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Improving healthcare decisions

Integrating the Patient Voice into Economic Assessments of Health Technologies

Nan Qiao, PhD
Chair, ISPOR Patient-Centered Special Interest Group

November 20, 2024

Panelists

- **Anke-Peggy Holtorf, PhD**

Managing Director, Health Outcomes Strategies GmbH, Basel, Switzerland;
@HTAi.org: Secretary of the Board + Steering Committee Member and Project Coordinator for the Patient and Citizen Involvement in HTA Interest Group (PCIG)

- **Andrew Briggs, DPhil**

Professor of Health Economics, Department of Health Services Research & Policy, London School of Hygiene & Tropical Medicine, London, United Kingdom

- **Bettina Ryll, MD/PhD**

Founder of the Melanoma Patient Network Europe, Uppsala, Sweden; Strategist, Vision Zero Cancer

COI Disclosure and Disclaimer

- **Nan Qiao** is a full-time employee of Merck Sharp & Dohme. Her presentation reflects her personal views.
- **Anke-Peggy Holtorf** is Managing Director of Health Outcomes Strategies, Secretary of the Board of HTAi; Steering committee member and project coordinator of PCIG @ HTAi.

Polling Question 1

Why are you here today? Pick your top reason!

- a) Love the topic
- b) Fan of the panelists
- c) Member of Patient-Centered SIG
- d) Other

Polling Question 2

What does “*integrating the patient voice*” mean to you in this context?

- a) QALY as a health outcome measure
- b) A societal perspective
- c) PED and patient communication
- d) Patients as research partners
- e) Select patient-relevant inputs and outcomes and incorporate patient values in decision-making

Objectives

To stimulate REAL DEBATE on the essence of incorporating the patient* voice into economic assessments of health technologies

- Assess the present state of integrating the patient voice in HTA and economic modeling, and recognize challenges and opportunities
- Identify best approaches for integrating the patient voice in health economic assessments
- Evaluate the impact of integrating the patient voice on economic assessment methods and outcomes, and consequently on healthcare decisions
- Explore additional potential roles for patients with the HTA process that could impact economic evaluation









*In this issue panel, patients encompass all types of representatives from a specific community of interest, including patients, carers/caregivers, family members, and other types of patient representatives^a

a. National Health Council. Who is the Patient?" Tool. <https://nationalhealthcouncil.org/who-is-the-patient-tool/>. Accessed on 10/7/2024

Economic Assessment of Health Technologies

The economic assessment plays a pivotal role within the HTAs of many countries, providing a systematic and comprehensive analysis of the economic implications associated with the introduction of new health technologies into healthcare systems.

CEA and BIA Requirements in 8 European Countries^a

	HAS 	IQWiG 	TLV 	NICE 	AIFA 	ZIN 	AOTMiT 	RedETS & ICP 
Perspective	Widest possible	Payer	Societal	NHS or societal if justified	NHS	Societal (indirect costs reported separately)	Public payer and patient	NHS and societal (rarely used)
CEA	Yes	Optional CBA	Yes	Yes	Yes	Yes	Yes	Not mandatory
BIA	Not mandatory but highly recommended	Yes	Not mandatory	Yes, BI to NHS	Yes	Yes	Yes, BI to payer	Yes, BI to NHS

By quantifying the economic implications of health technologies, economic assessments support pricing and reimbursement decision-making and priority setting, ensuring that scarce resources are allocated to interventions that offer the greatest overall value to patients, healthcare systems, and society.

a. Angelis, Lange, & Kanavos. Using health technology assessment to assess the value of new medicines: results of a systematic review and expert consultation across eight European countries. *Eur J Health Econ* 2018(19), 123–152. <https://doi.org/10.1007/s10198-017-0871-0>

Economic Assessment Post JCA Implementation^a

EU Level

(jointly done by the member states)

Clinical Assessment

- Health problems and currently used health technologies
- Description of health technology under assessment
- Relative clinical effectiveness
- Relative safety

National Level

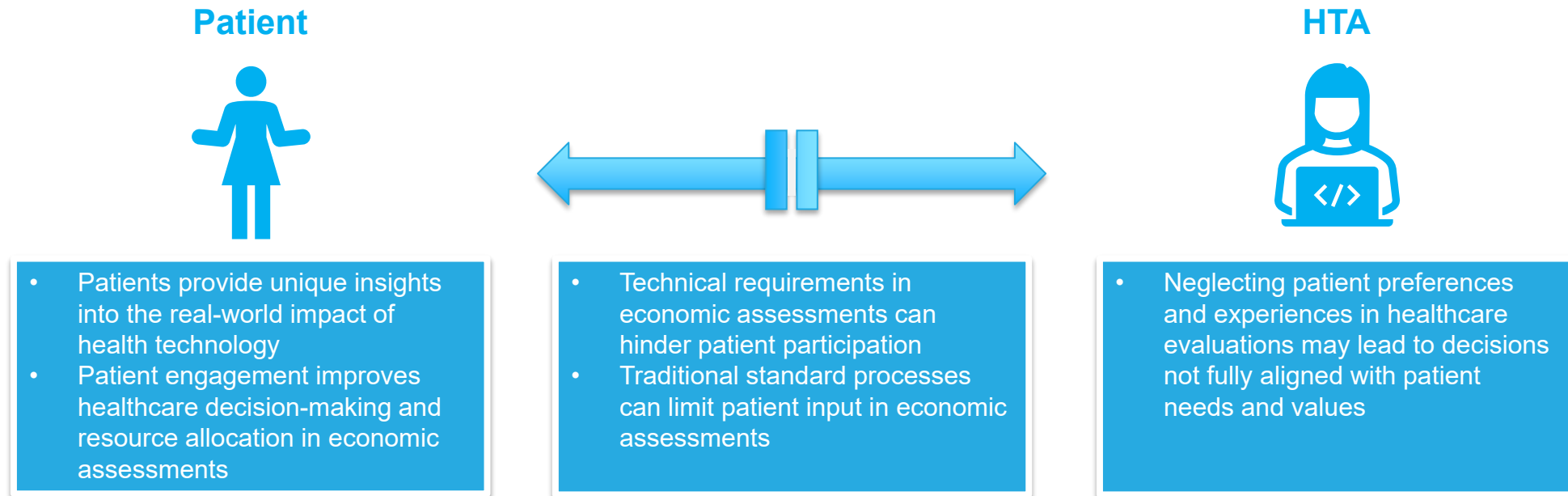
Non-Clinical Assessment

- **Economic evaluation**
- Ethical aspects
- Organizational aspects
- Social aspects
- Legal aspects

a. European Commission. IMPLEMENTING THE EU HEALTH TECHNOLOGY ASSESSMENT REGULATION. https://health.ec.europa.eu/document/download/84c1ec8f-9be3-4073-aceb-330764c93152_en?filename=hta_regulation-implementation_factsheet_en.pdf

Integrating the Patient Voice into Economic Assessments

In my opinion, **integrating the patient voice into economic assessments of health technologies** aligns with the ISPOR Patient-Centered SIG definition of “*patient engagement*”^a, emphasizing *the active, meaningful, and collaborative interaction with patients across all stages of the economic assessment process*.



a. Harrington, Hanna, Oehrlein, et al. Defining Patient Engagement in Research: Results of a Systematic Review and Analysis: Report of the ISPOR Patient-Centered Special Interest Group, Value in Health, 2020, 23(6), 677-688, <https://doi.org/10.1016/j.jval.2020.01.019>.

Examples of Patient Engagement in Economic Assessment

	Techniques to Include Carer Quality of Life in Economic Evaluation ^a	Early Economic Evaluation of CAR T-Cell Therapy for R/R B-Cell ALL ^{b,c}
Research type	Methodological	Applied
Patient partner	A LEAP (5 family caregivers with diverse experience)	An expert patient
Recruitment	Through mental health charity organizations and via a colleague	NR
Contributing areas	Focus group recruitment and interview, transcript coding, questionnaire design, Delphi study design, think-aloud interview, PTO design, dissemination	Setting the research question, determining the study design, informing the recruitment strategy/consent process/planned analysis
Partnership format	The LEAP and researchers met 12 times over 4 years, with meeting dates aligned with relevant work	Through several project meetings and with regular email correspondence
Impact	Created new recruitment and dissemination avenues, made surveys more accessible, ensured open and honest answers	<i>“Improved recruitment efforts,” “enhanced discussions with patients and caregivers,” “ensured reflective and representative analysis”</i>
Learnings	<i>“Lay participants and professional researchers need training and preparation,” “practical measures and soft skills are needed to ensure ongoing engagement,” “care needs to be taken to select appropriate research tasks for PPI”</i>	Additional time was needed to ensure the patient partner was comfortable to engage; attention was required to prevent an undue power dynamic between the patient partner and research participants

a. Al-Janabi, Coles, Copping, et al. Patient and Public Involvement (PPI) in Health Economics Methodology Research: Reflections and Recommendations. *Patient*. 2021 Jul;14(4):421-427. doi: 10.1007/s40271-020-00445-4. PMID: 32939688; PMCID: PMC7494378.

b. Wilson, Thavorn, Hawrysh, et al. Stakeholder engagement in economic evaluation: Protocol for using the nominal group technique to elicit patient, healthcare provider, and health system stakeholder input in the development of an early economic evaluation model of chimeric antigen receptor T-cell therapy. *BMJ Open* 2021;11:e046707. doi: 10.1136/bmjopen-2020-046707

c. Wilson, Thavorn, Hawrysh, et al. Engaging Patients and Caregivers in an Early Health Economic Evaluation: Discerning Treatment Value Based on Lived Experience. *PharmacoEconomics* 40, 1119–1130 (2022). <https://doi.org/10.1007/s40273-022-01180-4>

- +
 - Patient Involvement in HTA and Health Economic Analysis

Anke-Peggy Holtorf, Health Outcomes Strategies GmbH, Basel
Patient and Citizen Involvement in HTA Interest Group @ HTAi.org



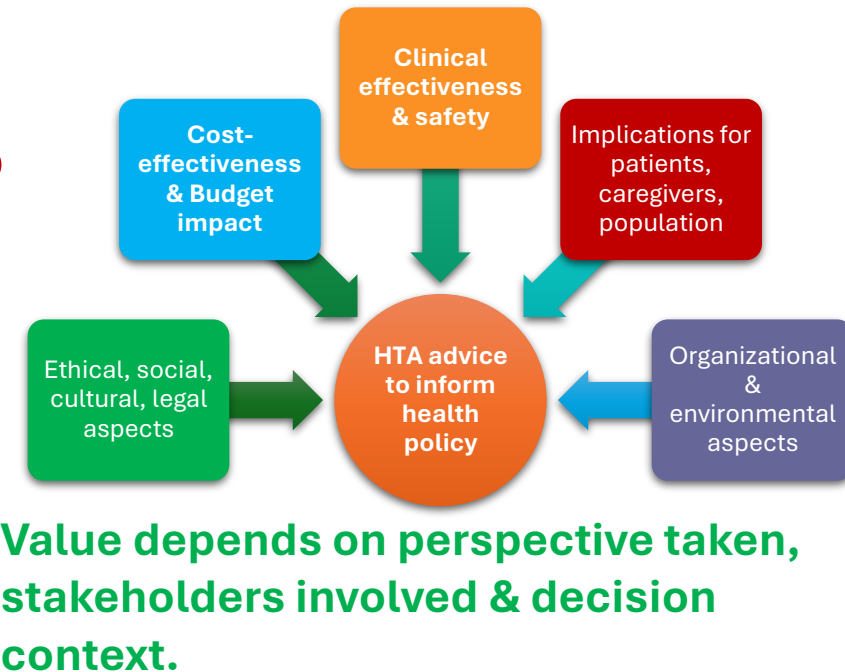
& ... What is Health Technology Assessment (HTA)?

HTA is a multidisciplinary process that

- uses explicit methods to determine the **value** of a health technology
- at different points in its lifecycle.

The purpose is to inform decision-making in order to promote an equitable, efficient, and high-quality health system.

value
↓
= Cost / outcome



Note 1: Definition of health technology (previous slide)

Note 2: The process is formal, systematic, and transparent, and uses state-of-the-art methods to consider the best available evidence.

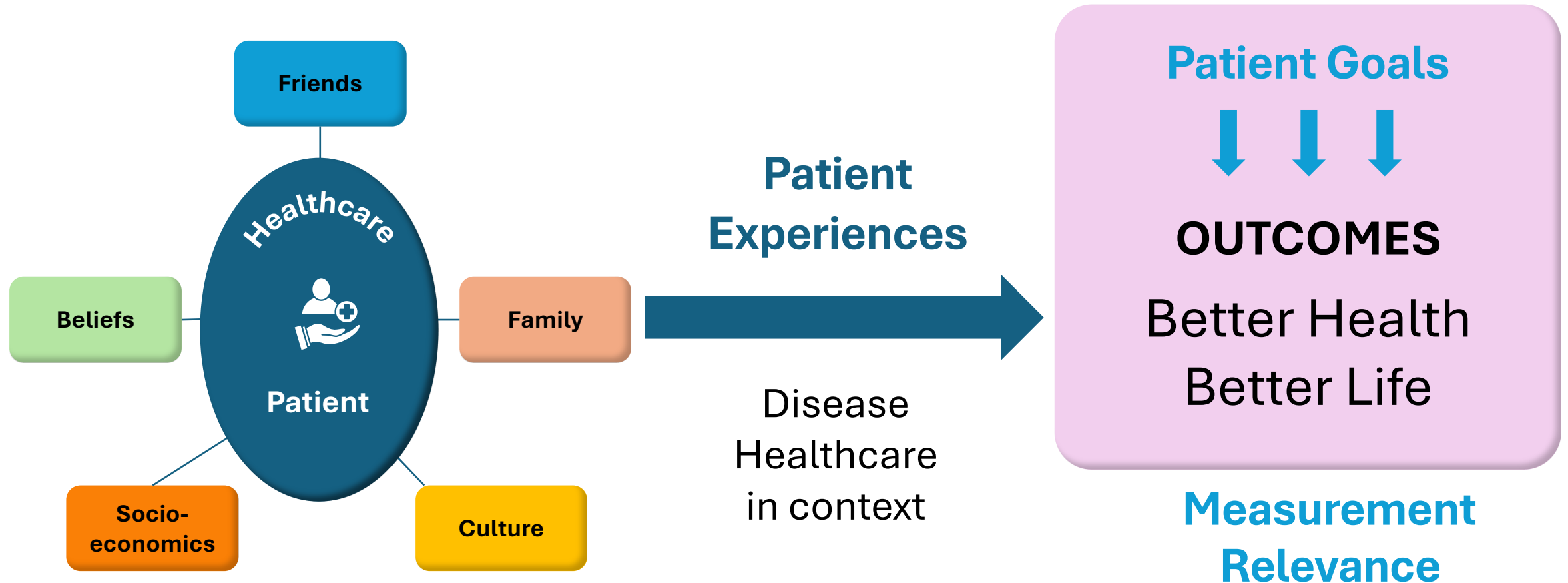
Note 3: The dimensions of value for a health technology may be assessed by examining the intended and unintended consequences of using a health technology compared to existing alternatives. These dimensions often include clinical effectiveness, safety, costs and economic implications, ethical, social, cultural and legal issues, organizational and environmental aspects, as well as wider implications for the patient, relatives, caregivers, and the population. The overall value may vary depending on the perspective taken, the stakeholders involved, and the decision context.

Note 4: HTA can be applied at different points in the lifecycle of a health technology, that is, pre-market, during market approval, post-market, through to the disinvestment of a health technology.

Defined in Multi-Organizational collaboration: INAHTA, HTAi, EUnetHTA, HTAsiaLink, RedETSA, the HTA Glossary Committee, ISPOR & WHO as observer

What Can a Patient Contribute to HTA?

Patients help to ensure that HTA determines Value that is relevant to patients



Patient Involvement in HTA Includes

PARTICIPATION



Two-way communication with patients to enable researchers & patients to learn from each other & solve problems along R&D

Current practice: attending meetings, consultation documents and workshops, receiving feedback ...

Strengths: local context, dynamic & responsive, capacity building

Research



Robust research into patients' needs, preferences & experiences using established explicit methods

Current practice: qualitative research, systematic reviews, patient preference studies, PROMs / PREMs

Strengths: Representativeness & allows addressing bias, strength of numbers / statistics, may include patients as co-researchers

How are Patients or Patient Organizations Involved via Research on Their Perspective?

Quantitative and qualitative research

- Patient Reported Outcomes
- Patient Experience Research
- Patient Preferences Research



Patient preference studies assess what matters most to patients, how much, and what tradeoffs patients are willing to make.

Patient Reported Experience Measures

Patient Reported Outcomes Measures

Digital Measures

Clinical Outcomes Measures

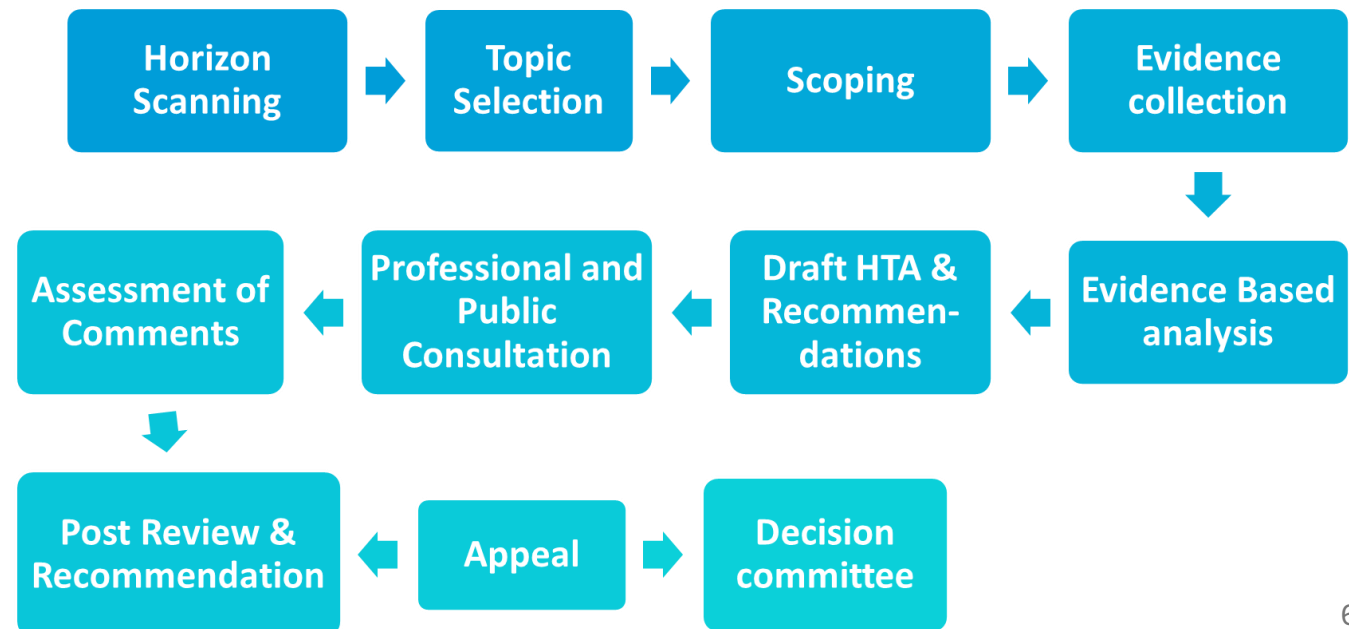
How are Patients or Patient(Organization)s Involved via Participation?

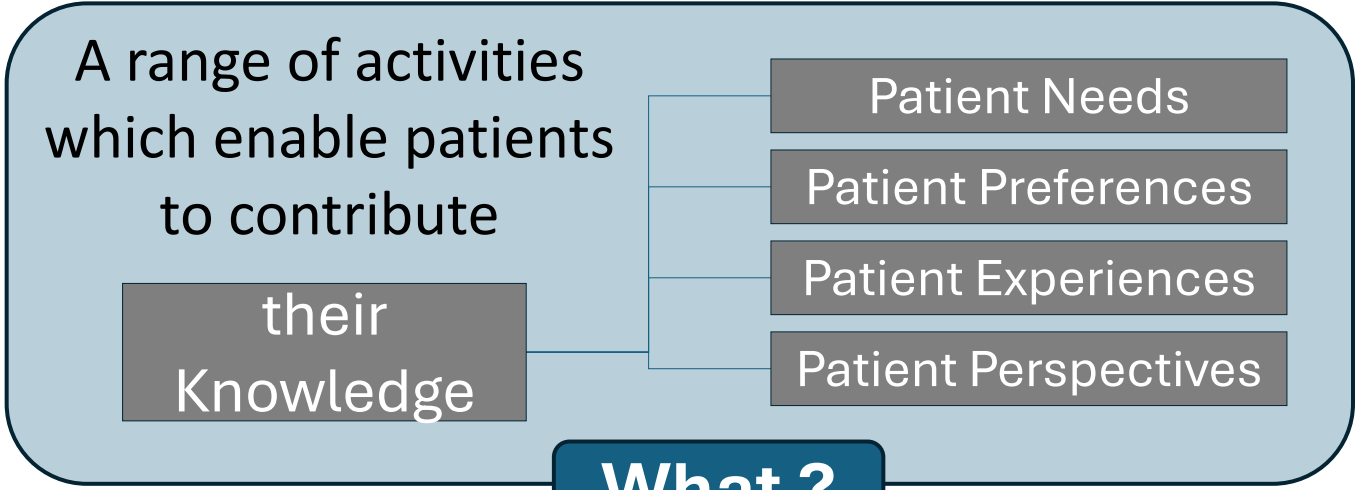
Participation

- Information
- Consultation
- Involvement
- Collaboration
- Empowerment



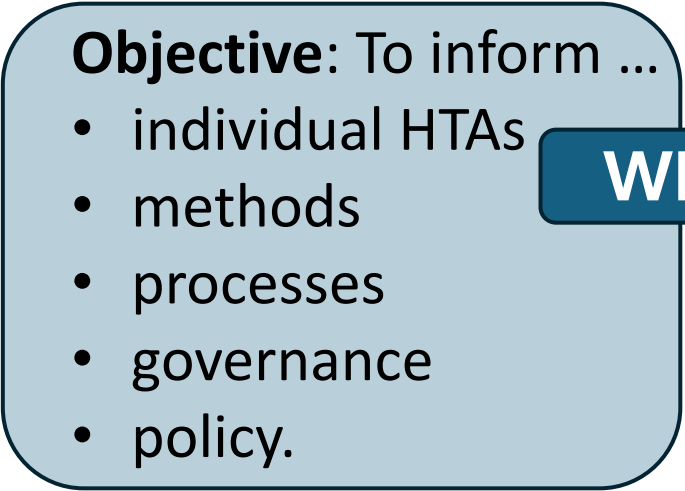
Organizational Level



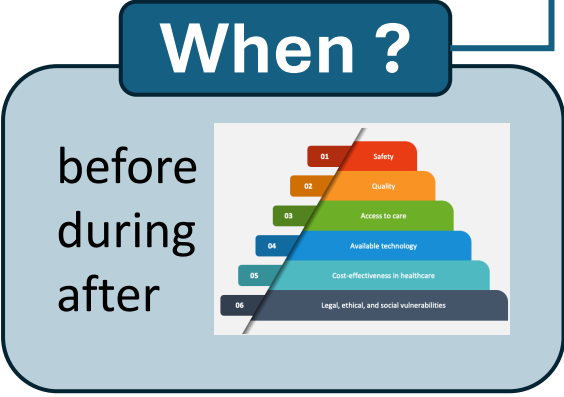


What ?

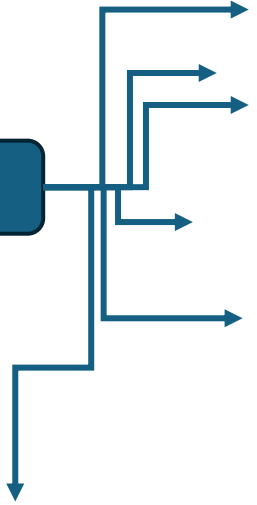
How ?



Why ?



Partnership and collaboration can result in a better outcome



What is the impact of Patient Involvement in HTA?

Impact on HTA recommendations / decision-making

- Data interpretation
- Patient and caregiver lived experience
- Patient needs
- New data consideration
- Decision direction
- Subpopulations
- Cost data
- Data limitations
- Patient acceptability of technology

Impact on HTA staff and processes

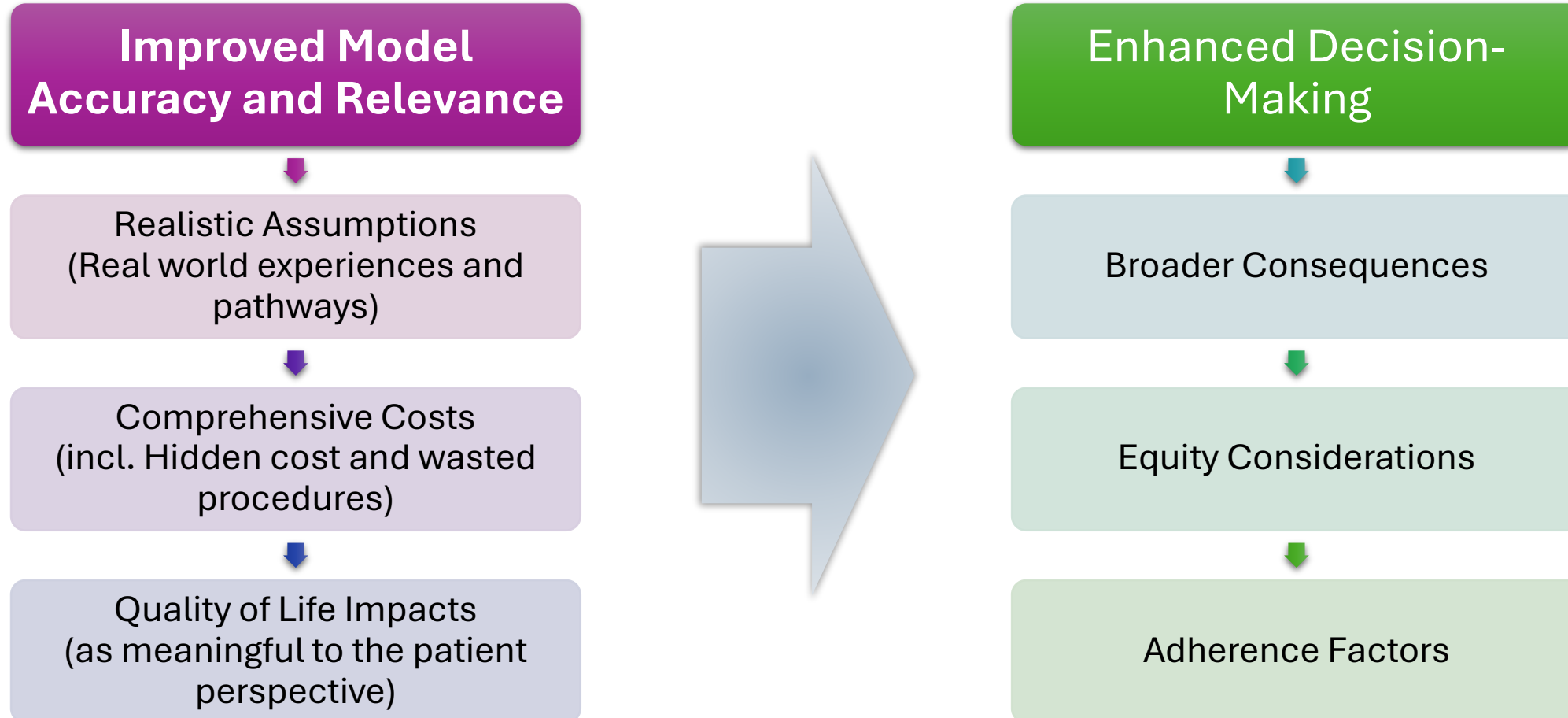
- HTA awareness of patient involvement importance
- Purpose-driven HTA
- Perceptions of patients as equal partners
- HTA engagement culture
- Direct contact / first-hand validation
- HTA process improvement

Impact on patient participants

- Acknowledgment
- Co-construction
- Culture of patient participation in HTA
- Patient awareness of HTA
- Patient decision acceptance

Most cited: ... **better understanding of patient experiences and needs** and **improved ability for data interpretation** ...

Patient Involvement in HE Modelling



Placing the patient voice on the health economists' bookshelf

Andrew Briggs

LONDON
SCHOOL of
HYGIENE
& TROPICAL
MEDICINE



GLOBAL
HEALTH
ECONOMICS
CENTRE

Andrew Briggs

Disclosure statement: I have acted as a consultant for many pharmaceutical and device manufacturers, as well as for WHO. I have acted as adviser to HTA agencies regarding methods of economic evaluation.

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The patient voice and the health economists' bookshelf

OVERVIEW

- Health is two-dimensional
- Culyer's bookshelf analogy
- Adding the patient voice
- Lessons learned

“ And in the end, it’s not the years in your life that count.
It’s the life in your years.”

Health Economics, Policy and Law (2016), **11**, 415–432 © Cambridge University Press 2016
doi:10.1017/S1744133116000049

Debate

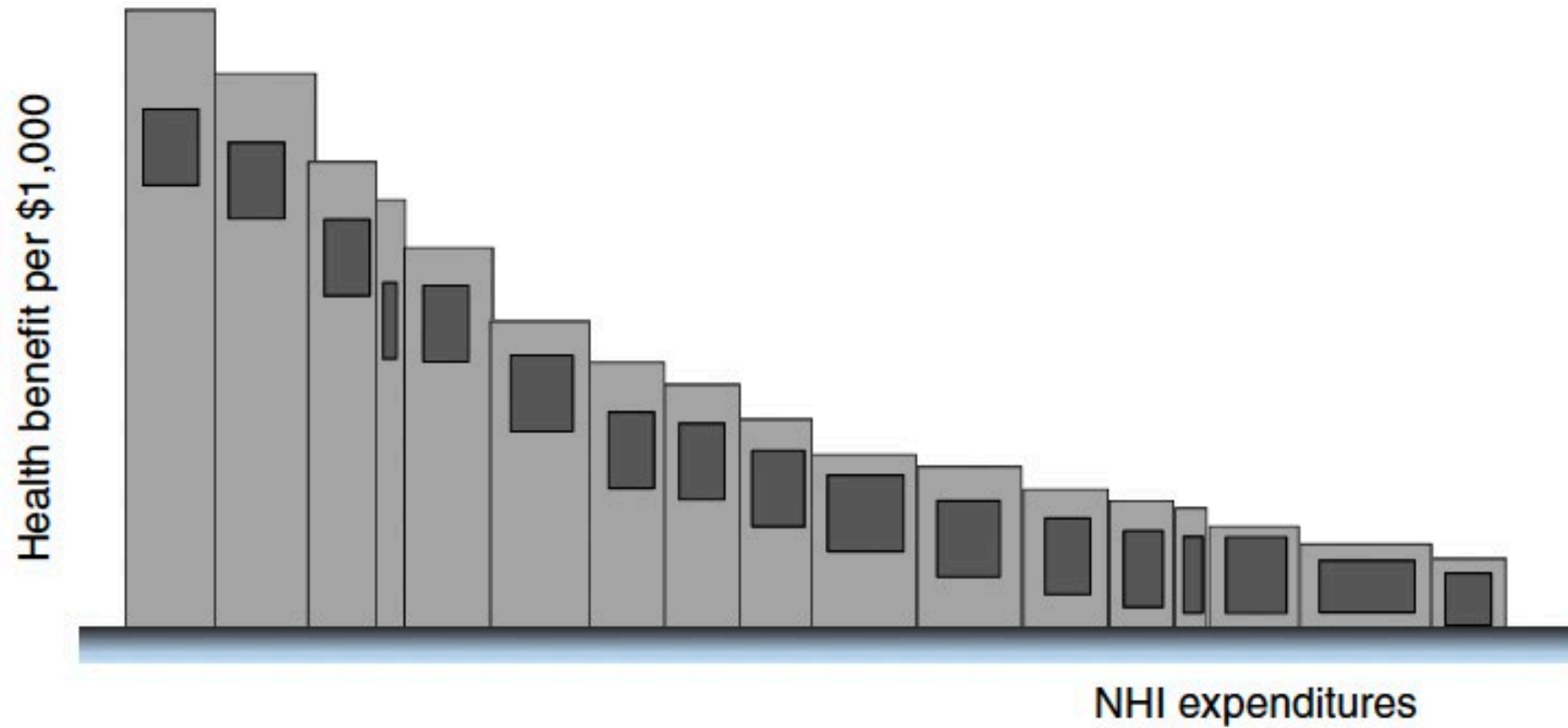
Cost-effectiveness thresholds in health care: a bookshelf guide to their meaning and use

ANTHONY J. CULYER*

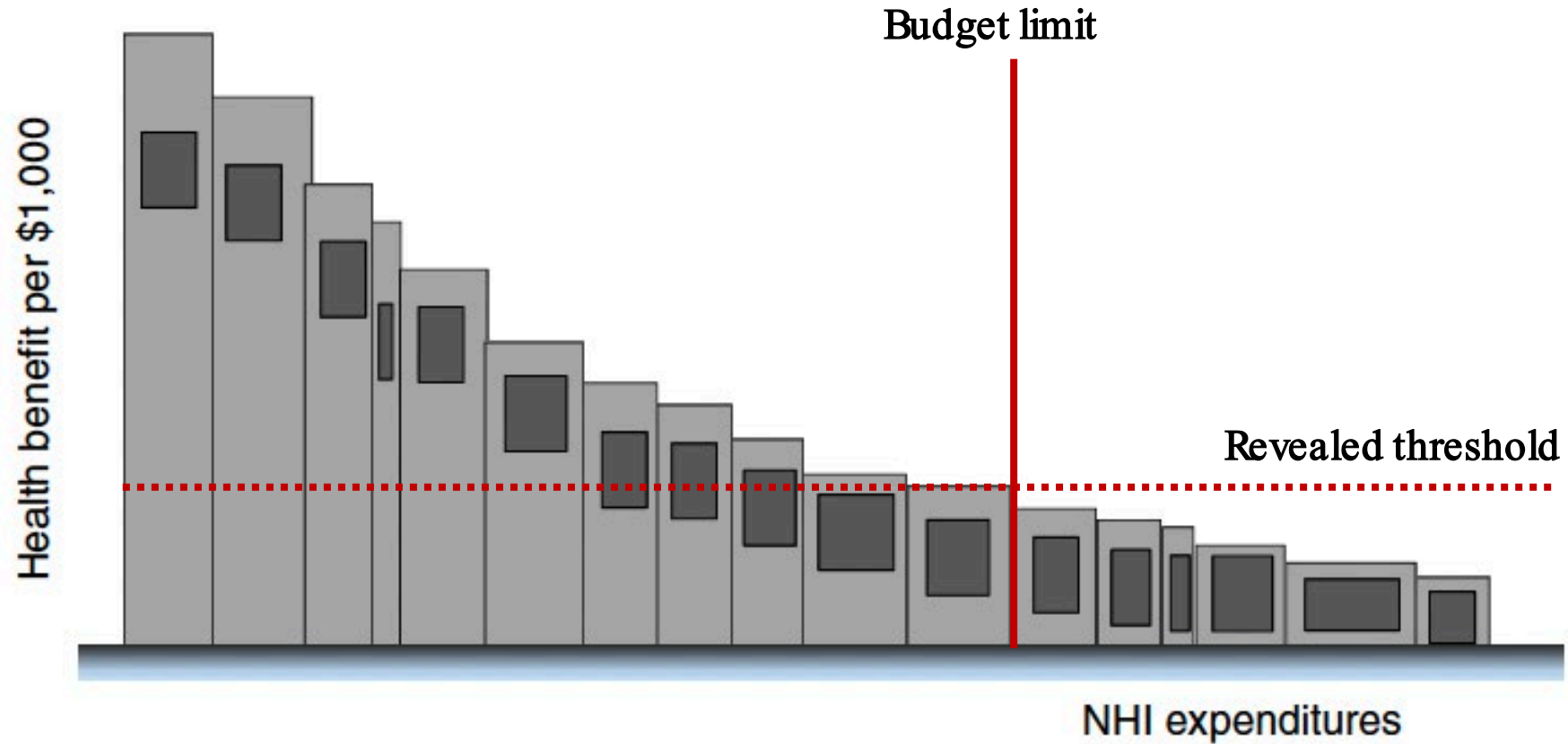
Institute of Health Policy Management and Evaluation, University of Toronto, Toronto, Canada

Centre for Health Economics, University of York, York, UK

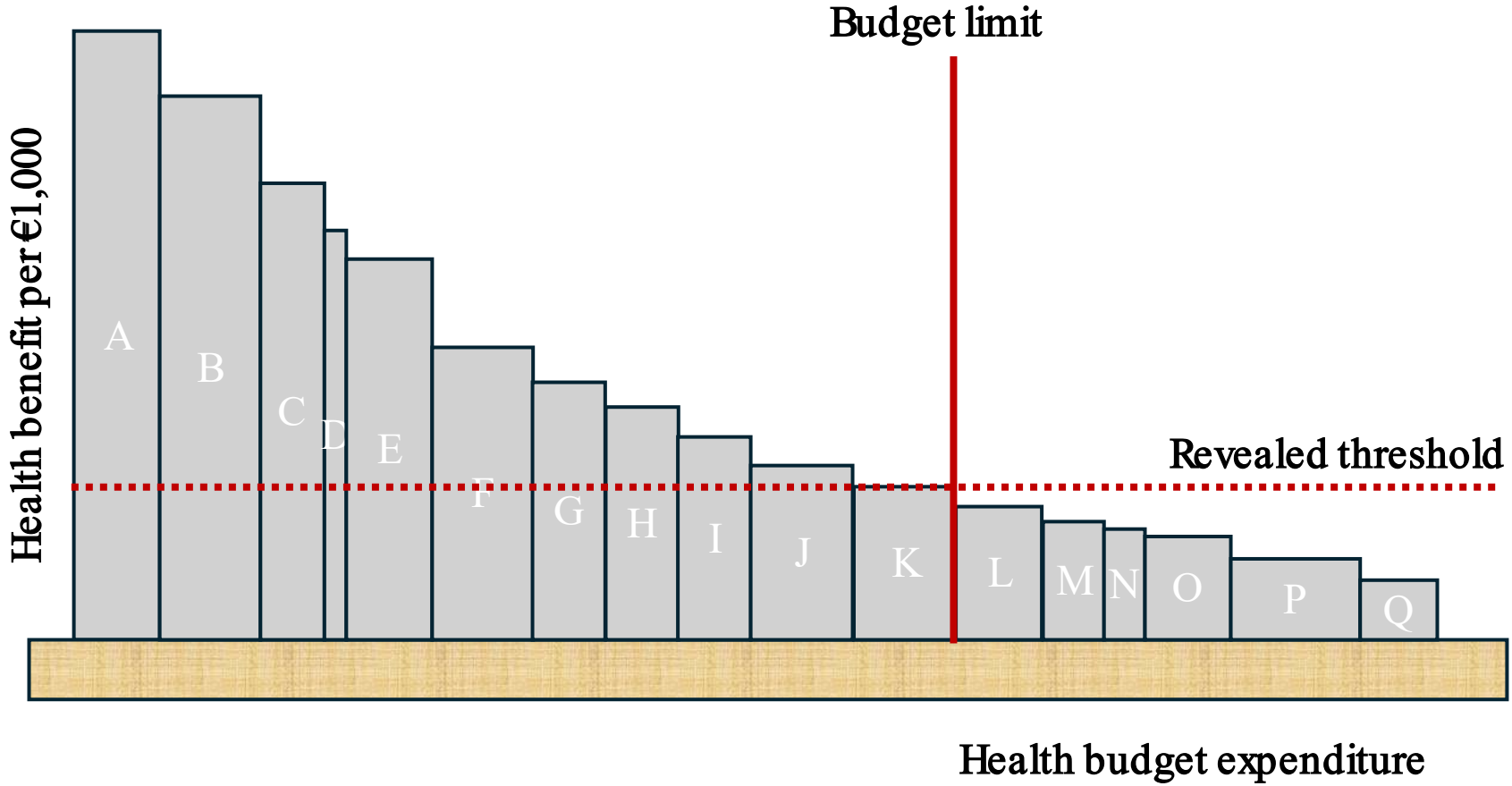
Culyer's bookshelf



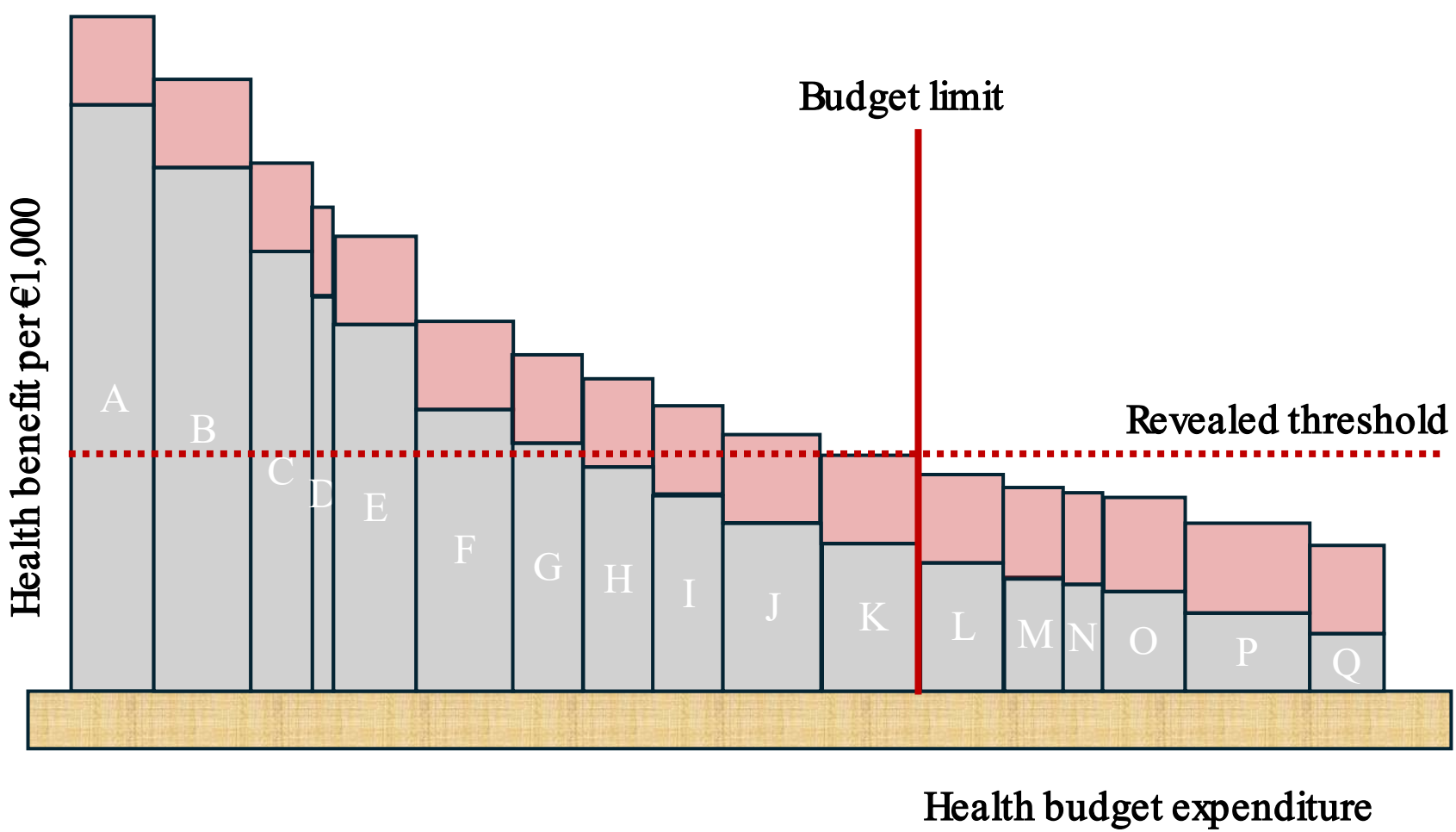
Budget defines the threshold



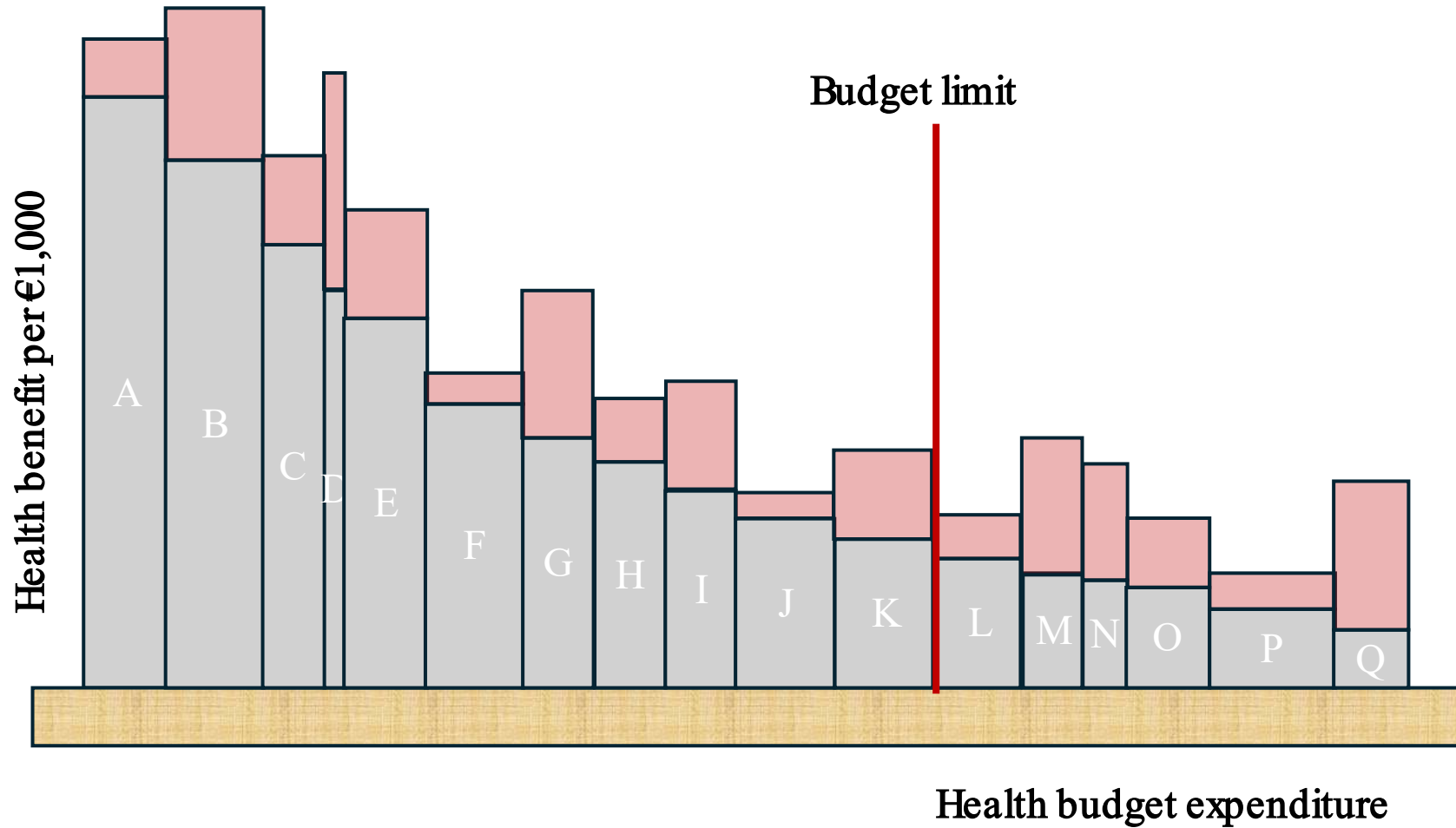
Placing the patient voice on the bookshelf



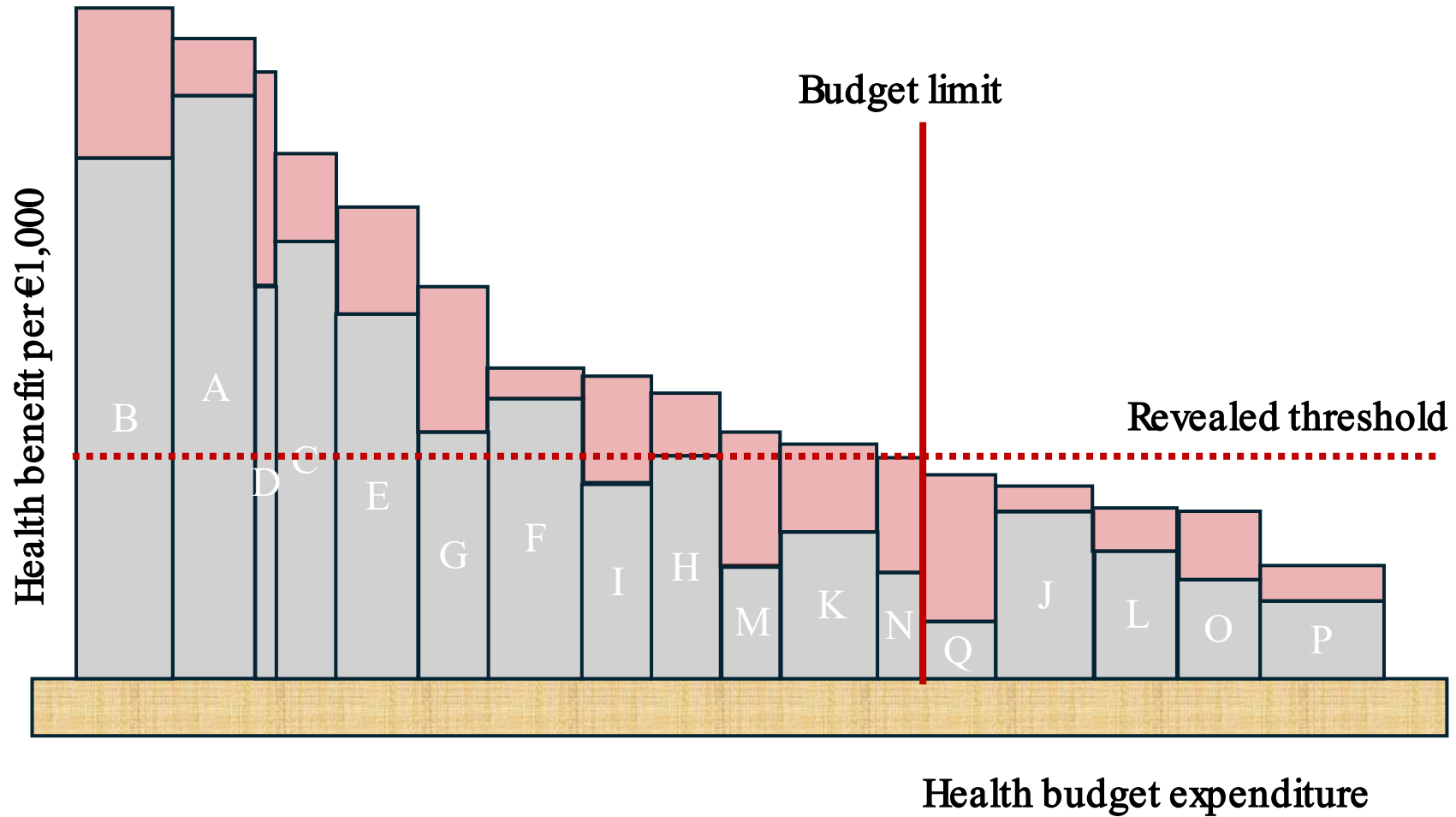
Placing the patient voice on the bookshelf II



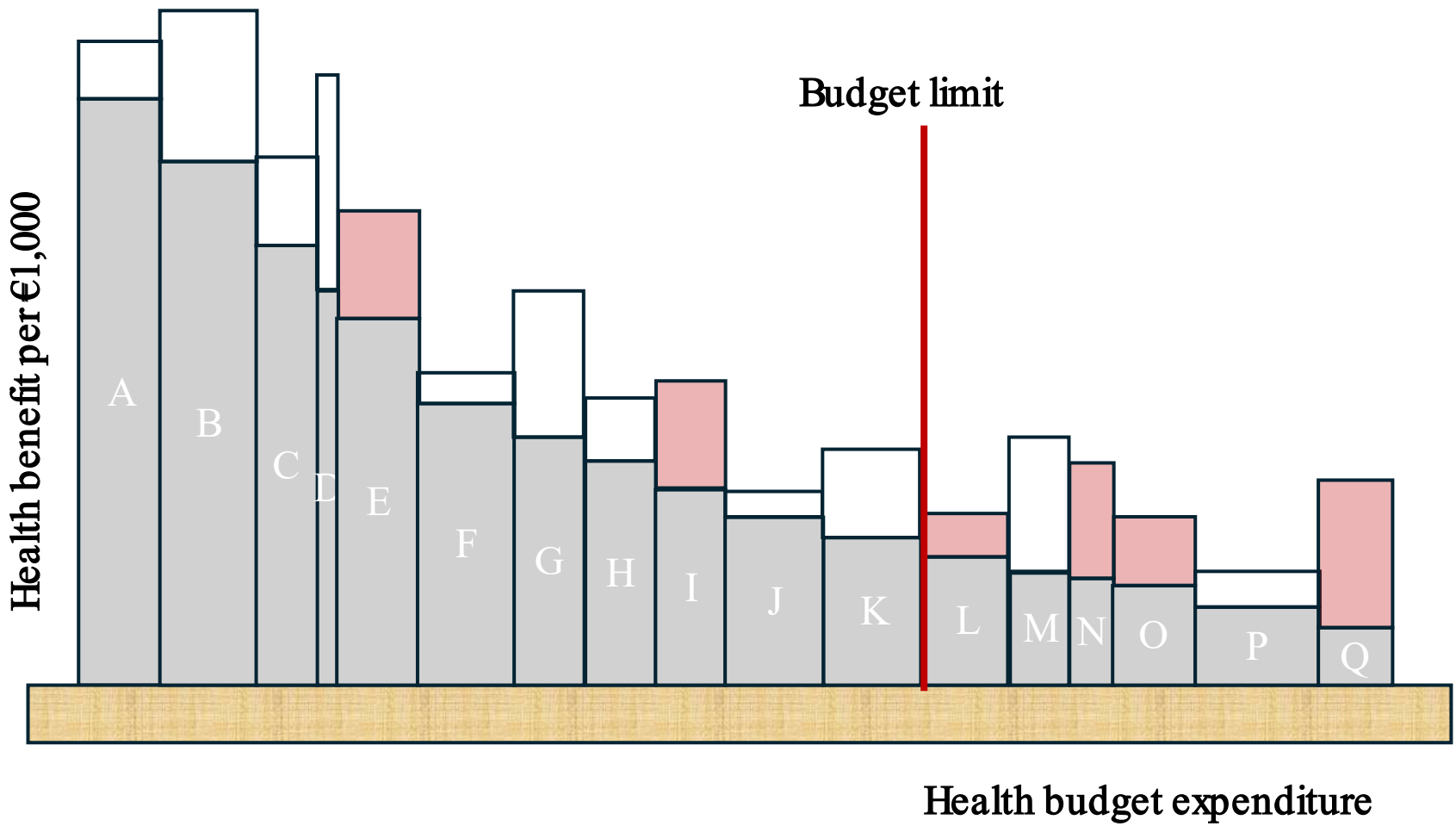
Placing the patient voice on the bookshelf III



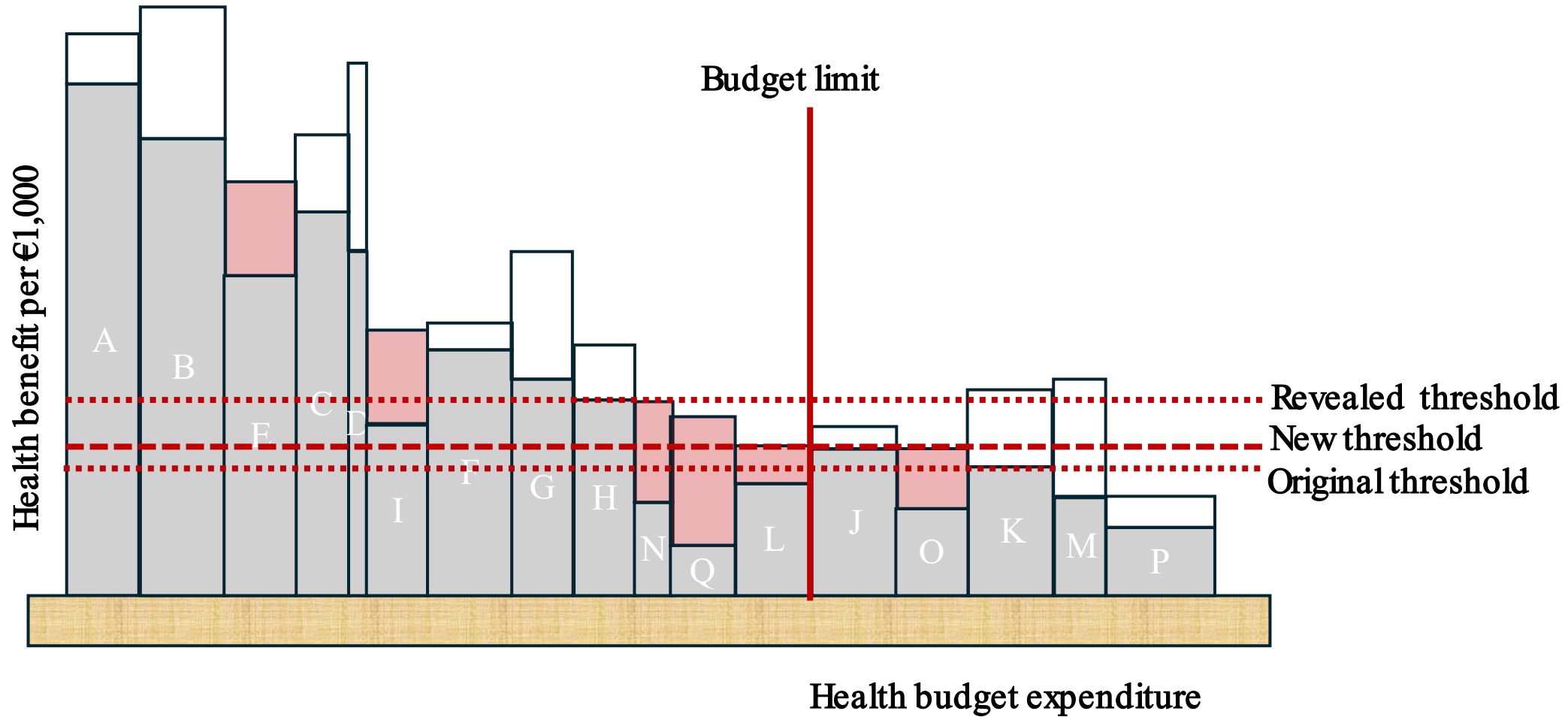
Placing the patient voice on the bookshelfIV



Voices not voice: the danger of selective application I



Voices not voice: the danger of selective application II



Health Economic Models Co-Produced with Patients

Opportunity to engage patients as legitimate stakeholders in the design of health economic models

- Choice of health states
- Granularity of health states
- Completeness of capture of important aspects for patients
- Consequent improvement in the acceptability of modelled analyses to patients
- Maximise the likelihood that patient important factors are captured
- Identify where there is additional value-add that models miss?

What have we learned?

- (Two dimensional) health measure is required for third party payer health systems if we are interested in maximising health of all patients
- In a budget constrained system, additional value from including the patient voice will increase (decrease) the EC (CE) threshold
- Uniform additional value will not impact the ordering of funded interventions
- Differential values will impact the ordering of funded interventions
- Selective use of patient voice can cost lives and exacerbate existing inequalities
- Important that methods for incorporating the patient voice are applied uniformly and fairly (critically including displaced technologies)
- Engaging patients as stakeholders in economic model development should lead to better models = better decisions

Integrating the patient voice into economic assessments of health technologies- a patient advocate's perspective.

Bettina Ryll, MD/PhD

Melanoma Patient Network Europe, founder
member of the first EU Cancer Mission Board

20th November 2024, ISPOR 2024

Declaration of interests

Bettina Ryll

- BR is the founder of MPNE. MPNE (Melanoma Patient Network Europe) is a volunteer-based network whose activities are or were funded by balanced support by the following companies: Amgen, BMS, Delcath, Idera, Incyte, Illumina, Immunocore, MSD, Merck Serono, Novartis, Pierre Fabre, Roche. Support never includes editorial rights, influence on MPNE's program nor activities. In addition, MPNE has received funding through 3 Horizon2020 grants (UMCURE, Share4Rare and iToBoS).
- **Unfortunately, we have been unsuccessful in diversifying our funding sources by support from regulatory, HTA and payer bodies or medical societies.**
- In the last 2 years, BR received personal consultancy fees for work from-
- AstraZeneca, BMS, CDDF, IQIB, IQVIA, MSD, Novartis, Pfizer, Roche, Sobi.
- BR's work for MPNE outside the Horizon2020 projects is non-remunerated
- Between 2019 and 2021, BR was member of the first EU Cancer Mission Board
- BR is currently employed at SIR, the Stockholm School of Economics Institute for Research and works as strategist for Vision Zero Cancer and TestBed Sweden for Precision Health in Cancer.

Patient and citizen engagement

- Our institutions owe transparency and accountability towards society.
- Solidarity-based healthcare systems are funded and supported by individuals in the belief they are protected in case of ill-fate.
- Those paying the ultimate price need to have a voice.

Nassim Nicholas

TALEB


Author of the
International
Bestseller
The Black Swan

SKIN IN THE GAME

allen lane



Patient and Public Involvement (PPI) in Health Economics Methodology Research: Reflections and Recommendations

Hareth Al-Janabi¹  · Jenny Coles² · John Copping² · Nishit Dhanji¹ · Carol McLoughlin¹ · Jacky Murphy² · Jean Nicholls²

Published online: 17 September 2020
© Springer Nature Switzerland AG 2020

Abstract

Patient and public involvement (PPI) can be used in methods research, as well as applied research, in health economics. However, methods research goals may seem quite abstract when compared to the lived experiences of lay participants. This article draws on 4 years of PPI in a research project to develop methods for including family carer outcomes in economic evaluation. Key challenges in using PPI for health economics methods research relate to (1) training and preparation, (2) maintaining involvement, and (3) selecting suitable tasks. We suggest three criteria for selecting a research task for PPI input based on task importance, professional researcher skills gap, and potential PPI contribution.

Patient engagement from the other side of the table

...as no one is sitting here waiting to be engaged.

[Health Qual Life Outcomes](#). 2018; 16: 222.

PMCID: PMC6267816

Published online 2018 Nov 29. doi: [10.1186/s12955-018-1047-z](https://doi.org/10.1186/s12955-018-1047-z)

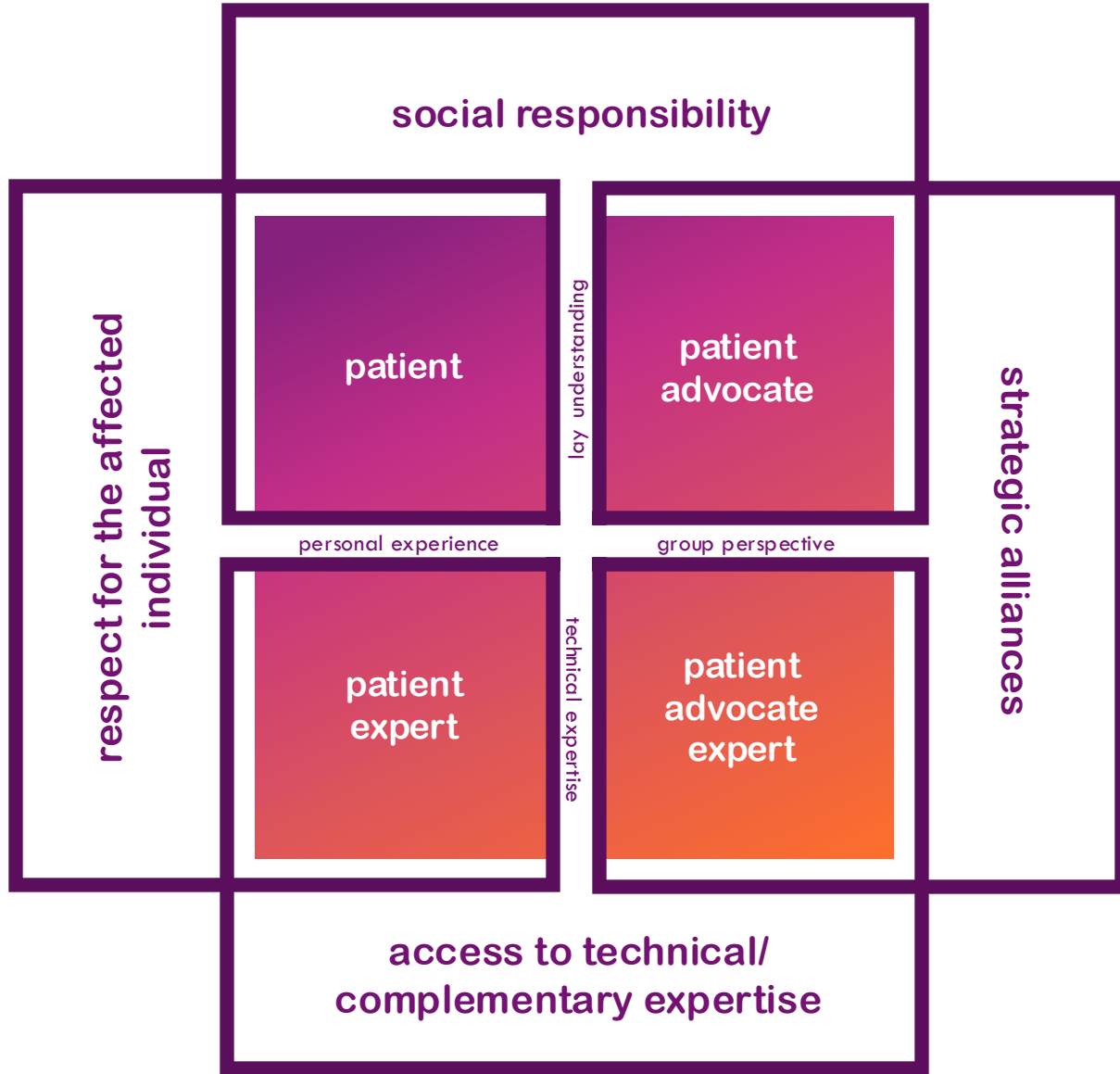
PMID: [30497502](https://pubmed.ncbi.nlm.nih.gov/30497502/)

Social media as a tool for assessing patient perspectives on quality of life in metastatic melanoma: a feasibility study

[Amr Makady](#),^{#1,2} [Rachel R. J. Kalf](#),^{#1} [Bettina Ryll](#),^{3,4} [Gilliosa Spurrier](#),³ [Anthonius de Boer](#),² [Hans Hillege](#),⁵
[Olaf H. Klungel](#),² [Wim Goettsch](#),^{1,2} and on behalf of GetReal Workpackage 1

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Concepts of patient engagement



PCM4EU

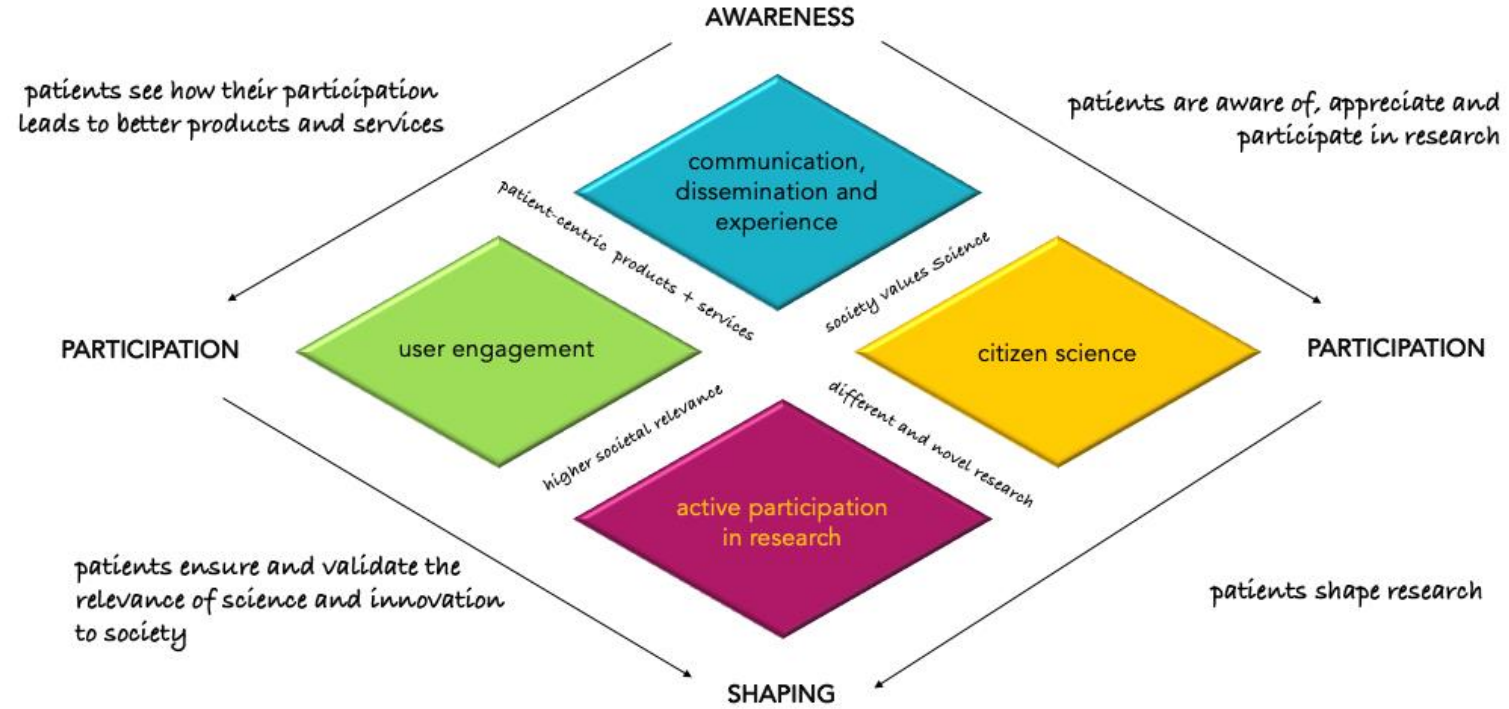
CC BY-NC-SA 4.0 PCM4EU, based on MPNE v2.0



**Funded by
the European Union**

personal experience societal perspective
group perspective
technical expertise

patient engagement v 1.5



patient ability to define and shape



patient time invested

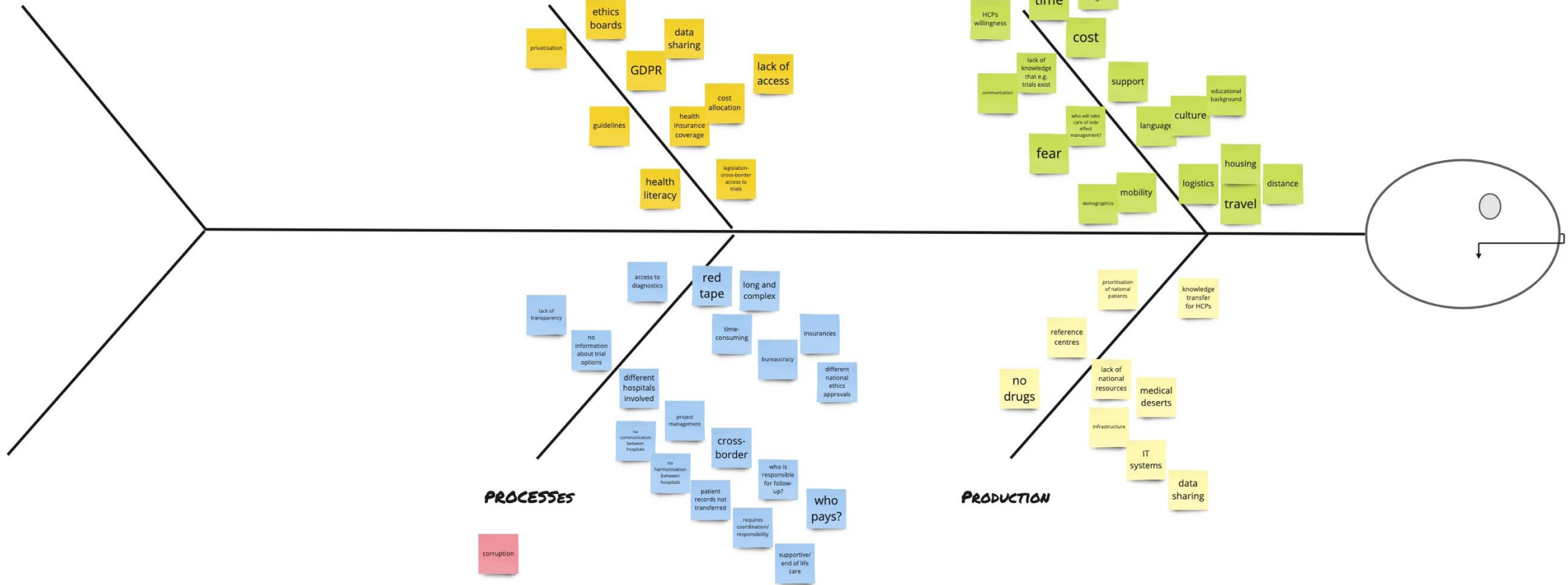


patient's expertise outside disease experience required



(C) CCBYSA





A series of unfortunate events



Precision medicine today

MPNEconsensus 2024

patient consensus meeting on data, AI
and data-dependent business models

registration closed

31st January- 2nd February 2024

Fraunhofer Institute for Telecommunications, Heinrich
Hertz Institute, HHI, Berlin

Lanolin Fabrik, Salzufer 15/16

Building on the concept of the [first MPNE consensus meeting](#), we are delighted to invite you to the patient consensus meeting on data, AI and data-dependent business models organised by the Melanoma Patient Network Europe, supported by European Union Horizon 2020 research and innovation funding under the iToBoS project, grant agreement 965221.

iToBoS



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 965221.



MPNE overarching consensus statements on data and AI

1. Data use is an ethical imperative as people are dying and we have data that could save them.
2. Data should be used but not abused. Individuals and societies need to be effectively protected against abuse; this will require new ethical frameworks for the use of data and AI, diverse risk mitigation strategies and proactivity and agility in the approach.
3. The approach to data use should be risk-appropriate, similar to the benefit/ risk trade-offs we see with drugs. Data is not a one-size-fits all category: differentiation of different data sets and the risks associated with them is critical.
4. Health data is common goods- it is not acceptable to extract value for a few and settle patients and society with the risks; this will require new models for business and research beyond platformization.
5. Altruism gaslighting is unacceptable: people have valid concerns and reservations, e.g. with regards to their privacy, protection against misuse and one-sided value extraction.
6. We need zero-trust environments beyond the reach of single parties, institutions or governments for the handling of data, in particular, for genomic data, to protect the rights of citizens.
7. Governance needs to ensure that those most affected by the risks have a voice.
8. We see that tech is not able to self-regulate, control therefore has to occur at the level of laws and regulations. We need hard guardrails of what is permissible, real-time monitoring and effective enforcement, e.g. existential fines to ensure compliance.
9. We need future-proof approaches, taking into account 'the unknown unknowns' of future technologies. We need to consider the risks for today's but also for future generations.
10. We as patient community need spaces for learning, exchange and debate to develop understanding and a position on complex topics- such as data and AI- that affect patients.

Community Advisory Board on hrQoL- Jan 2024

hrQoL endpoint for PRIME-ROSE cohorts



Scope 5/8

The primary and secondary endpoints of the pragmatic clinical trial should target overall survival, patient-preferred clinical benefit, patient-reported outcomes and **quality of life issues considered important by and for cancer patients and their caregivers**. Such endpoints should be defined together with patients and their caregivers through research models that use open knowledge, (social) innovation systems and support end-user engagement (e.g. living labs).

Objective 2

Healthcare professionals and academia will generate clinical evidence, by evaluating effectiveness in randomised or cluster-randomised academic investigator-initiated [1] pragmatic clinical trials, how to best perform and deploy evidence-based treatment interventions that improve outcomes in real life for routine healthcare, **including quality of life**, for cancer patients who often present with co-morbidities;

Objective 3

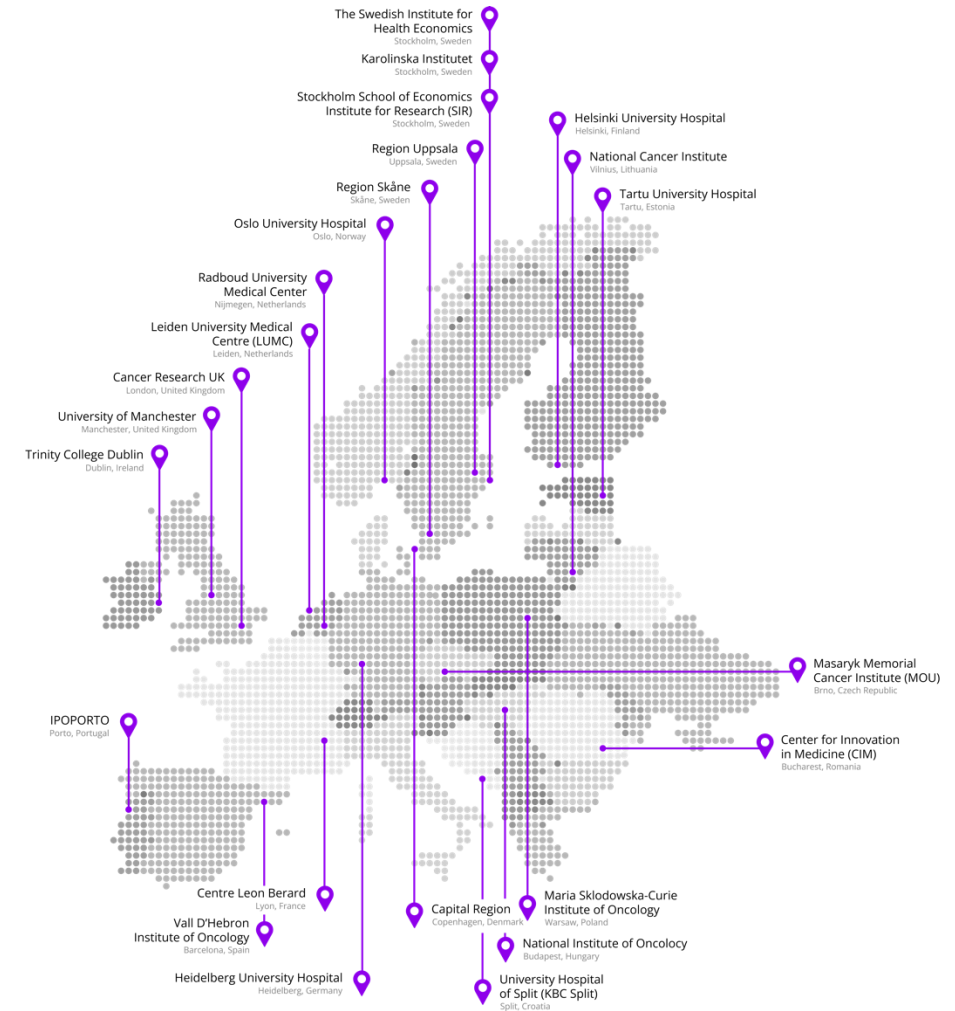
National healthcare providers, policymakers and authorities in EU Regions, Member States and Associated Countries will have **the evidence to implement optimised and affordable treatments in their healthcare systems, including in everyday medical practice.**

needed

hrQoL endpoint for PRIME-ROSE cohorts; to be determined by patients, clinicians, regulators, HTAs, payers M24



Funded by the European Union



Example for impact of patient engagement in iToBoS

T11.1

activities

interaction at MPNE event- Bootcamp Lisbon 2022: iToBoS partner LJ presenting his work on AI

outputs

idea to see whether visual transformation of a mole into a piece of individual art could help patients better cope with their Melanoma diagnosis

demonstration/ testing at MPNE events

outcomes

survey into the potential for Art Therapy

great interest by some MPNE members to engage (some patients use art as a form of coping)

positive oral feed-back at MPNE conference 'it's all very technical but you can see they CARE!'

impacts

potential new tool to help patients cope

potential for other applications, e.g. better recognition of malign transformation

iToBoS project became 'experientable' and with that, more accessible and approachable

increased understanding about European projects and research in general

some personal learnings

- Effective engagement is a long-standing, collaborative process that needs to include experimentation and learnings
- You need to start early and on neutral territory- the moment a product is involved, parties have vested interests
- To be truly effective, engagement has to be a two-way process where no side holds the absolute right to framing and interpretation
- Patient communities have to step up and contribute proactively
- Solid methodology is key, we need 2 level learning- at the level of the results as well as the methodology
- We need to find methods to include people not like us, e.g. Design Thinking

Thank you

bettina.ryll@mpneurope.org