

How to **INFLUENCE** reimbursement decisions as a patient advocate?

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Who makes what decision: Regulator

Regulator: European Medicines Agency (EMA)

- Aims to establish if **benefits** > **risks**

Regulation (EC) No. 726/2004

- Demonstrate **safety** (e.g. toxicity) and **efficacy** (e.g. survival, quality of life)
- No need to show the best choice (or relative efficacy)
- Economics not considered
- Approved indication – a specific population where benefit has been shown



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Who makes what decision: HTA/Payer

HTA/Payer (e.g. NICE)

- Reimbursement decisions made on a national level
- Different challenges across Europe – based on healthcare budgets, GDP, political willingness to pay
- As a result different treatments are available to patients in different EU countries (because they are unaffordable if not reimbursed)
- HTA: Is it a cost-effective use of resources?
- Payer: Willingness and ability to pay? What is the budget impact?

NICE
National Institute for
Health and Care Excellence

Decisions for the public or patients?

“Facilitate development and access to medicines: EMA is committed to enabling timely patient access to new medicines, and plays a vital role in supporting medicine development **for the benefit of patients**”

<https://www.ema.europa.eu/en/about-us/what-we-do>

“NICE's role is to improve outcomes **for people using the NHS** and other public health and social care services.”

<https://www.nice.org.uk/about/what-we-do>

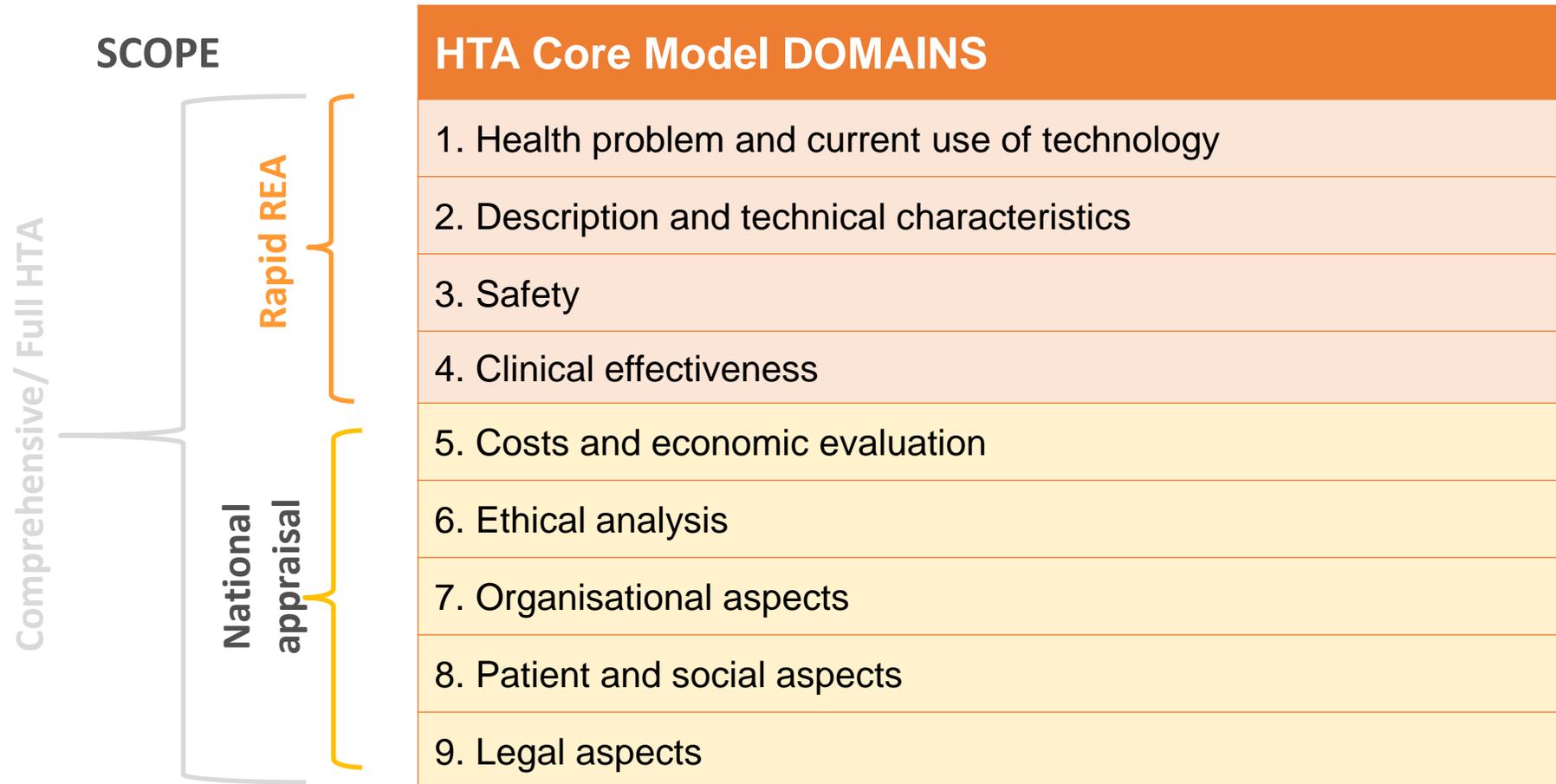


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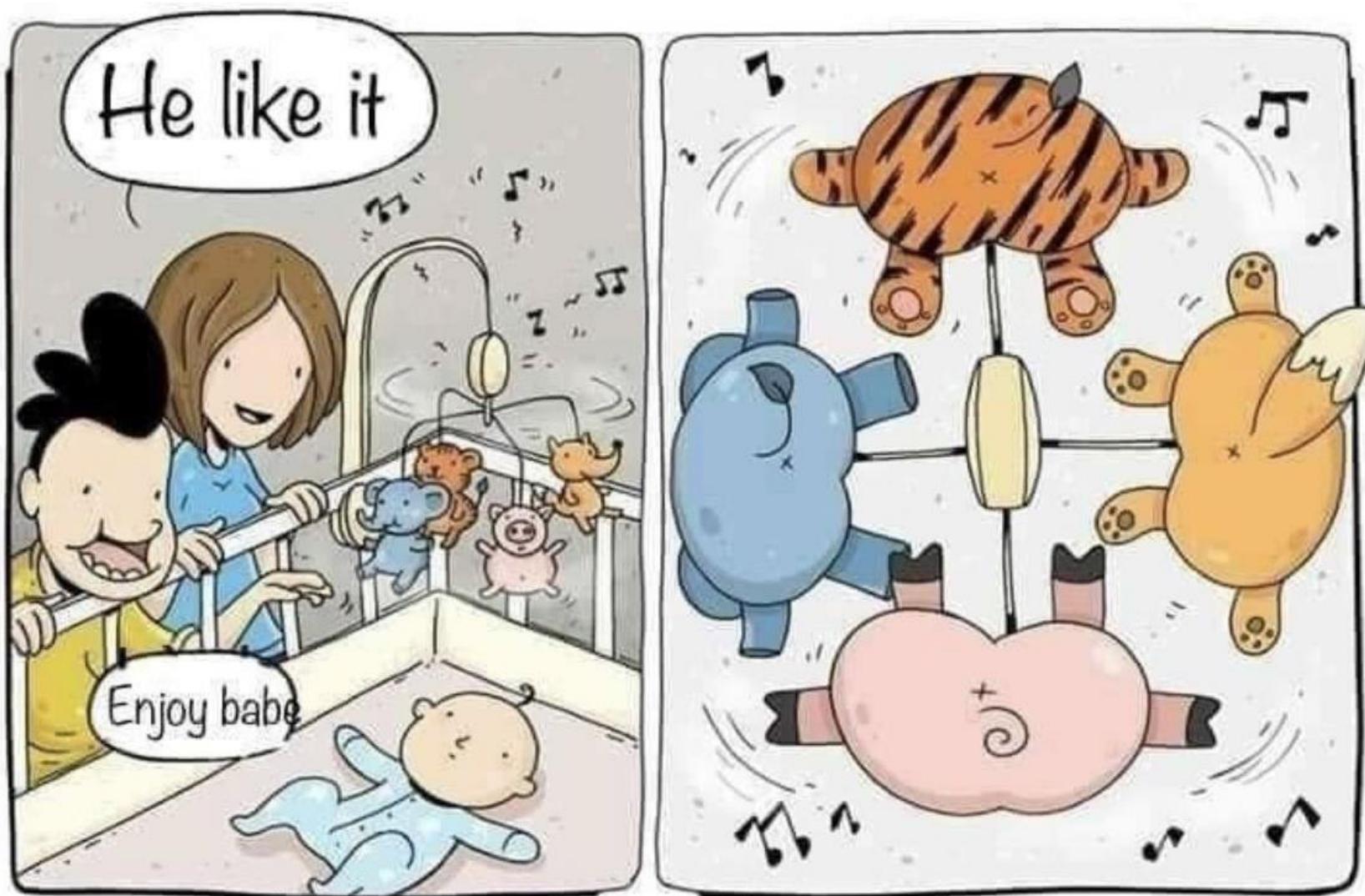
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What do they consider in HTA? EUnetHTA HTA Core Model



Why do we need patient involvement in HTA?



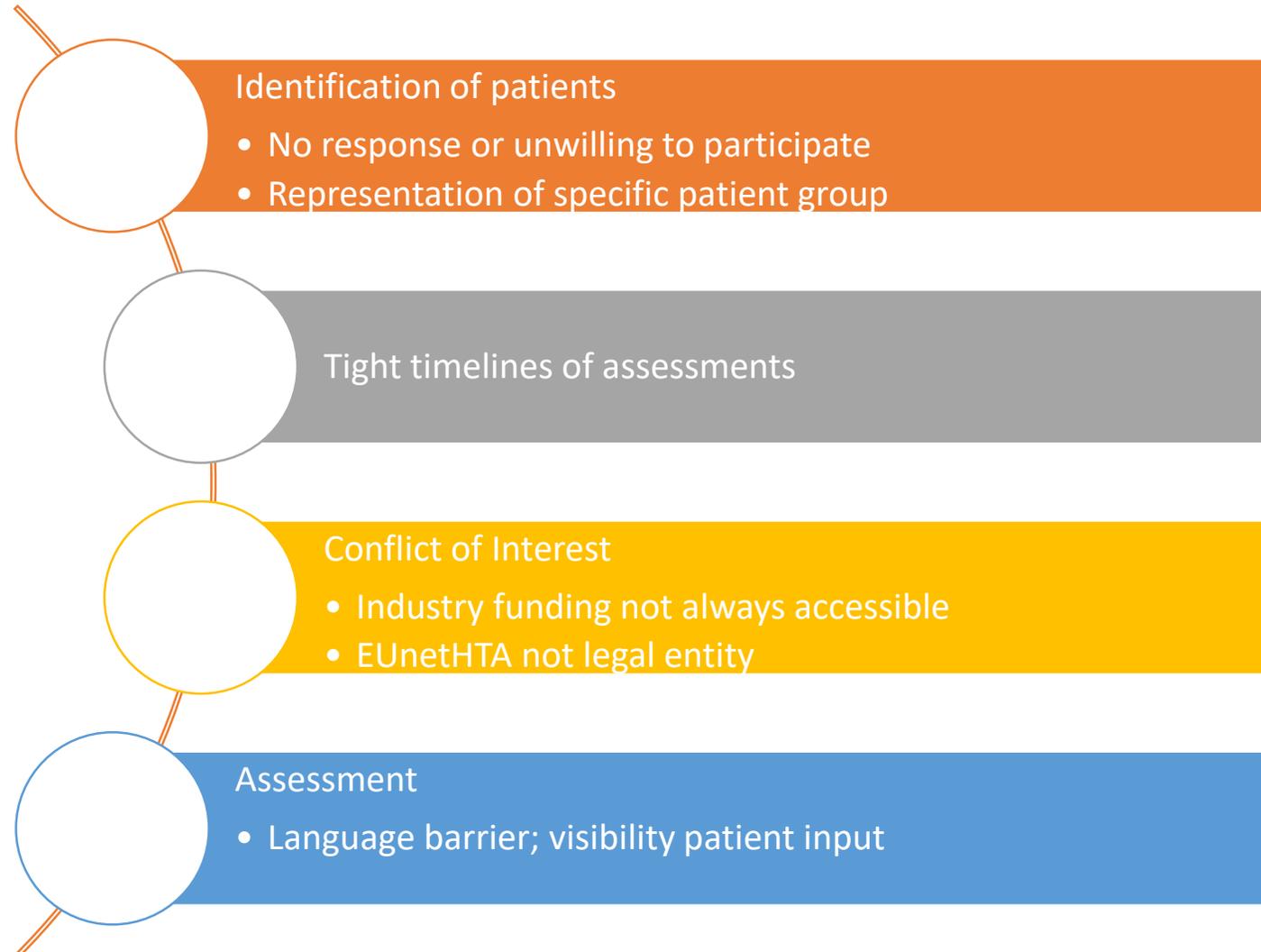
What is the role of patient advocates in HTA?

From the perspective of the HTA agency:

“NICE's approach to patient and public involvement is based on two key principles:

- that lay people, and organisations representing their interests, have **opportunities to contribute** to developing NICE guidance, advice and quality standards, and support their implementation, and
- that, because of this contribution, our guidance and other products have a **greater focus and relevance** for the people most directly affected by our recommendations.”

Challenges with patient involvement (from an HTA perspective)



A comparison of the different UK processes

| | NICE | SMC | AWMSG |
|-------------------------|-----------------|--|-------------------------------------|
| Scoping | ✓ | X | X |
| Technical Engagement | ✓ | X | X |
| Evidence Submission | ✓ | ✓ | ✓ |
| Patient Focused Meeting | ? | ✓ PACE (For rare and end of life medicines) | ✓ CAPIG (For rare diseases only) |
| Committee Meetings | ✓ | ✓ | X (Public Gallery) |
| Opportunity to Appeal | ✓ (ACD and FAD) | X | X |
| Publication | ✓ | ✓ | ✓ |

Does patient involvement have an impact?

- Patient Organisations and Patient Experts “do not feel that their efforts to **contribute** to the process are seen as being **credible** by NICE committees” (NICE Patient Group Workshop, Jan 19)
- Patient testimony is usually qualitative (e.g. patient testimony), so the **impact on decision making** is not usually obvious (NICE and Myeloma UK, Measuring Patient Preferences, June 19)
- Where is the opportunity to impact in a QALY based system?
 - Survival X
 - Quality of Life ?

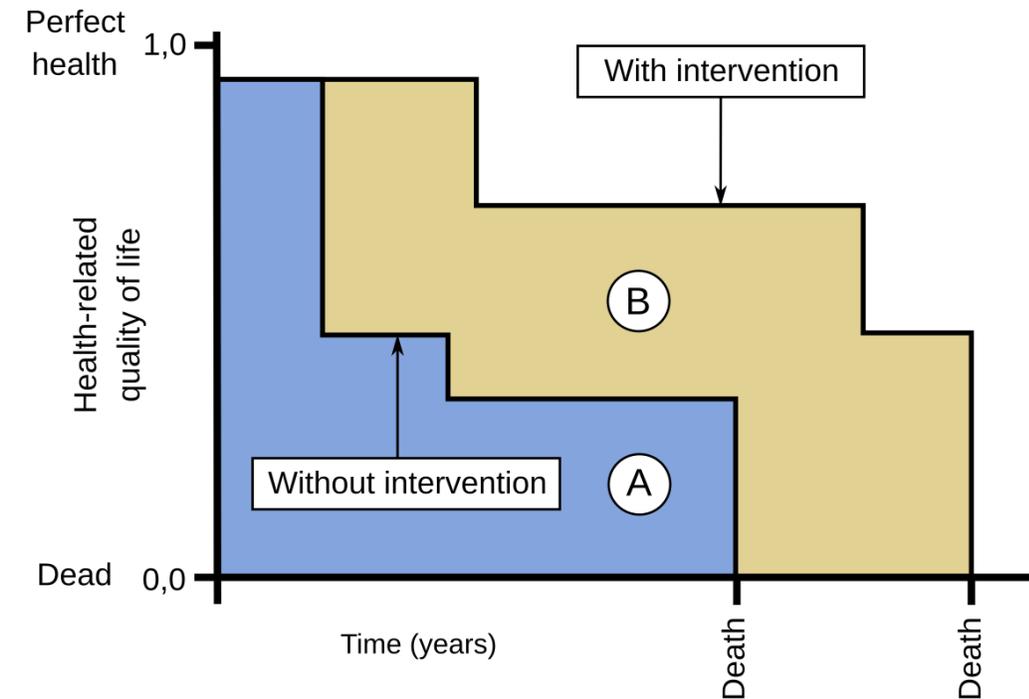


Image: www.publichealthnotes.com/qaly-quality-adjusted-life-years/ (Accessed 12.06.19)

Responsibility on patient organisations

- Developing **capability** and **capacity** to engage effectively in access decisions
- Evidence-based advocacy of patient views and experience
 - Trainings on evidence-based advocacy
 - Generate evidence – in a format that stakeholders can use for decision making

Evidence-Based Advocacy

Advocating in a targeted, evidence-based, well-educated and professional manner, and measure impact and outcomes of what we do.

