to the Kidney Check trial as an example of listening to patient partners and incorporating their feedback

Negative

Engaging patient partners early in the process but not keeping them up to date on the progress of the trial initiation

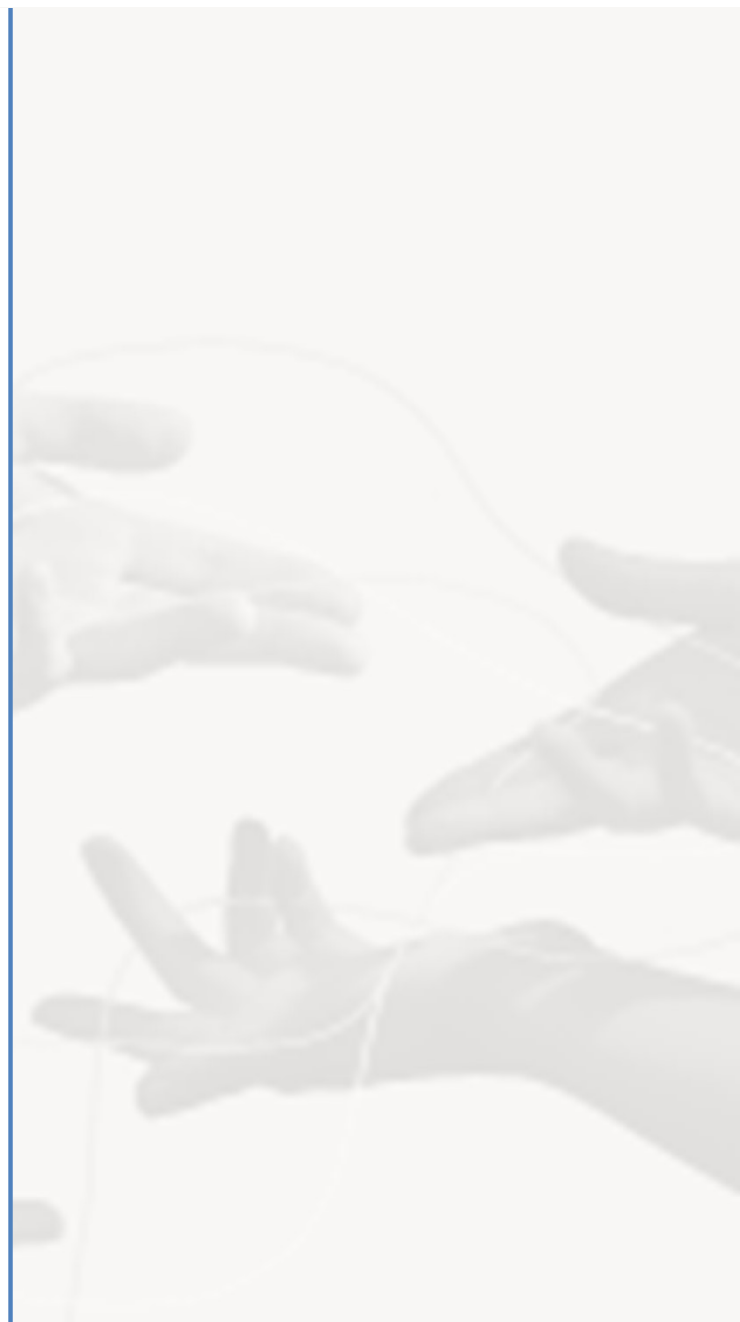


Take home message

Researchers need training and support in how to work with people with lived experience on a clinical trial

It is a different type of training than what the people with lived experience will require, but it is necessary to have successful engagement.

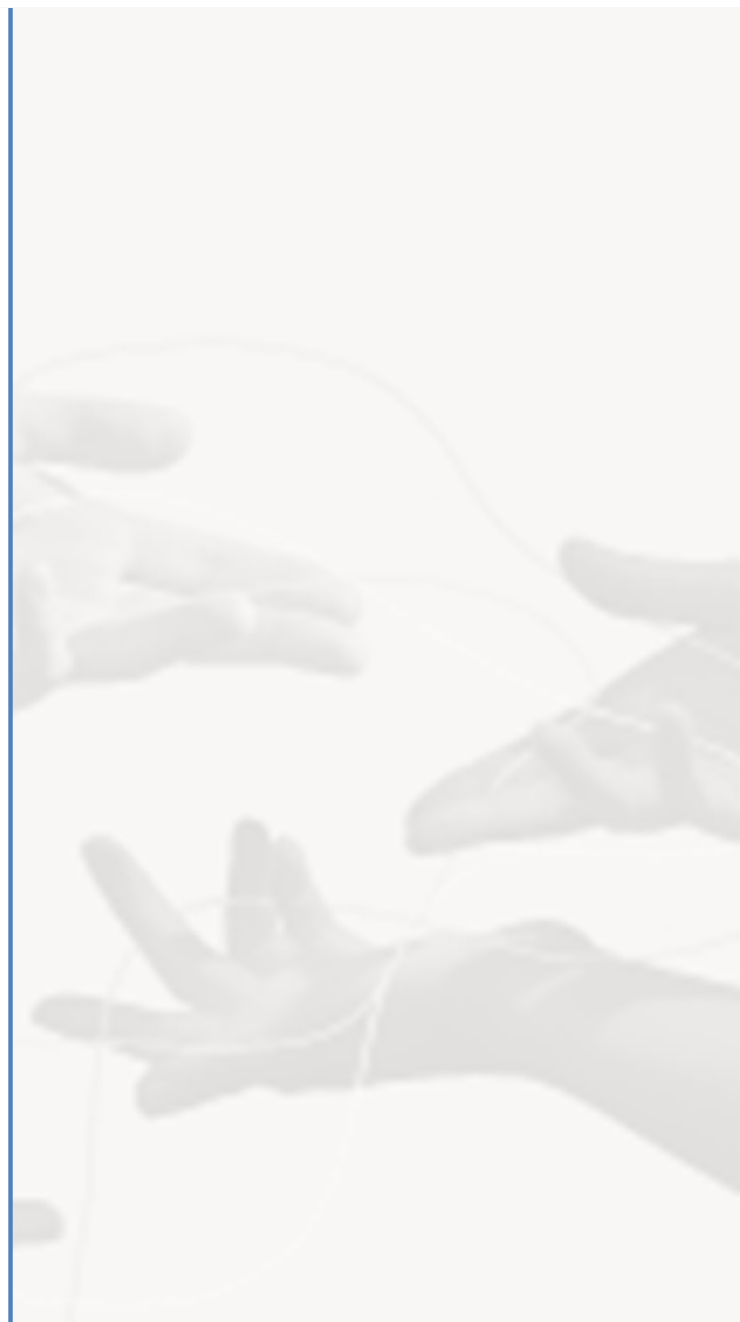
The Patient Engagement Committee of ACT has members and resources that can help.



Myth #3

Patient engagement is just inviting people with lived experience to the table

By Atobrhan Godlu



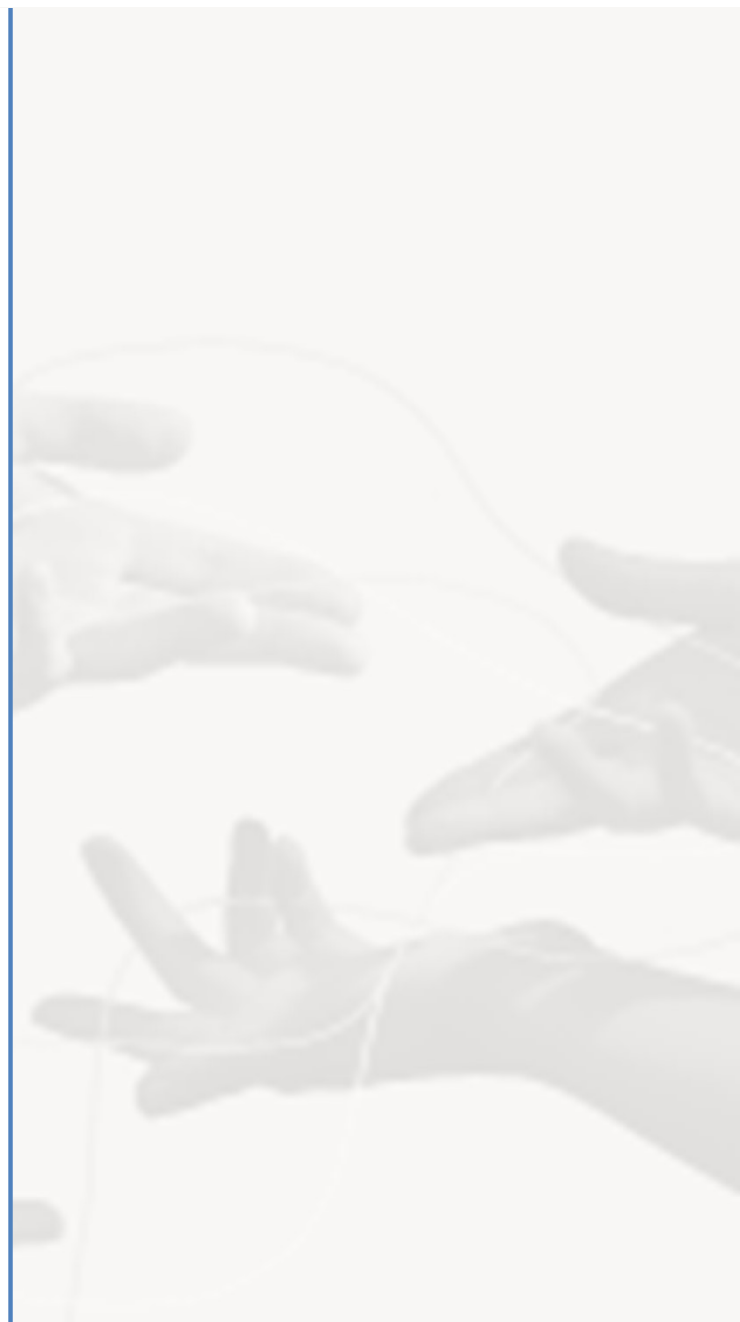
Patient partners and Patient participants

Patient Partner:

An equal partner on a research team whose valued lived experiences and expertise can inform and advise at all stages of the research cycle.

Patient Participant:

Traditional role of patient as subject/participant in research study.



Patient Engagement and It's importance:

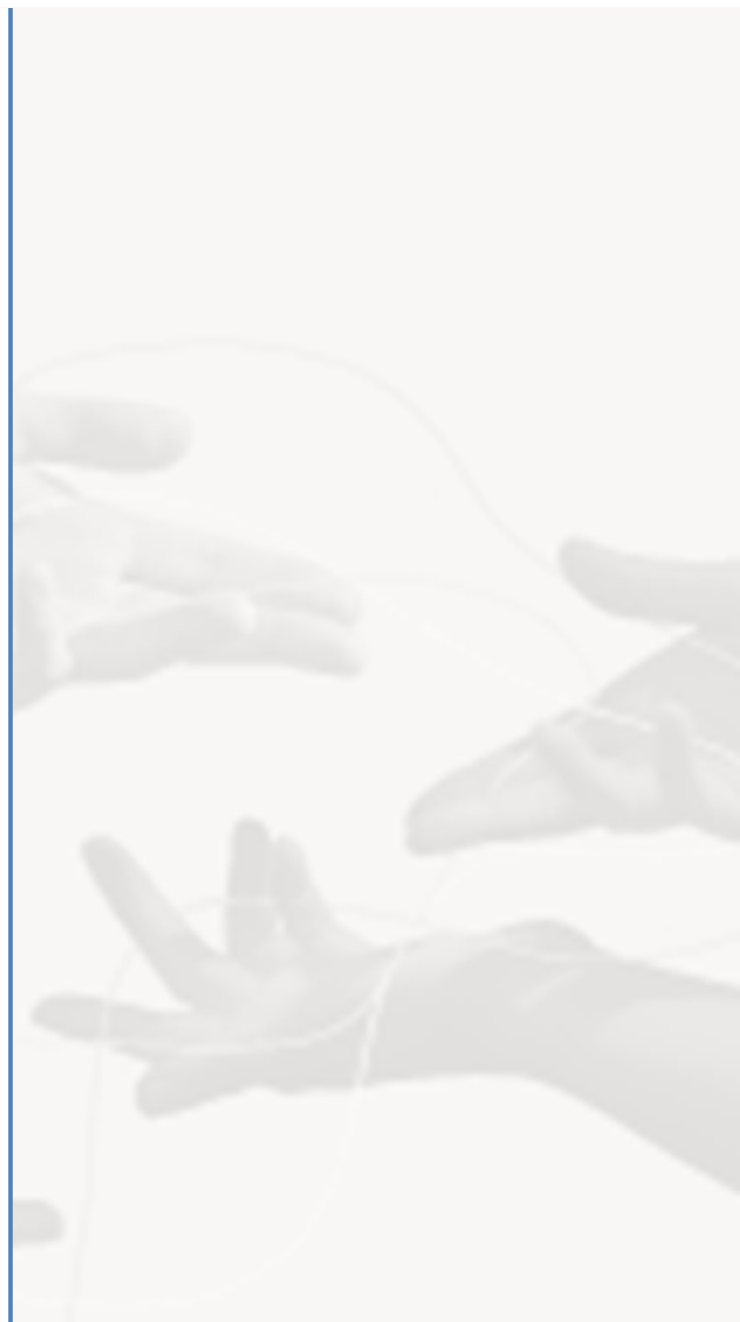
What is patient engagement?

➤ **Patient engagement occurs when patients meaningfully and actively collaborate in the governance, priority setting, and conduct of research.**

Why is patient engagement in research important from an ethical perspective?

A meaningful patient engagement:

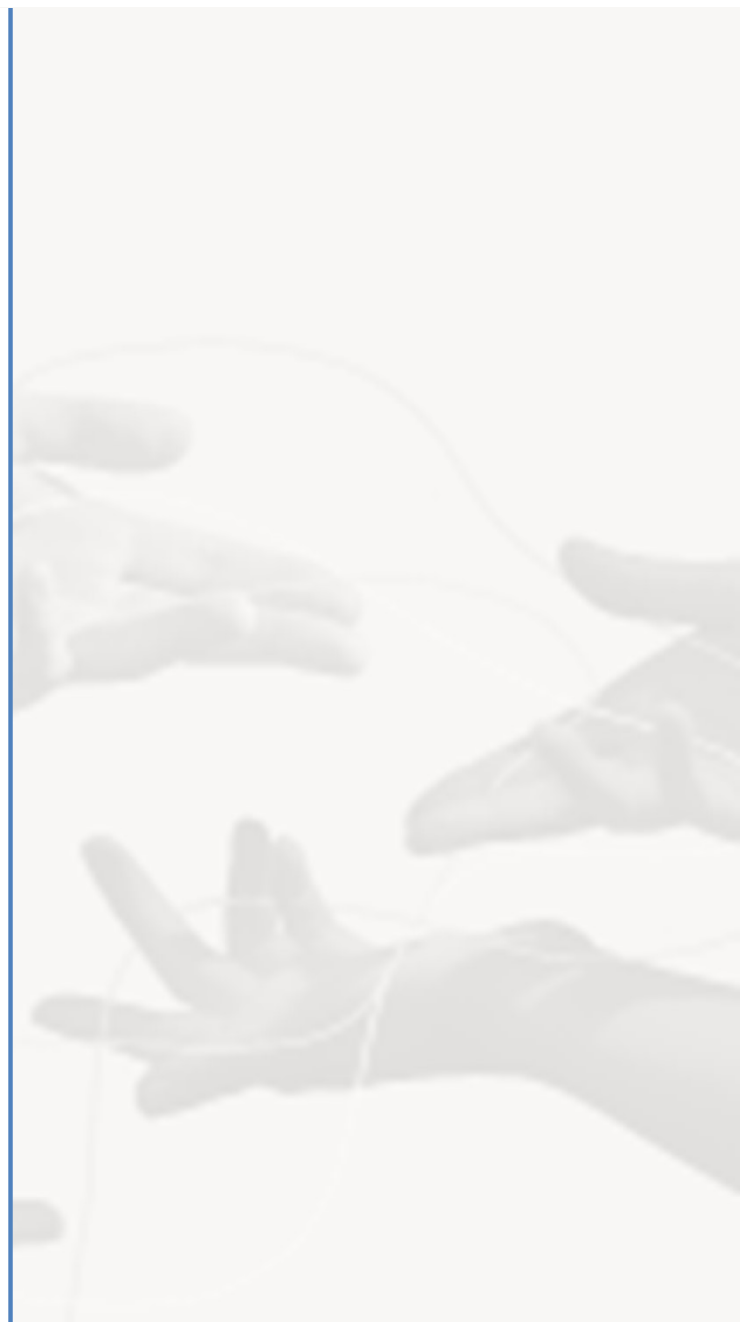
- **grounds research in a deep understanding of the health situations and the living or lived experiences of actual patients, including groups that are typically under-represented in research, to make research more relevant and usable by those patients;**
- **promotes research methods that are culturally safe, respectful, and appropriate;**
- **legitimizes research in the eyes of the community that the research is intended to benefit**
- **Create a respectful dialogue and discussion where each person can speak in their own voice**



Levels of engagement

Patients may take on specific tasks in the research process based on their skill levels.

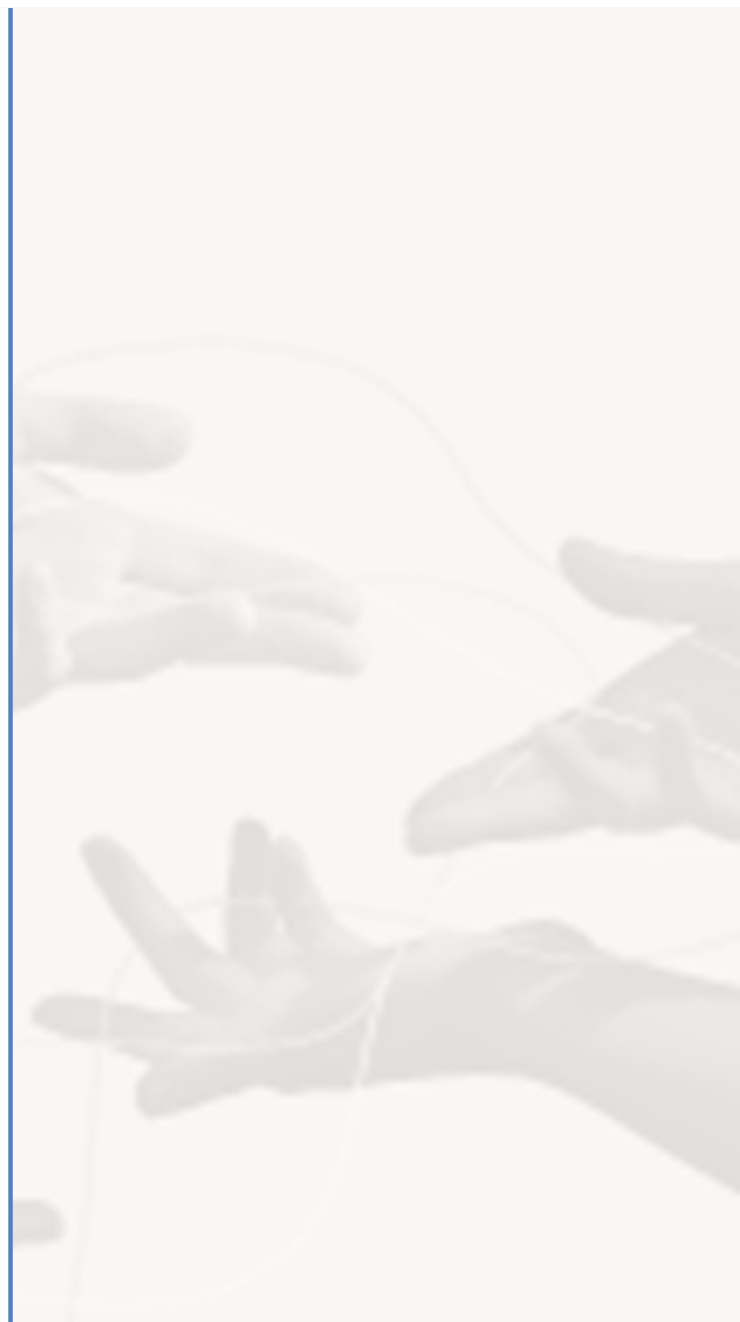
- **can lead focus groups and do interviews.**
- **Or can be partners in design and implementation, or co-authors of the various outputs from the study.**



Typical stages of a research project

- **Priority setting and planning**
- **Development of the research proposal**
- **Scientific review**
- **Ethics Review**
- **Oversight of a research project**

- **Recruitment of research participants**
- **Data collection and Data analysis**
- **Knowledge exchange and translation**



Myth #4 - Partners With Lived Experience *cannot* be Principal Investigators on CIHR Grants

By Annette Majnemer



CIHR Website: Principal Applicants (PWLE)

- * Applicant Profile CV (instead of Common CV)
- * Register for CIHR PIN
- * Complete Equity, Diversity and Inclusion Self-Identification Questionnaire
- * Certificate of Completion of Sex and Gender Module
- * Most Significant Contributions (paragraph form)
- * Define Indigeneity (if appropriate)



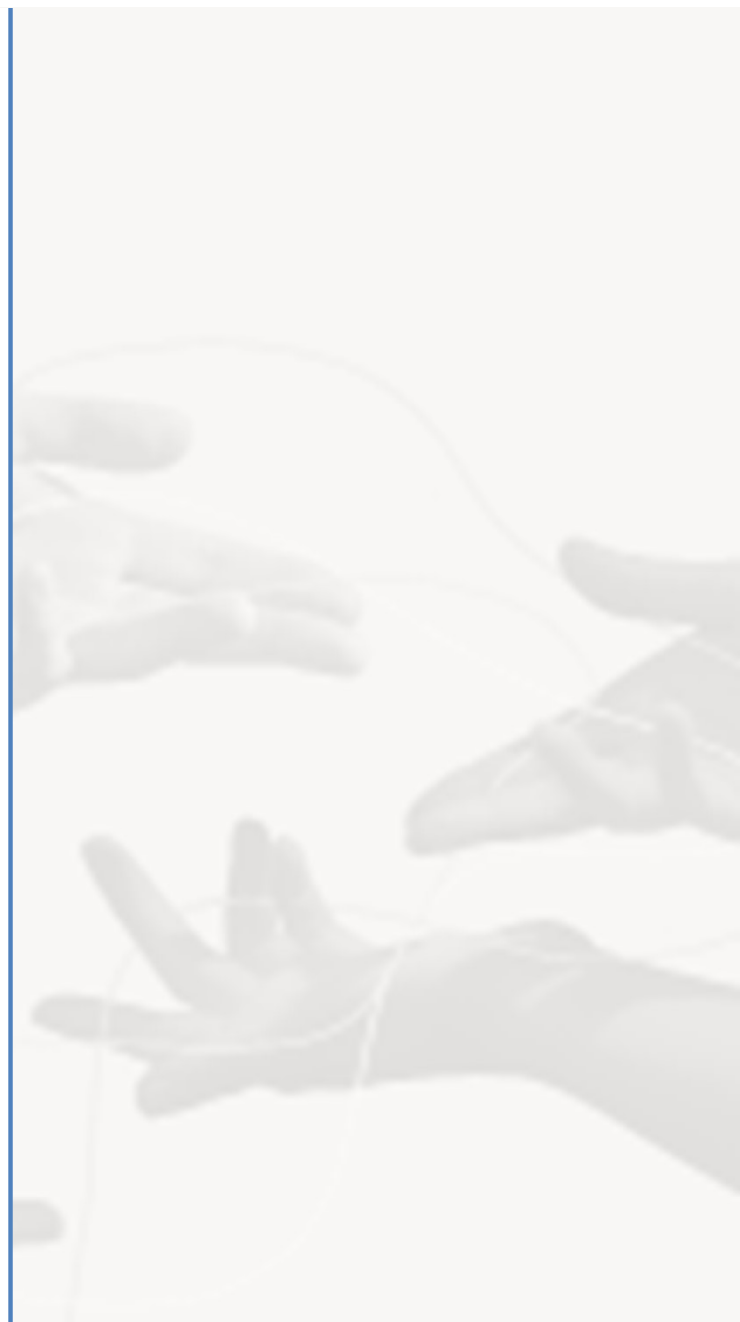
Further Elaboration

* Applicant Profile CV (instead of Common CV)

- Personal statement
- Positions and honours
- Contributions to knowledge creation, knowledge sharing, and/or knowledge translation
- Other relevant info (optional)

* Most Significant Contributions (paragraph form)

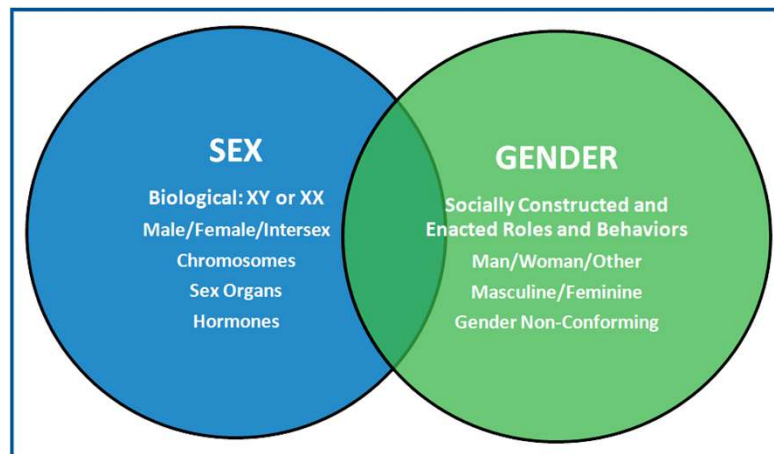
- Publications, presentations, intellectual property, standards, code, datasets, other knowledge translation activities, etc
- Training and mentorship
- Degrees, credentials, awards, certificates etc
- Clinical practice, policy development, community engagement etc
- Specialized training, strategic employment positions, etc



Sex and Gender Module

" I really really struggled with this training. It is really not meant for someone who has no knowledge of medicine and high-level research ethics issues. I have very good knowledge of sex and gender, but in this context it was difficult, because it relied on your understanding that these concepts and how they relate to certain medical conditions, it was quite difficult to complete. "

- PWLE, CHILD-BRIGHT



PWLE can be PIs, but...



* Sex and gender module requires high level knowledge of medical applications, theoretical concepts. Can be challenging for most to complete.

* Registering a PIN number can be hard to navigate

* Challenges with what to include in Applicant Profile CV and Significant Contributions

> Will likely need support to complete these sections



Myth #5 - Patient Partners are Offended by Compensation

By Amanda Doherty-Kirby



Patient Experience Journal
Volume 5, Issue 3 – 2018, pp. 6-12

Commentary

Patient partner compensation in research and health care: the patient perspective on why and how

Dawn P. Richards, *Five02 Labs and the Canadian Arthritis Patient Alliance*, dawn.p.richards@gmail.com

Isabel Jordan, *Rare Disease Foundation*, isabeljordan@me.com

Kimberly Strain, *Independent Patient Partner*

Zal Press, *Patient Commando*, zal@patientcommando.com

Busting the myth - Compensation



Reimbursement of expenses



Recognizes patient partner's time, skills, and expertise

While some choose to volunteer as patient partners and have the means to do so, there are many reasons to compensate patient partners¹:

- **Equity**
- **Different motivations**
- **Respect for vulnerability**
- **Commitment**
- **Removes barriers**
- **Respect, value, and trust^{2,3}**

1) Richards DP, Jordan I, Strain K, Press Z. Patient partner compensation in research and health care: the patient perspective on why and how. *Patient Experience Journal*. 2018; 5(3):6-12. doi: 10.35680/2372-0247.1334. 2) Hamilton CB, Hoens AM, Backman CL, McKinnon AM, McQuitty S, English K, Li LC. An empirically based conceptual framework for fostering meaningful patient engagement in research. *Health Expect*. 2018 Feb;21(1):396-406. doi: 10.1111/hex.12635. 3) Skinner JS, Williams NA, Richmond A, Brown J, Strelnick AH, Calhoun K, De Loney EH, Allen S, Pirie A, Wilkins CH. Community Experiences and Perceptions of Clinical and Translational Research and Researchers. *Prog Community Health Partnersh*. 2018;12(3):263-271. doi: 10.1353/cpr.2018.0050. 3)

Considerations

- Assumptions/Bias
- Institutional Policies and Procedures
- Flexible Payment Options
rate, form, timing
- Income/Tax Implications
- Privacy
- Budget/Funding
- Non-Monetary Forms of Recognition

COMMENTARY

Open Access

Identifying potential barriers and solutions to patient partner compensation (payment) in research

Dawn P. Richards^{1,2,3,4*}, Kelly D. Cobey^{5,6}, Laurie Proulx^{3,7}, Shoba Dawson⁸, Maarten de Wit⁹ and Karine Toupin-April^{10,11,12,13}

Published in final edited form as:
Citizen Sci. 2019 March 8; 4(1): . doi:10.5334/cstp.184.

Patients as research partners; how to value their perceptions, contribution and labor?

Elise Smith^{1,*}, Jean-Christophe Bélisle-Pipon², David Resnik¹

¹National Institute of Environmental Health Sciences, National Institutes of Health, Research Triangle Park, NC, 27709 USA

²The Petrie-Flom Center for Health Law Policy, Biotechnology, and Bioethics, Harvard Law School, Cambridge, MA 02138 USA

Patients and Families as Partners in Patient-Oriented Research: How Should They Be Compensated?

Monika Novak-Pavlic, PhD,^{1,2} Jan Willem Gorter, MD, PhD,^{1,3,4} Michelle P. Phoenix, PhD, SLP,^{1,2} Samantha K. Micsinszki, PhD, RN,^{1,2} Kinga Pozniak, PhD,¹ Lin Li, BSN, RN,⁵ Linda Nguyen, PhD,^{1,2} Alice K. Soper, MSc,^{1,2,3} Elaine Yuen Ling Kwok, PhD, SLP,¹ Jael N. Bootsma, PhD,^{1,2} Francine Buchanan, PhD,⁶ Hanae Davis, PhD,^{1,7} Sandra Abdel Malek, MSc,^{1,2} Karen M. van Meeteren, BSc,⁸ Peter L. Rosenbaum, MD, DSc (HC)^{1,3}

¹CanChild Centre for Childhood Disability Research, McMaster University, Hamilton, Canada; ²School of Rehabilitation Science, McMaster University, Hamilton, Canada; ³Department of Pediatrics, McMaster University, Hamilton, Canada; ⁴Department of Rehabilitation, Physical Therapy Science & Sports, UMC Utrecht Brain Center, University Medical Center Utrecht, Utrecht, Netherlands; ⁵School of Nursing, McMaster University, Hamilton, Canada; ⁶SickKids, Toronto, Canada; ⁷University Health Network, Toronto, Canada; ⁸OuderInzicht, Amsterdam, Netherlands

Take home messages

Patient partners should have a choice in how they are compensated and/or recognized

Budget for partnering with patients

Know institutional procedures and guide patient partners as needed

Researchers should initiate the conversation with patient partners



1. Initiate the conversation. Ask patient partners how they would prefer to discuss compensation (eg, in person, phone, videoconference, e-mail, etc).



2. Be prepared. Find out as much as possible about logistics in advance, including: what is possible, what are the potential implications (eg, additional income, disability payments, etc), when does payment happen, what is required (eg, paperwork, invoice, etc)?



3. Confirm the patient partner's preferences for compensation. It is a patient partner's choice to receive or not receive compensation, and in the manner of his or her choosing. The amount should reflect engagement length and effort.

Compensation

Monetary considerations:

- Lump sum or hourly rate
- Date(s) of payment, etc

Nonmonetary considerations:

- Gift cards, payment of phone bills/internet bills, attendance for a course/conference of their choosing, etc



No compensation

Considerations:

- What other forms of recognition are available? For example, authorship or acknowledgment if appropriate?
- Is the patient partner okay with these other forms of recognition? Decisions around acknowledgment should remain with the patient partner (eg, if someone lives with a stigmatized condition, then he or she may have preferences around public acknowledgment)



4. Take care of the details. If you encounter any issues at your institution/organization, be honest with the patient partner about these and be prepared to do required follow-up.

Richards, D.P., Jordan, I., Strain, K. and Press, Z., 2020. Patients as partners in research: how to talk about compensation with patient partners. *journal of orthopaedic & sports physical therapy*, 50(8), pp.413-414.

<https://www.jospt.org/doi/epdf/10.2519/jospt.2020.0106>

Myth #1

Clinical trials are too complicated for patients to be meaningfully engaged as partners

Myth #3

Patient engagement is just inviting people with lived experience to the table

Myth #2

Only patients need training

**Myths
busted!**

Myth #4

Partners with lived experience cannot be Principal Investigators on CIHR grants

Myth #5

Patient partners are offended by compensation

Patient and Family Engagement in Clinical Trials: *How, When, and Why*



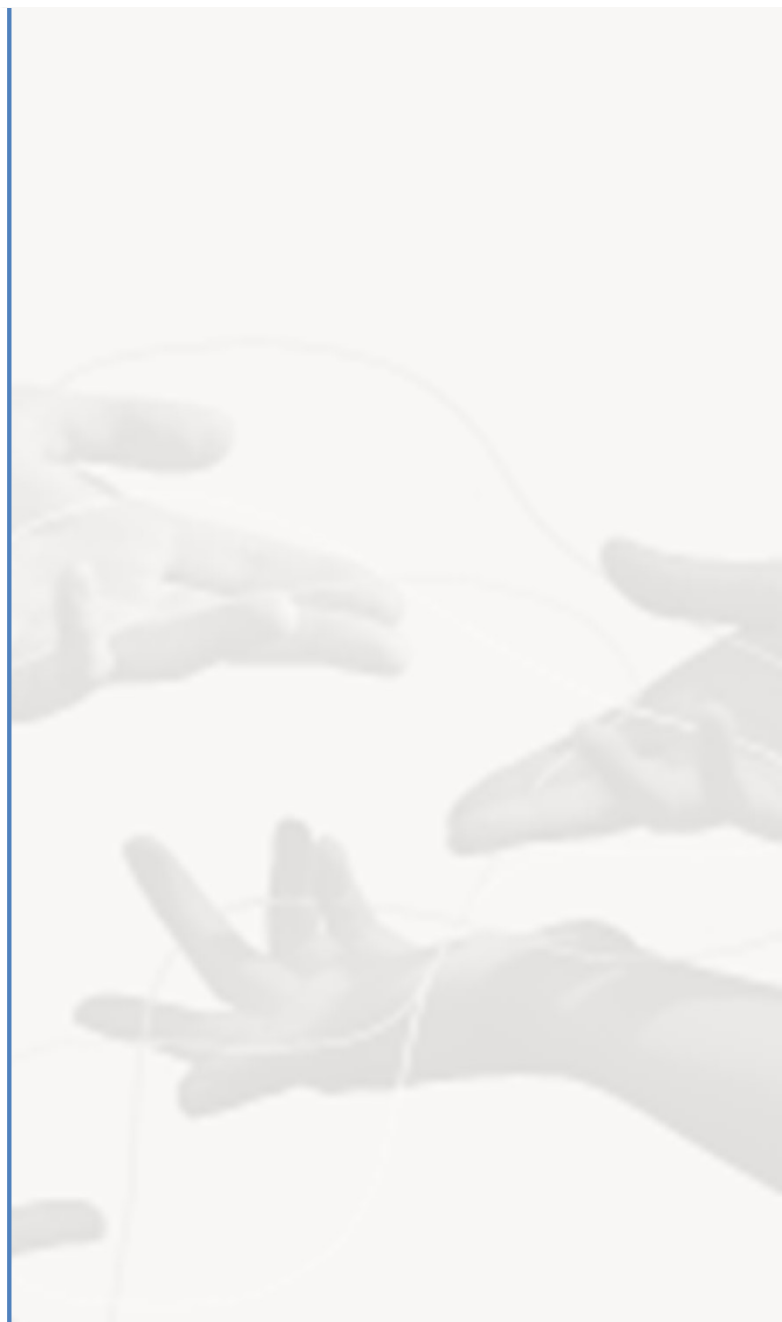
Resources and references: [CTO Participant Experience Toolkit](#); [Bagley et al. 2016](#); [Crocker et al. 2018](#); [Manafo et al. 2018](#)

This resource was created as part of the McMaster University, CanChild, and Kids Brain Health Network Family Engagement in Research Certificate of Completion Program. Copyright © 2021 (B. Bedir, N. Butcher, L. Mendonza, A. Walker). All Rights Reserved.

Busting myths is part of the patient engagement journey!

We appreciate the efforts of so many to advance patient engagement in clinical trials.

Time for Q&A



**ACT brings together
hundreds of researchers
from 28 networks and
11 trial units—from *Nova
Scotia to British Columbia
and Nunavut*—to maximize
research impact and
knowledge mobilization.**

