# Patients' voice matter when determining **outcomes** to measure in trials and evidence synthesis

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EUGMS Congress 2023, submitted symposium – Helsinki – 21 September

# Conflict of interest

- No conflict of interest to disclose

# PPI for determining outcomes in trials

- Why for?
- How frequent is it?
- What is the impact?
- What are the challenges?

Illustration: PPI for selecting outcomes in medicines optimisation trials

# Terminology

## Outcome

- A measurement or observation used to assess the impact of an intervention or exposure, including both harms and benefits.

## Core outcome set (COS)

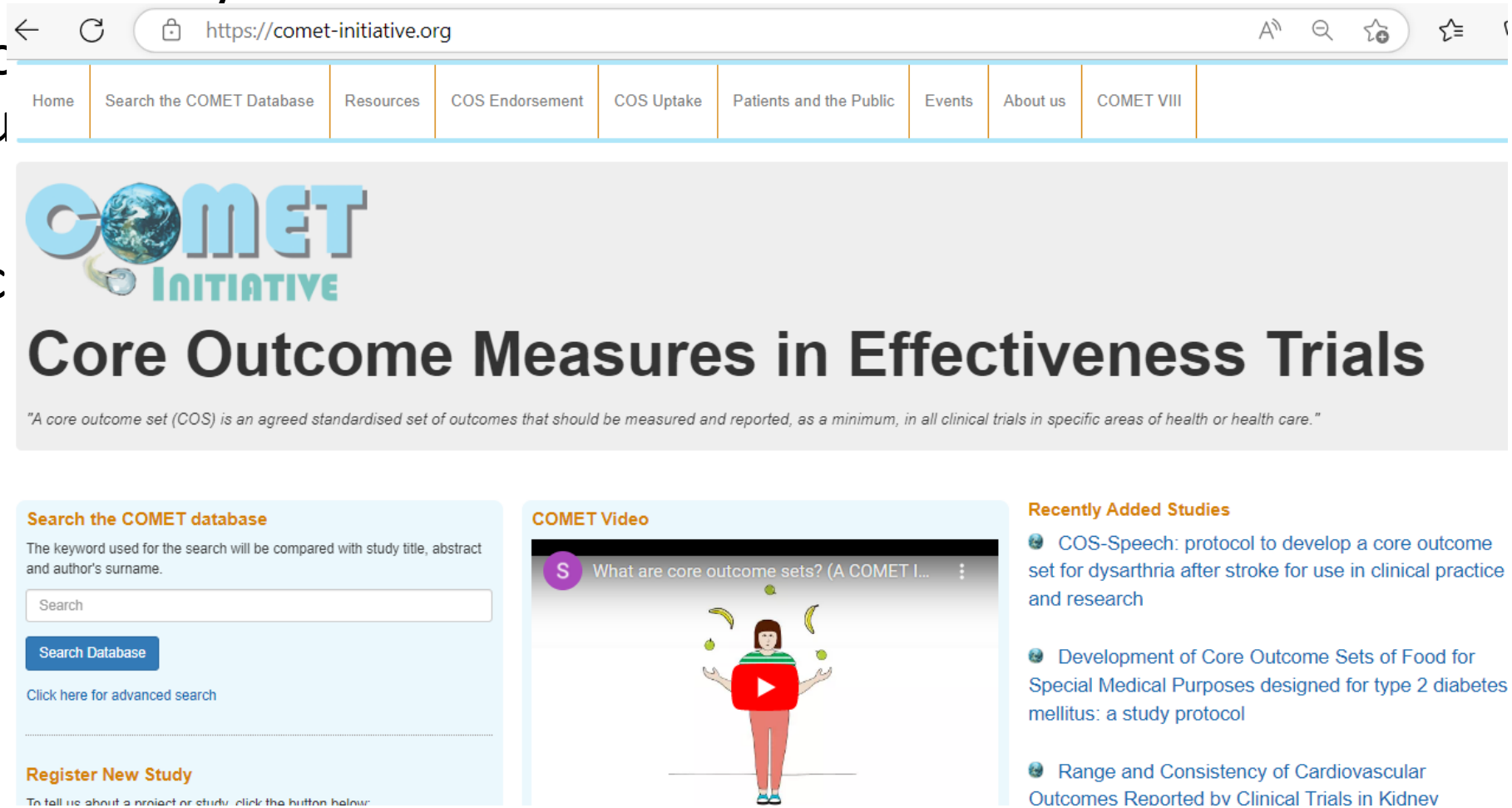
- An agreed standardised set of outcomes that should be measured and reported, as a minimum, in all clinical trials in specific areas of health or health care

Williamson et al. Trials 2012

# Core outcome sets

## Benefits

- Increases consistency across trials
- Maximise pc
  these key ou
- Much more
- Major reduc



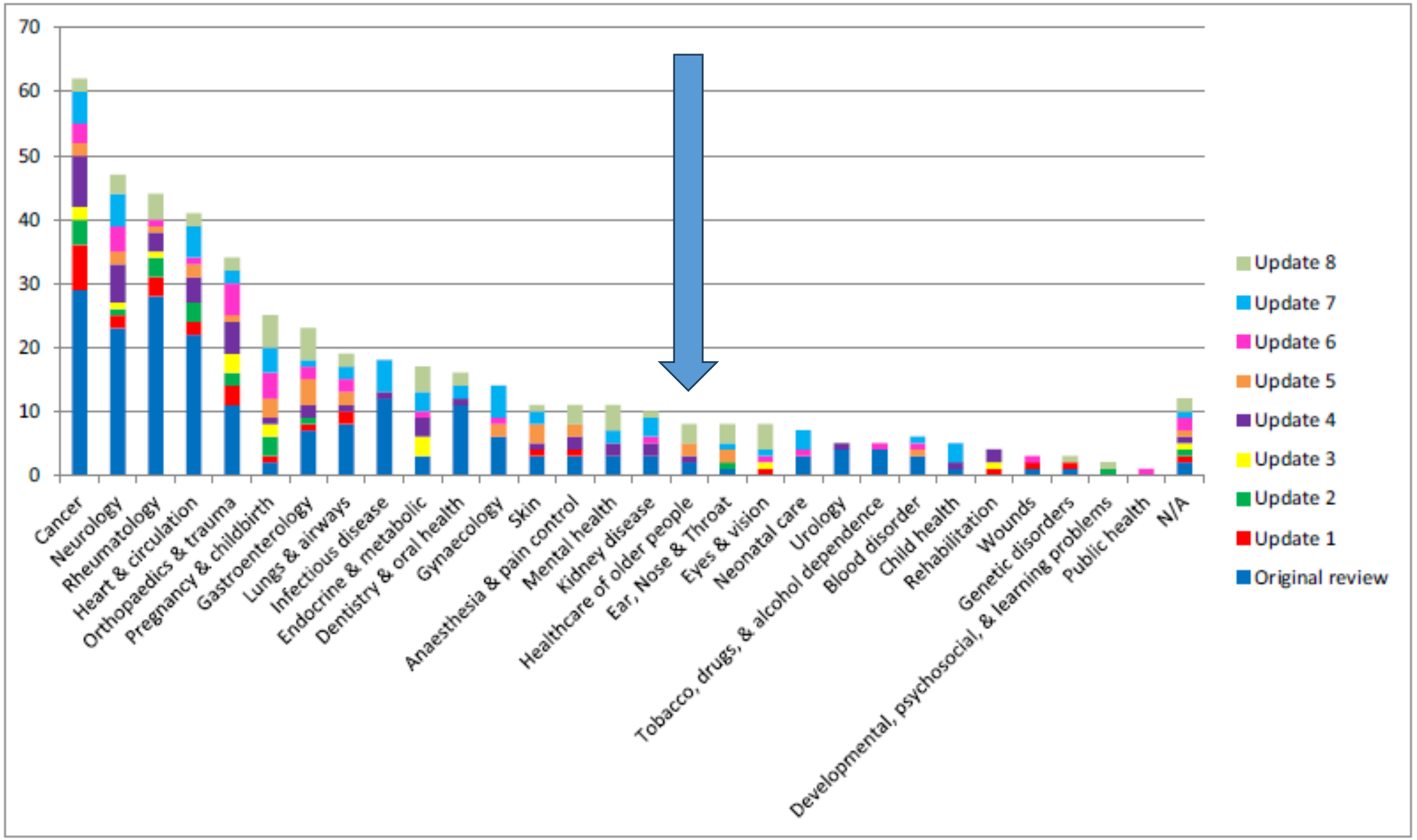
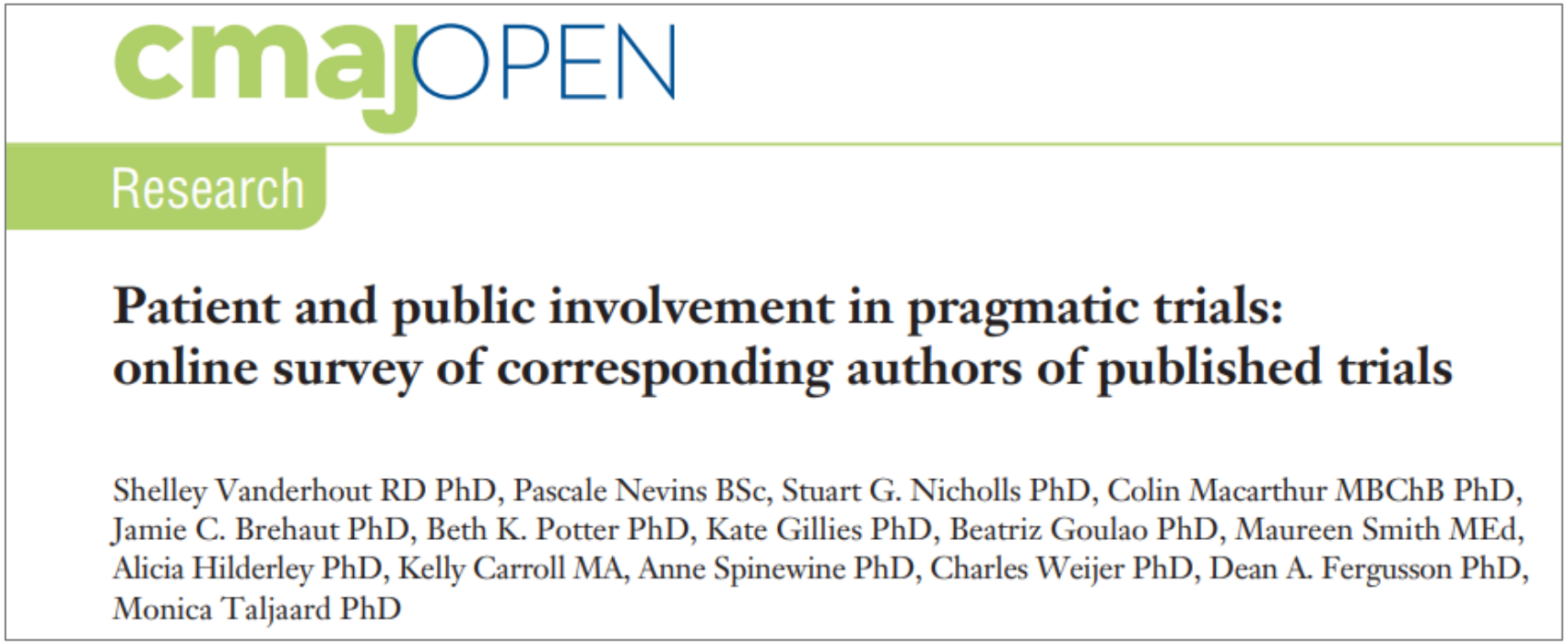



**Fig. 1.** COS disease categories ($n$ = 480).

# PPI for selecting outcomes: why for?

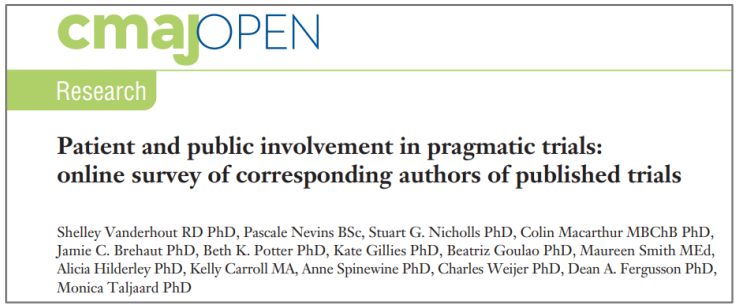
- Importance of consulting patient stakeholders, as their perspectives differ from those of clinicians
- Patients tend to assess outcomes in the context of their own life, whereas clinicians focus on their patients' physiological or health status
- Pragmatic trials: trials that choose outcomes of obvious importance to patients
- Selecting outcomes that are relevant for older people are important for overcoming the recognized age discrimination in clinical trials

# How frequent?




- Pragmatic trials published from 2014 to 2019
- 710 authors (27.5%) reported on 710 unique trials and completed the survey

CMAJ Open 2023 September 19. DOI:10.9778/cmajo.20220198


cmajOPEN

Research

**Patient and public involvement in pragmatic trials: online survey of corresponding authors of published trials**

Shelley Vanderhout RD PhD, Pascale Nevins BSc, Stuart G. Nicholls PhD, Colin Macarthur MBChB PhD, Jamie C. Brehaut PhD, Beth K. Potter PhD, Kate Gillies PhD, Beatriz Goulao PhD, Maureen Smith MEd, Alicia Hilderley PhD, Kelly Carroll MA, Anne Spinewine PhD, Charles Weijer PhD, Dean A. Fergusson PhD, Monica Taljaard PhD

- 710 trials
- Authors reporting PPI: 47%
- Specific aspects of the study where partners were engaged:
  - Developing interventions 71%
  - (…)
  - Selecting outcomes 27%

Vanderhout et al. CMAJ Open 2023 September 19.

- 96 trials with older adults
- 48%
  - 86%
  - (…)
  - 36%

In preparation.

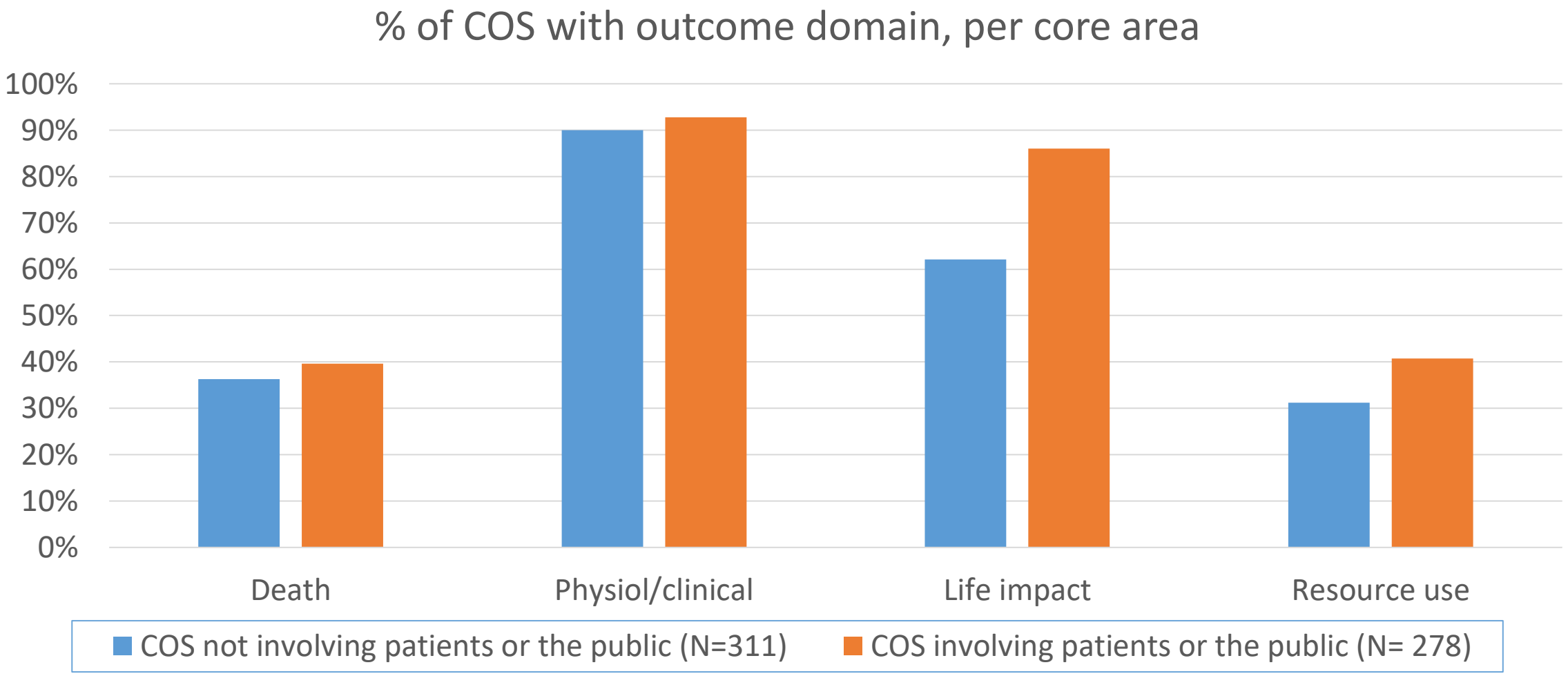
# Developing COS

- PPI: patients help researchers to design, oversee and/or disseminate the COS study
- Patient participation: patients share their views about outcomes and how they consider them to be

- Updated systematic review: 110 COS published in 2020-2021:
- Inclusion of public participants is increasing
- Patients or other members of the public involved in: 82%

Dodd et al. J Clin Epidemiol. 2023;158:127-133

# Patient participation impacts outcome selection in COS




Dodd et al. J Clin Epidemiol. 2023;158:127-133

# Challenges

- Language: clear, engaging and accessible for all
- Accessing patients / the public
- Methods of involving patients
- Making sure that patients are at ease when mixing with other stakeholders
- Maintaining patient involvement

https://www.comet-initiative.org/

# Ressources to help

- Researchers involving patients and the public
  - Articles, recorded presentations & webinars
  - Consensus meetings
  - Preparing COS information for patients
  - Plain language resources
  - Social media
  - Patient and Public Involvement
  - Ethics

https://comet-initiative.org

**COMET Patient and Public Involvement Toolkit**

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

COMET People and Patient Participation, Involvement and Engagement (PoPPIE) working group

https://comet-initiative.org/Patients/POPPIE

# Illustration

# Core Outcome Sets in medicines optimisation

OPERAM

RESEARCH ARTICLE Open Access

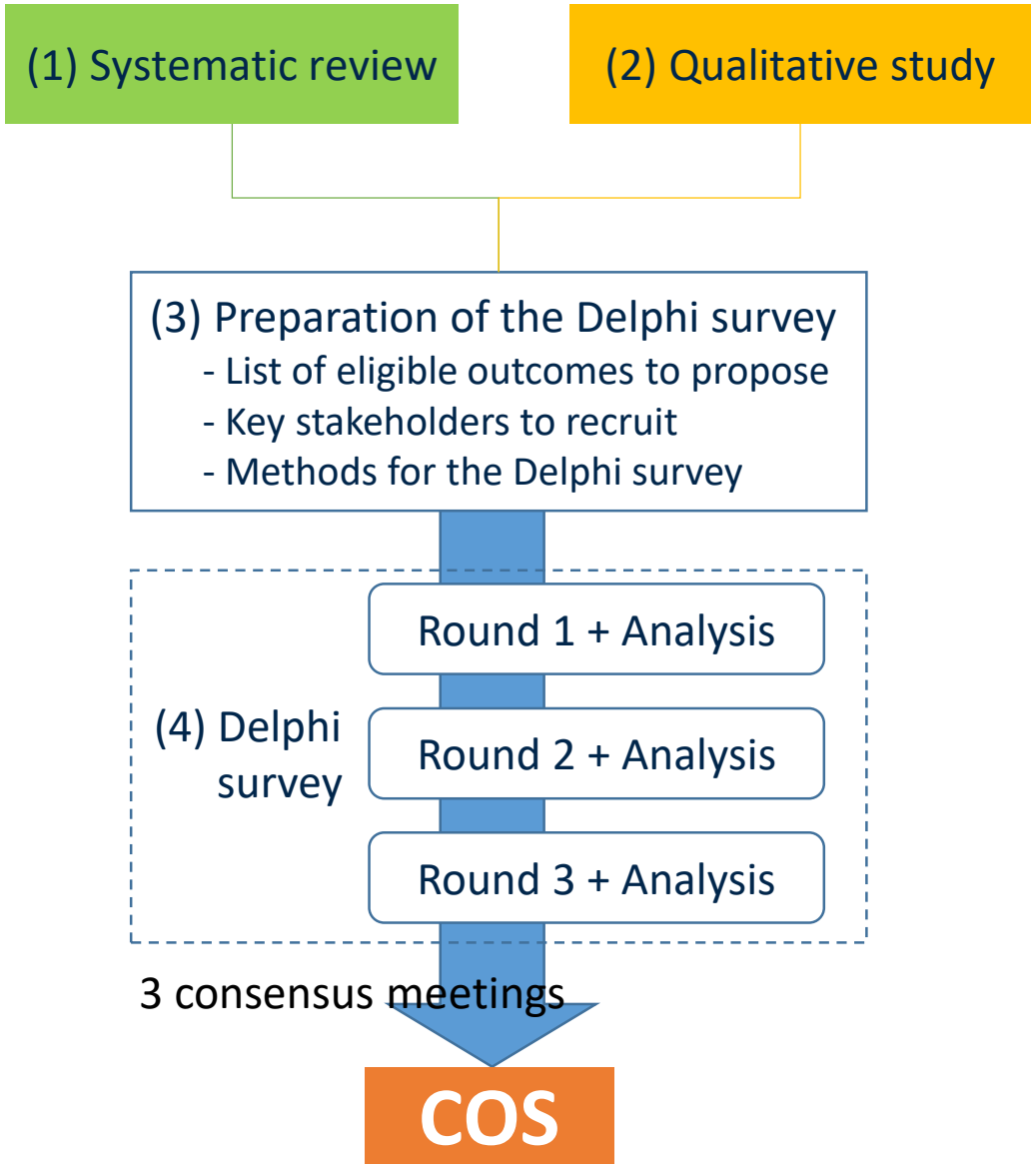
# International core outcome set for clinical trials of medication review in multi-morbid older patients with polypharmacy

Jean-Baptiste Beuscart[1,2*], Wilma Knol[3], Shane Cullinan[4,5], Claudio Schneider[6], Olivia Dalleur[1,7], Benoit Boland[8], Stefanie Thevelin[1], Paul A. F. Jansen[3], Denis O'Mahony[9], Nicolas Rodondi[6,10] and Anne Spinewine[1,11]

Beuscart et al. BMC Medicine (2018) 16:21

This project has received funding from the European Union's Horizon 2020 research and innovation programme under the grant agreement No 634238, and is supported by the Swiss State Secretariat for Education, Research and Innovation (SERI) under contract number 15.0137

**Scope**: Medication review in older patients (≥ 65 years) with polypharmacy and multimorbidity




Beuscart et al. BMC Medicine (2018) 16:21

| Domain | Outcome |
|---|---|
| Adverse events | Drug-related hospital admissions |
| Medication use | Overuse |
| | Underuse |
| | Potentially inappropriate medications |
| | Clinically significant DDI |
| Patient-reported outcomes | Health-related quality of life |
| | Pain relief |

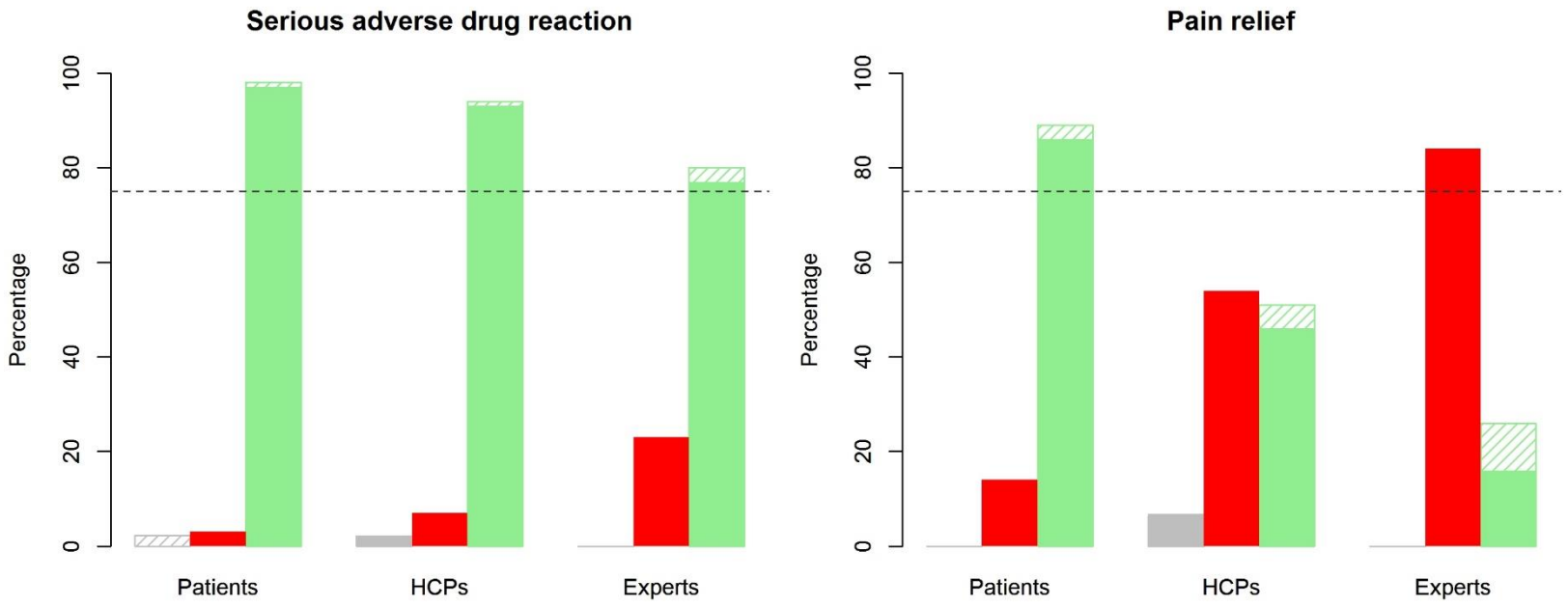
# COS: important focus on patient participation

- Interviews: 15 patients
- Delphi rounds: 55 patients out of 150 participants in total (4 countries); 45% aged 80 and over
- Consensus meeting: 6 patients

- Specificities / adjustments made in the protocol to maximise participation and retention

Beuscart et al. BMC Medicine (2018) 16:21

# Patient participation impacted the final set




- % YES in the full analysis
- % YES in the sensitivity analysis
- % NO in the sensitivity analysis
- % “Unable to score” in the full analysis
- % “Unable to score” in the sensitivity analysis

# Other COS in medicines optimisation

Trials Aimed at Improving the Appropriateness of Polypharmacy in Older People in Primary Care. Rankin et al., JAGS 2018.

- 41 public participants in the Delphi
- Some differences in scoring between the public and expert panels
- 'Patient knowledge' would not have been included in the final COS if only experts had been included.

Optimising prescribing in older adults in care homes. Millar et al., Trials 2017.

Hospital deprescribing trials for older people under the care of a geriatrician. Martin-Kerry et al., Age and Ageing 2022.

- 18 older people and carers participated
- Impact on the COS in several aspects: outcomes felt not important or acceptable to collect (e.g. ADL, burden from medicine)

# PPI for determining outcomes in guideline development

# PPI in guideline development

- International consensus on the importance of PPI in guideline development
- Contribution:
  - assessing guideline priorities, identifying key populations, identifying outcomes, informing whether findings are meaningful, prompting holistic approaches to care, assessing how recommendations interact with patient values, and writing plain-language guideline versions
- Strategies for PPI in guideline development
  - Participation: Patients/consumers join with the guideline development group as members
  - Consultation: to obtain views from a large number of individuals
  - Communication: as part of the dissemination and implementation strategies
- Substantial gap between PPI standards in guideline development and current practice

Armstrong and Bloom Research Involvement and Engagement (2017) 3:19; Bryant EA, et al. BMJ Open 2022;12:e055428

RESEARCH Open Access

# Enhancing patient and public contribution in health outcome selection during clinical guideline development: an ethnographic study

Alice M. Biggane[1*], Bridget Young[2], Paula R. Williamson[3,4], Erin Whittingham[5] and Jessie Cooper[6]

- PPI contributors' input in the guideline development process: often of limited scope, particularly in selecting health outcomes.
- Key constraints : technical content and language, assumed differences in the health-related priorities between PPI contributors and health professionals, linear timeline of the guideline development process.
- However, PPI contributors can influence the selection of relevant health outcomes.
- Important: role of the committee chair, training and support for all committee members, use of plain language.

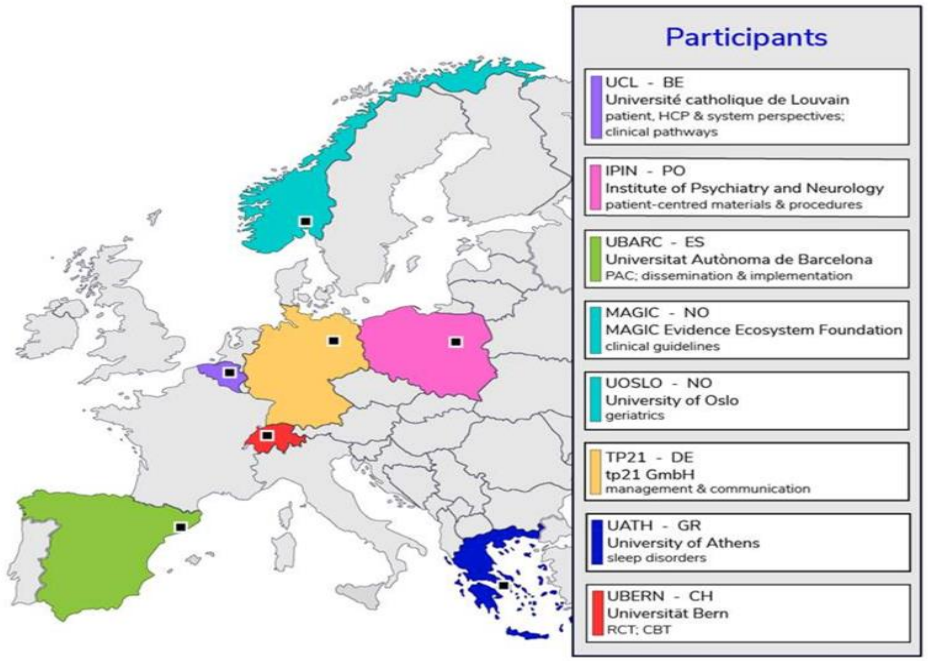
Biggane *et al. BMC Health Services Research (2022) 22:361*

# Illustration

# BE-SAFE

Implementing a patient-centred and evidence-based intervention
to reduce BEnzodiazepine and sedative-hypnotic (BSH) use
to improve patient SAFEty and quality of care

09/2022 – 08/2027

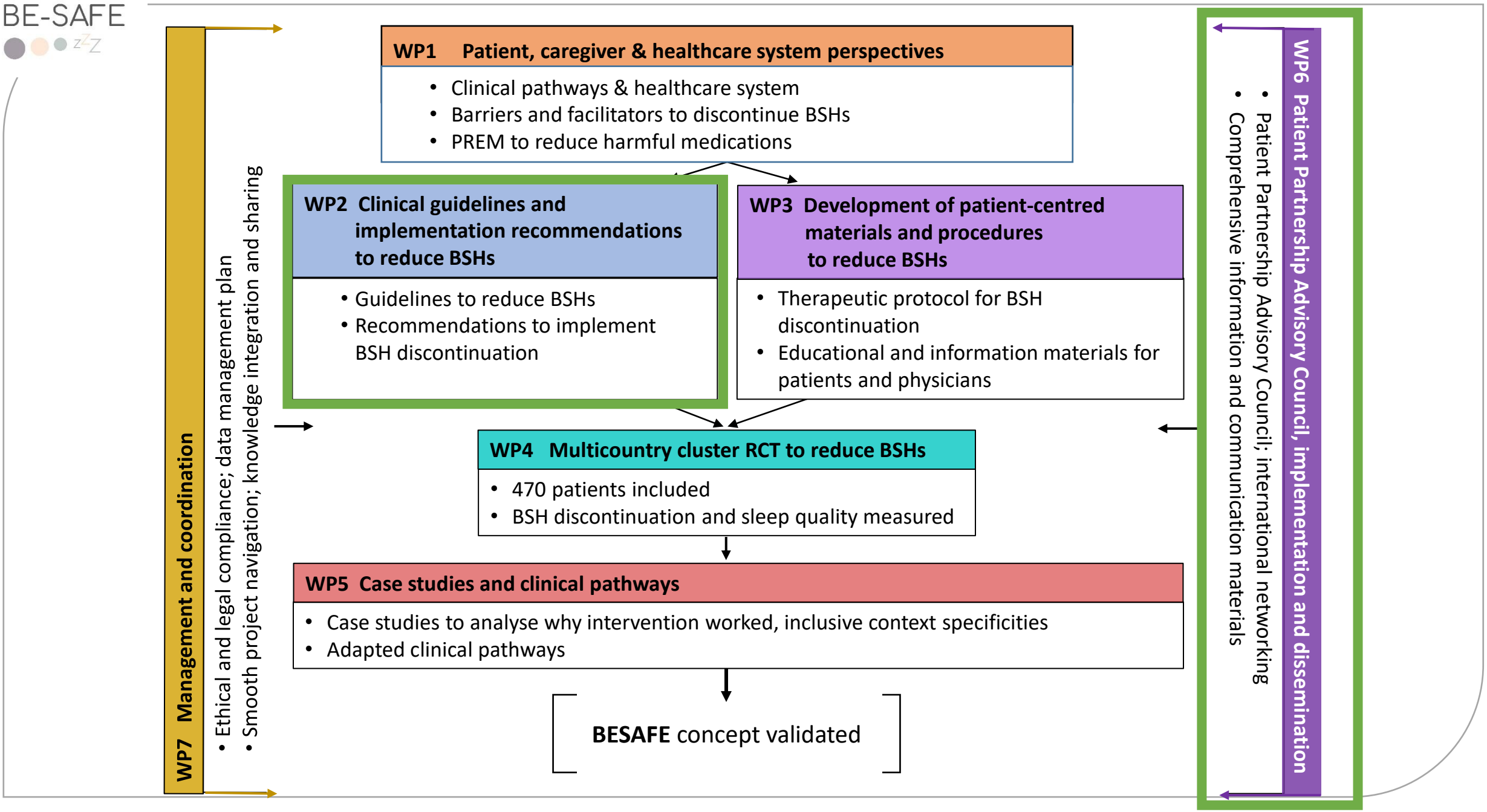



+ 2 network partners: J Grimshaw (OHRI)W Levinson (UTOR)

Goal: to improve patient safety by addressing knowledge and practice gaps related to the reduction of BSHs used for sleep difficulties

"BE-SAFE" is supported by the European Union's Horizon Europe research and innovation programme under the grant agreement No 101057123, and by the Swiss State Secretariat for Education, Research and Innovation (SERI) (contract No 22.00116).

BE-SAFE


WP1 Patient, caregiver & healthcare system perspectives
• Clinical pathways & healthcare system
• Barriers and facilitators to discontinue BSHs
• PREM to reduce harmful medications
WP2 Clinical guidelines and implementation recommendations to reduce BSHs
• Guidelines to reduce BSHs
• Recommendations to implement BSH discontinuation
WP3 Development of patient-centred materials and procedures to reduce BSHs
• Therapeutic protocol for BSH discontinuation
• Educational and information materials for patients and physicians
WP4 Multicountry cluster RCT to reduce BSHs
• 470 patients included
• BSH discontinuation and sleep quality measured
WP5 Case studies and clinical pathways
• Case studies to analyse why intervention worked, inclusive context specificities
• Adapted clinical pathways
BESAFE concept validated
WP7 Management and coordination
• Ethical and legal compliance; data management plan
• Smooth project navigation; knowledge integration and sharing
WP6 Patient Partnership Advisory Council, implementation and dissemination
• Patient Partnership Advisory Council; international networking
• Comprehensive information and communication materials

MAGIC Evidence Ecosystem Foundation

https://magicevidence.org/

GRADE approach

Evidence to Decision framework

## Improving patient care through trustworthy guidelines, evidence summaries, policy and decision aids

MAGIC is a non-profit. Our vision is to increase value and reduce waste in healthcare through a digital and trustworthy evidence ecosystem. MAGICapp is our core platform, bringing digitally structured and user-friendly guidelines, evidence summaries and decision aids to clinicians and patients.

BMJ RapidRec

- We will include relevant end-users including front-line clinicians, allied healthcare workers, and patient representatives as full panel members with the ability to influence the process from the choice of outcomes considered to writing the recommendations
- Guidelines will be peer reviewed by a similar spectrum of people, including patients

- Each panel will include patient representatives and will list all considerations important to them at the outset
- Panels will systematically consider and report literature on patient values and preferences

Siemieniuk et al., BMJ 2016;354:i5191

app.magicapp.org/#/guideline/7507

BE-SAFE

BE-SAFE: Deprescription of benzodiazepine and sedative hypnotics (BSHs) in insomnia disorder

v5.1 published on 30/08/2023

Table des matières



# 1 Guideline overview

This guideline is developed as a part of the **BE-SAFE project**, funded by the research and innovation programme and the Swiss State Secretariat for Educ (SERI)

Plus >

## 4.1 Panel members

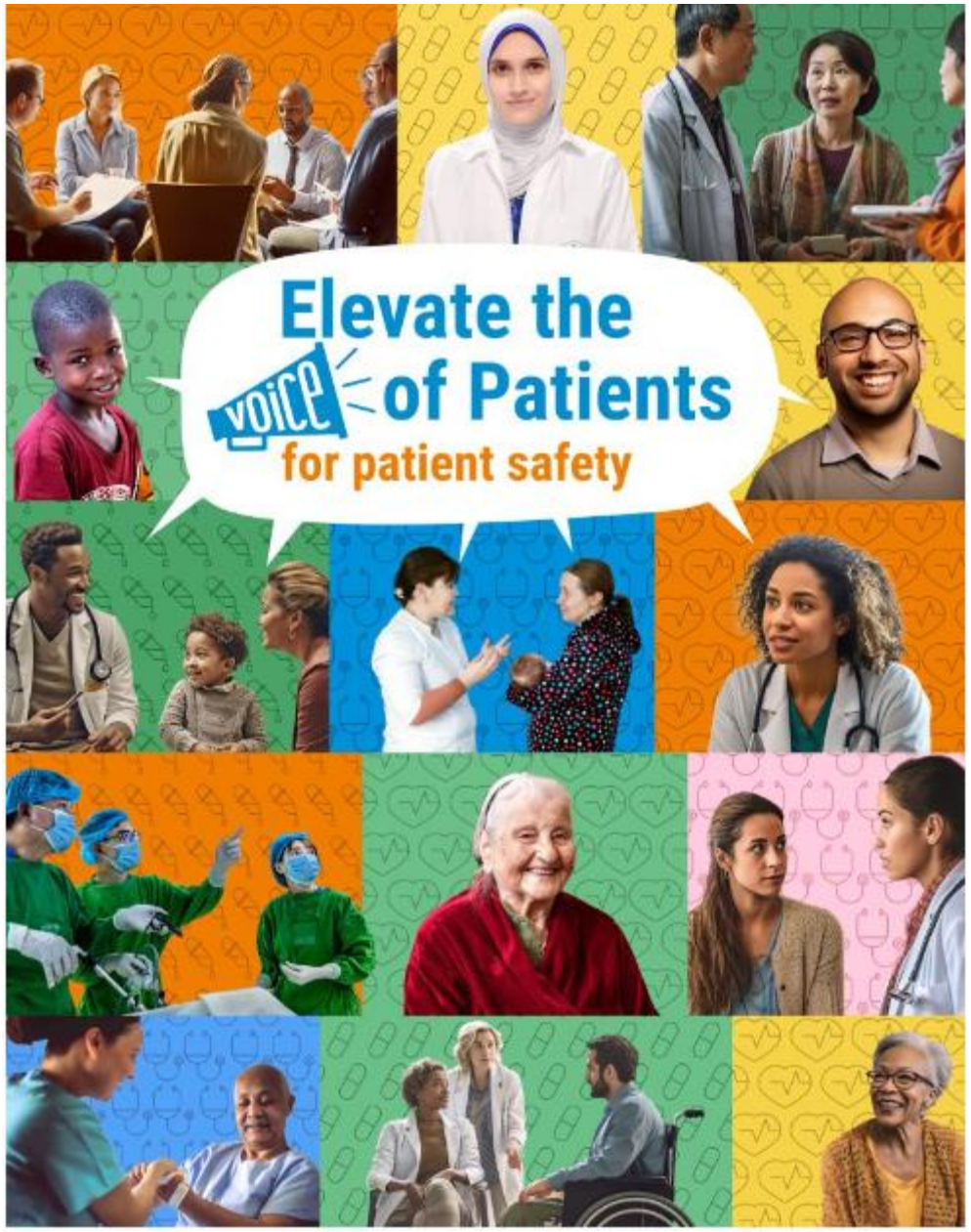
The BE-SAFE BMJ RR panel consists of 23 members. There are two clinical co-chairs (one female and one male), one methods co-chair (male), one methods co-chair trainee (male), two systematic reviewers (one female and one male), two senior methodologists (males), one implementation lead (male), one implementation trainee (female), eight clinical experts (two females and six males), one social worker (female), one epidemiologist (male) and three patient partners (two females and one male).

# WP6 Patient Partnership Advisory Council (PAC)

Lead: Fundació Salut i Envelliment UAB, Barcelona, Spain

- 6 local PACs in 6 European countries
- 1 international PAC

- Year 1
  - Training provided to PAC members; to partners
  - Involvement in initial activities (intervention development, trial protocol and informed consent,…)




Online event

2023

# Patients Involvement in Quality and Safety Research

**26 SEPTEMBER**
From 16:00 to 18:00 CET

## Learning objectives:

1. Share the benefits achieved in the involvement of patients in patient safety research in the current course of the projects.
2. Discuss the methodological challenges being presented and the actions to face them.
3. Promote recommendations at different levels to encourage and make the involvement of patients more effective.

## Program

| | |
|---|---|
| 16:00 - 16:15<br>Welcome EU Officer | 16:45 - 17:00<br>SAFEPOLYMED project<br>www.safepolymed.eu |
| 16:15 - 16:30<br>SAFEST project<br>www.safestsurgery.eu | 17:00 - 17:15<br>DELIVER project<br>www.deliverproject.eu |
| 16:30 - 16:45<br>BESAFE project<br>www.besafe-horizon.eu | 17:15 - 17:30<br>Patients representative voice |
| 17:30 - 17:45<br>Questions and Answers (Discussion) | |
| 17:45 - 18:00<br>Closing and End of the Event | |

## How to register

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