

Patient-Based Evidence in Mechanism-Based Drug Repurposing: Barriers and Facilitators

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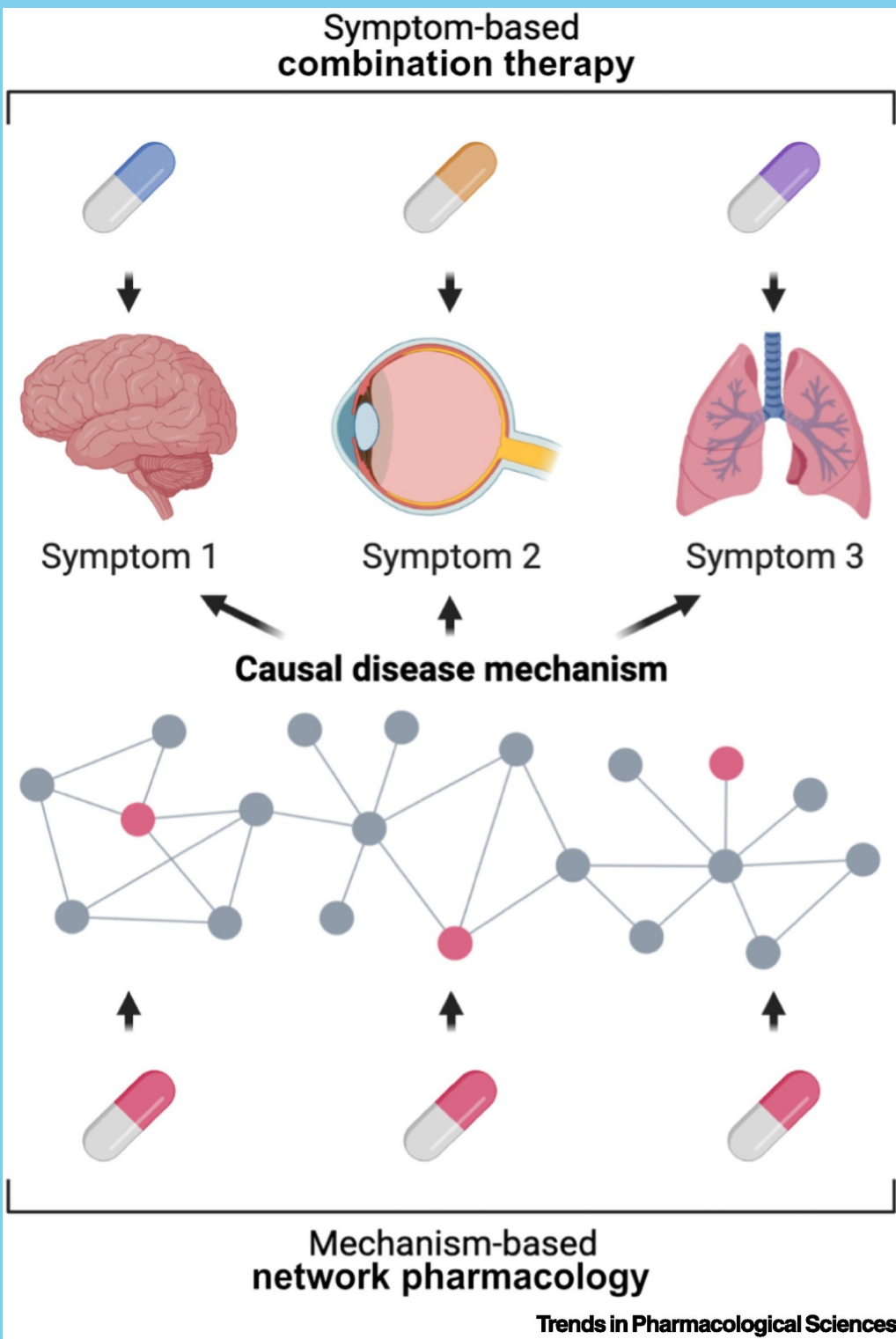
Merel Kimman, Manuela Joore, Sabine Grimm
Department of Clinical Epidemiology and Medical Technology Assessment, Maastricht University Medical Centre+
Contact: merel.kimman@mumc.nl



Background & Aim

- Mechanism-based drug repurposing finds new uses for existing drugs by focusing on disease mechanisms rather than on symptoms.
- Patients’ perspectives, captured via patient-reported outcome measures (PROMs) and qualitative interviews, are essential to assess a drug’s impact on health and quality of life.
- However, this patient-based evidence (PBE) has played a limited role in (mechanism-based) drug repurposing studies.
- **This study identified barriers and facilitators for using PBE in mechanism-based drug repurposing studies.**

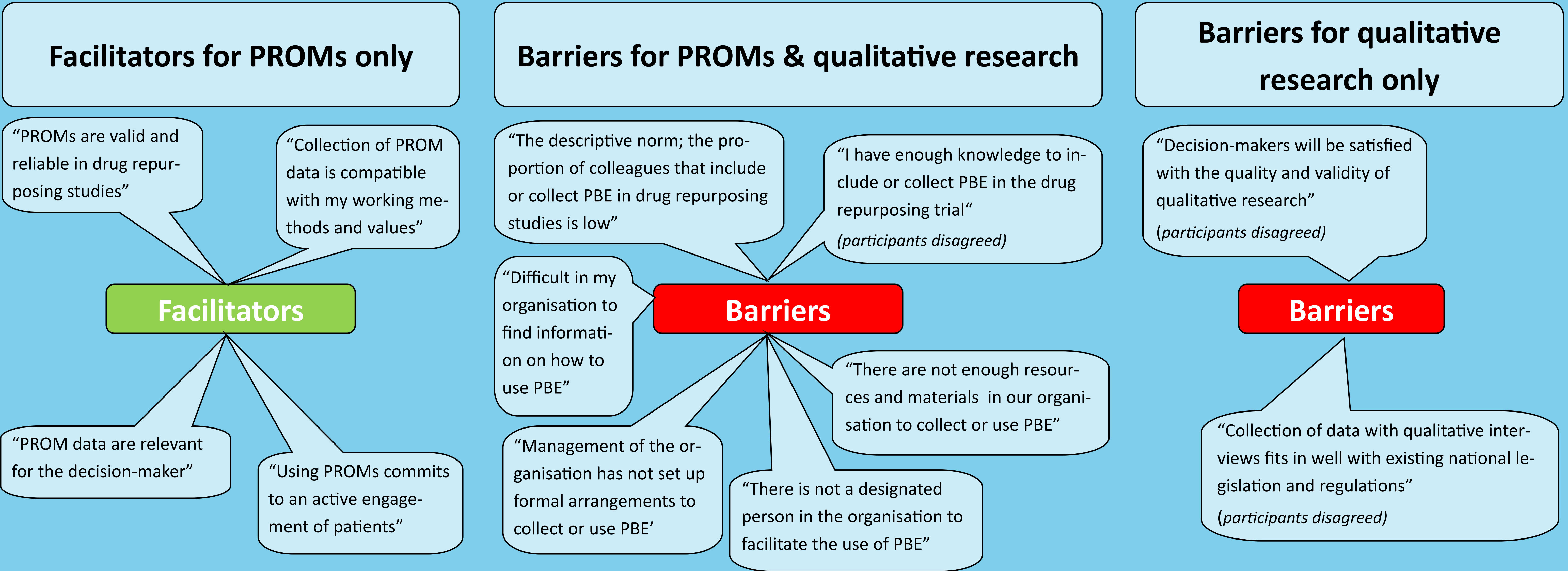
Mechanism-based drug repurposing



Methods

- Survey: **the Measurement Instrument for Determinants of Innovations (MIDI)**: 21 determinants associated with the innovation (i.e. PBE), the user, the organization, and socio-political context.
 - Statements for PROMs and qualitative research
 - Two open questions
- Participants: pharmaceutical companies (n=4), small to medium enterprises (n=1), charities or funders (n=2), patient representatives (n=2), clinical investigators (n=1), academic organisations (n=5), government institutions (n=2), and regulators (n=1).
- Analysis: If $\geq 20\%$ of participants (totally) disagree, the determinant is a **barrier**; if $\geq 80\%$ (totally) agree, the determinant is a **facilitator**.

Results MIDI-survey



Results open questions

- Data collection on patient perspectives should start early and is valuable in all stages of the development of a (repurposed) drug.
- Evidence of a mechanism being ‘treated’ may be more distant to a direct patient’s response as opposed to symptom-based research; available PROMs may not adequately capture treatment effect.
- Identifying or developing *specific* PROMs is challenging.
- Qualitative research and *generic* PROMs may be well-suited to capture intended and unintended effects of the mechanism-based drug.
- Qualitative research may be especially valuable during the follow-up of small clinical trials to fully capture the impact of the drug or sequence of drugs.

Conclusions

- While stakeholders agreed that PBE is essential to assess the value of a repurposed drug, the MIDI-survey revealed more barriers than facilitators for the use of PBE in (mechanism-based) drug repurposing studies.
- PROMs are an accepted methodology to capture patients’ perspectives, but the value of qualitative research is not yet widely acknowledged.
- Evaluation of a mechanism-based drug instead of a symptom-based drug poses additional challenges for using PBE.
- Accessing information and resources needed for PBE collection is an obstacle for all stakeholders.
- **Awareness, education, organizational facilitation, and standardization of PBE collection are needed for its effective use in mechanism-based drug repurposing.**

Do you have experience with (mechanism-based) drug repurposing? You can still participate in this research. Please scan the QR-code.

References

Figure: C. Nogales et al. Network pharmacology: curing causal mechanisms instead of treating symptoms. Trends in Pharmacological Sciences, Volume 43, Issue 2, 136 – 150, February 2022

MIDI: M. Fleuren et al. Towards a measurement instrument for determinants of innovations. International Journal for Quality in Health Care, Volume 26, Issue 5, 501– 10, October 2014