

# Evolving endpoints in cancer trials, the HTA perspective

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The views expressed are those of the presenter and should not be understood or quoted as being made on behalf of the Norwegian Medicines Agency (NoMA), the European Medicines Agency (EMA) or its scientific committees, nor the European Commissions Health Technology Assessment Coordination group (HTACG).

# There are many guidelines but this one is important

EUnetHTA 21

**EUnetHTA 21 – Individual Practical Guideline Document**

**D4.4 – OUTCOMES (ENDPOINTS)**

***ORIGINAL TITLE SERVICE CONTRACT: PRACTICAL GUIDELINE ON ISSUES  
ENCOUNTERED ON THE ENDPOINTS IN JCAS/CAS***

**Version 1.0, 25/01/2023**  
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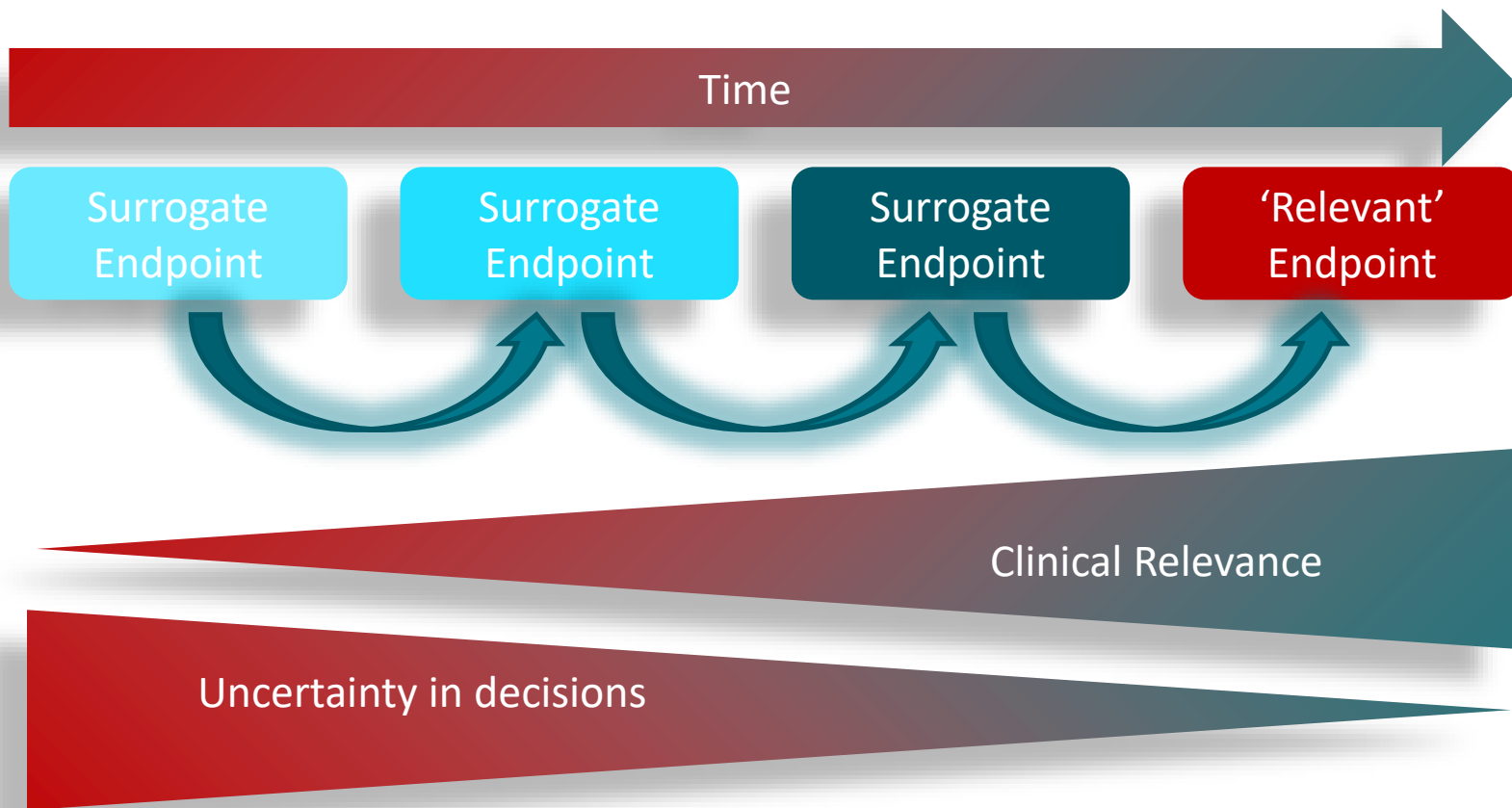
# The HTA definition of a surrogate outcome

- outcome that is intended to replace an outcome of interest that cannot be observed in a trial
- provides an indirect measurement of effect in situations in which direct measurement of a patient-centred effect is not feasible or practical
- may be a biomarker that is intended to substitute for a patient-centred outcome, or it may be an intermediate outcome
- is expected to **only predict** the treatment effect
- The use of surrogate outcomes in assessment of the clinical added benefit of a health technology can be controversial since the validity of surrogate outcomes has rarely been fully established in a rigorous manner
- Only a few surrogate outcomes have been shown to be true measures of tangible clinical benefit

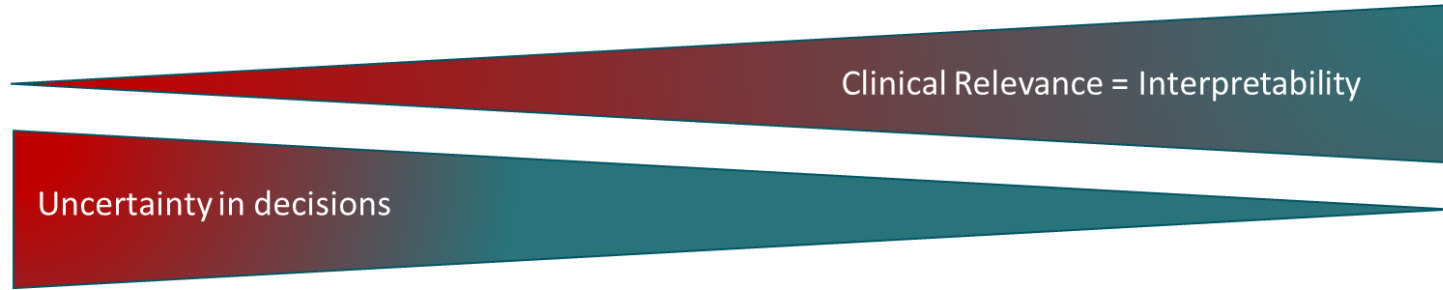
# It is all about decision making

- Endpoints have different ‘value’ to those that need to make a decision
- B/R → is there enough signal for efficacy to outweigh safety risks
- C/E → is there proven causality, precision and likelihood to translate in a clinical relevance experience for ‘my’ patients
- Physicians and patients → is the surrogate meaningful on the individual level, is that information enough to make me choose one treatment over another

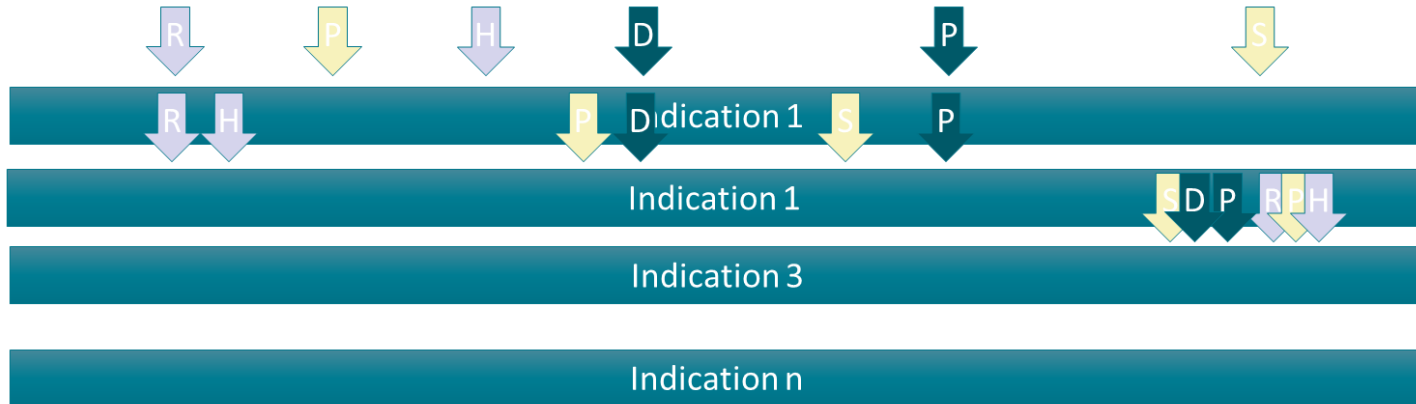
# Uncertainty is piling up



# The Sweet-spot



The range of sweet-spots





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