

KU LEUVEN



# Are Patients' Perspectives Implemented in Clinical Trial Designs? A Literature Review and Semi-Structured Interviews to Explore Current Practices, Barriers, and Improvement Opportunities

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## Introduction

#### Patient experience data (PED)

Patientreported outcomes

Patientreported experiences

Patient preferences

Patient input



PED increasingly recognised by stakeholders as an important source of information that should be considered when making decisions



Challenges hamper the systematic implementation of PED in decision-making, including in clinical trials

# **Objectives**

This study aims to investigate current practices, barriers, and improvement opportunities regarding the implementation of patients' perspectives into clinical trial designs.

## Methods

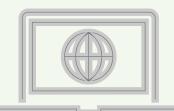
## Literature review



Literature review with systematic search string in

- PubMed
- Embase

for reviews and guidelines on PED implementation in clinical trial design



Grey literature review of key regulatory and stakeholder guidance documents

#### **Qualitative interviews**



Purposive sampling



Semi-structured interviews with key stakeholders, including physicians from Belgian hospitals (n=10)



Ad verbatim transcription



\* Based on preliminary insights

Thematic framework analysis

#### Results\*

## **BARRIERS**

- Lack of guidelines and standardized methodologies
- Subjectivity of patient-reported outcome (PRO) data
- Cost and time constraints for collecting PED
- Patient burden and survey fatigue
- Representativeness of patient sample
- Lack of scientific knowledge of patients when giving input

## **OPPORTUNITIES**

- Development of disease-specific guidelines and PRO measures, and creation of core outcome sets
- Validation of patient-informed and patient-relevant endpoints
- Methodological guidance on patient-reported experience (PRE) measures and patient preferences (PP)
- Increased use of direct patient input

## FACILITATION OF MORE EFFECTIVE PED IMPLEMENTATION THROUGHOUT THE DRUG LIFE CYCLE

Pre-clinical drug development

Clinical drug development

Market access and reimbursement

Clinical practice

Define unmet needs to guide research agenda

Understand impact of disease and its treatment on patients

Indicate trade-offs to inform benefit-risk assessment

Inform the development of guidelines and standards

Define research questions

Inform trial study protocol, including design, recruitment and feasibility

Help resource allocation by considering patients' needs and preferences

Inform appropriate use of secondary data

Help develop clear and concise study materials Help report back study results to patients

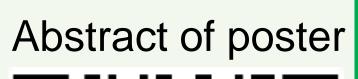
Inform relevant endpoints

## **Abbreviations**

PED: Patient Experience Data, PRO: Patient Reported Outcome, PRE: Patient Reported Experience, PP: Patient Preferences

# Acknowledgements

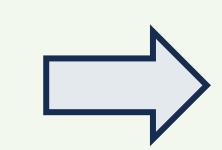
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# Conclusion

- Development of standards and disease-specific best practices
- Stimulate early involvement of patients in clinical trial designs
- Multi-stakeholder collaboration



Tailor decision-making to meet patients' needs and preferences for a more patient-centred healthcare system