Monitoring and Evaluating Patient Engagement in Medicines Development: How literature informed a draft evaluation framework.

Teresa Finlay, University of Oxford; Lidewij Vat, Vrije Universiteit Amsterdam; Paul Robinson, MSD, London.

Please do not cite or distribute. This content and further detail will be published in a forthcoming journal article

PARADIGM
Patients Active in Research and Dialogues for an Improved Generation of Medicines

Key findings and conclusions

- Use of terminology is inconsistent
- Outcomes and impacts are commonly described
- Indicators are repeatedly suggested
- Methods and measurement tools are limited
- Existing approaches are largely qualitative and focused on the research process
- No standardised approaches to assess the outcomes and impact of patient engagement exist
- Consensus-based monitoring and evaluation frameworks are needed

Background

Cultural change is needed to embed patient engagement (PE) as standard practice in medicines research and development. This could be enhanced and supported by measures that demonstrate the 'return on engagement'. There is increased focus on evaluation of PE, although motives vary according to each groups' vested interests. Not least, evaluation could contribute to the business case for PE by securing funding whilst simultaneously improving PE activities and contributions. This could ultimately result in more appropriate, acceptable and available treatments.

PARADIGM aims to contribute to a sustainable framework that enables meaningful PE and demonstrates 'return on engagement' for all stakeholders. We focus on three key points in medicines development:

- Research and priority setting
- Design of clinical trials
- Early dialogues with regulators and HTA bodies

In addition PARADIGM is particularly interested in three populations

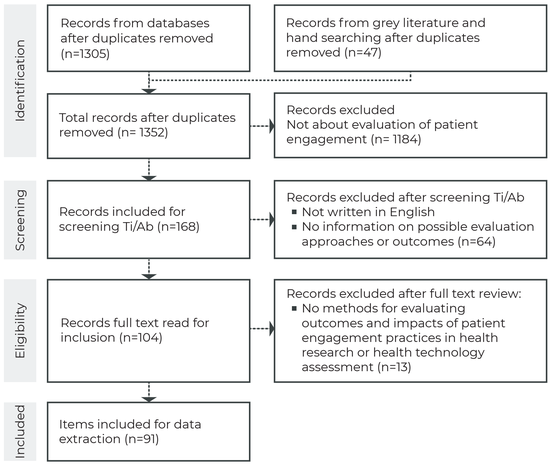
- Adults
- Children and young people
- Older people living with dementia

One important output is the design of a monitoring and evaluation framework for PE at the three key points. This literature review represents the first step in our work. It maps benefits, costs and challenges of PE and some indicators for monitoring and evaluation.

Methods

- Scoping literature review based on Arksey and O'Malley (2005).
- Conducted between May and July 2018
- Search terms included 'patient, public, engagement, involvement, framework, impact, indicator, measure, outcome'
- PCORI and INVOLVE databases
- CINAHL, Embase, Medline, PsychInfo and PubMed databases
- Grey literature including reports and online documents
- Focus on quantitative measures
- Extracted data from 91 publications coded and thematically analysed to provide a detailed account and summary (Braun and Clarke 2006).
- PARADIGM partners commented on preliminary results and have contributed to manuscript and presentation drafts.

Flow diagram of results

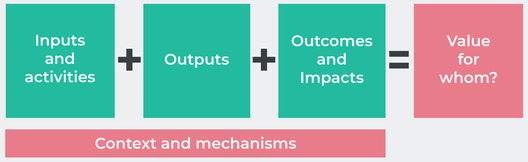


Benefits, costs and challenges of PE with some indicators for measurement

Stakeholder group	Examples of Benefits	Examples of costs and challenges	Examples of indicators
Patient partners	Empowerment, acquiring research knowledge, meaningful activity, compensation for engagement, products that better meet patients' needs.	Tokenistic inclusion, training and engagement take time, incur travel and/or carer costs, possible loss of income, stress	Rating of partner influence across study phases, rating perceived relevance and importance of studies, whether study addresses un-met need
Society	Increased acceptability and trust in research by all parties, increased knowledge and distribution of information	Conflict and power struggles, increased time and financial costs, difficulty representing severely ill or disabled	
Research participants	Improved experience of participation in research, more acceptable research process		Reading level of study documents, rating of study documents, number of changes to trial as a result of feedback
Researchers	More worthwhile research, better recruitment, enhanced knowledge and skills, career advancement	Time and financial costs, stress, fear of tokenism, methodological concerns	Number of studies gaining funding and approval
Industry	Improved efficiency, better understanding of patients' experiences, better study design, increased regulatory success, strategic value	Increased costs for trial budgets, duration and efficiency, impact on return on investment	Number of protocol amendments, recruitment and retention rates, diversity of study participants, time to HTA decisions
Regulators and HTA bodies	Increased transparency of decision making, decisions meet patients' needs, improved quality of assessment and relevance to local context	Increased uncertainty of policy making when different views require different policy responses	Perceived impact of PE in HTA, perceptions of the content of HTA reports and recommendations
Others (payers and providers)	More useful, legitimate evidence for policy decision-making	Stakeholders' conflicting goals and change lead to uncertainty about applying study recommendations	Perceived relevance and usefulness of research evidence

Results

- Mainly qualitative studies
- Valuable insight on process of PE, outcomes and impact
- Few describe relationship between input, output, outcome and impact
- Most studies focus on the benefits of PE on research
- What is missing is the value for all stakeholders involved
- Limited attention given to the context and mechanisms that influence outcomes and impact



Next steps

June 2019

Literature review article: Complete and publish

September 2019

Indicators:
- Which are relevant?
- To whom?
- Feasible to measure?
- How to measure?

Draft Monitoring and Evaluation Framework:
- Test with case studies using van Mierlo et al (2010)
- Refine and develop further with patients, pharma companies and HTA bodies
- Ensure return on PE for all stakeholders

Autumn 2020

Monitoring and Evaluation Framework: Complete and publish

References

Arksey H, O'Malley L 2005 Scoping studies: Towards a methodological framework. ***International Journal of Social Research Methodology*** 8(1): 19-32. doi: 10.1080/1364557032000119616

Braun V, Clarke V 2006 Using thematic analysis in psychology. ***Qualitative Research in Psychology*** 3(2): 77-101. doi: 10.1191/1478088706qp063oa

INVOLVE 2019 *INVOLVE: **National Institute for Health Research. Resource Centre*** https://www.invo.org.uk/resource-centre/libraries/evidence-library/ accessed 21/01/2019

PARADIGM 2018 ***Patients Active in Research and Dialogues for an Improved Generation of Medicines.*** Available at: https://imi-paradigm.eu/our-work/. Accessed 21/01/2019

PCORI 2019 ***Patient Centered Outcomes Research Institute: Engagement in health research literature explorer.*** https://www.pcori.org/literature/engagement-literature accessed 21/01/2019

Van Mierlo BC, Regeer B, van Amstel M, et al 2010 ***Reflexive Monitoring in Action: A Guide for Monitoring System Innovation Projects.*** Communication and Innovation Studies, WUR; Athena Institute, VU.