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## Merel Kimman, Manuela Joore, Sabine Grimm

Department of Clinical Epidemiology and Medical Technology Assessment, Maastricht University Medical Centre+

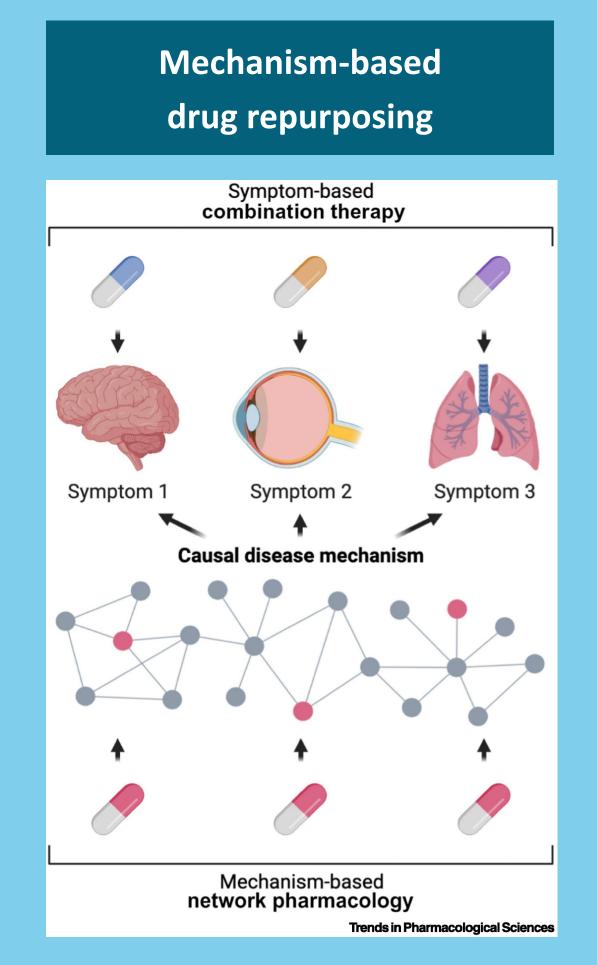
Contact: merel.kimman@mumc.nl





## 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.



## 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 ≥20% of participants (totally) disagree, the determinant is a barrier; if ≥80% (totally) agree, the determinant is a facilitator.

## Results MIDI-survey

## **Facilitators for PROMs only**

"PROMs are valid and reliable in drug repurposing studies"

"Collection of PROM data is compatible with my working methods and values"

## **Facilitators**

"PROM data are relevant for the decision-maker"

"Using PROMs commits to an active engage-ment of patients"

## Barriers for PROMs & qualitative research

"The descriptive norm; the proportion of colleagues that include or collect PBE in drug repurposing studies is low"

"Difficult in my organisation to find information on how to use PBE"

"Management of the organisation has not set up formal arrangements to collect or use PBE'

"I have enough knowledge to include or collect PBE in the drug repurposing trial"

(participants disagreed)

## Barriers

"There are not enough resources and materials in our organisation to collect or use PBE"

"There is not a designated person in the organisation to facilitate the use of PBE"

# Barriers for qualitative research only

"Decision-makers will be satisfied with the quality and validity of qualitative research"

(participants disagreed)

## Barriers

"Collection of data with qualitative interviews fits in well with existing national legislation and regulations"

(participants disagreed)

## 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

