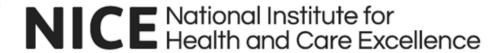
Highly Specialised Technologies Programme at NICE

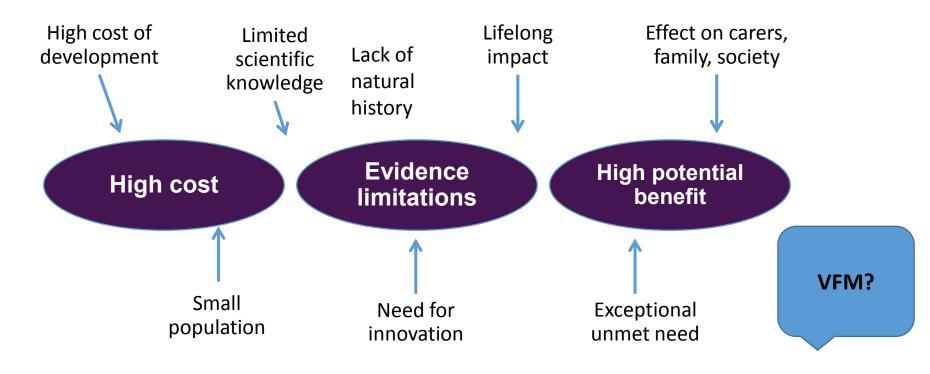
Sheela Upadhyaya

Associate Director: Highly Specialised Technology Program (NICE)

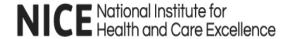
ECRD - May 2018



The HST Program for Rare and ultra-rare conditions



HST: Evaluates high-cost technologies for exceptionally rare conditions, for commissioning by NHS England



HST Methodology

- The HST program how considers cost-effectiveness in terms of incremental cost per QALY
 - Below £100k/Q, decision is normally based on cost-effectiveness estimate
 - Above £100k/Q, judgements take account of the magnitude of benefit and the additional QALY weight that would be needed to support recommendation

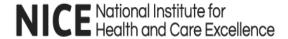
Incr QALYs	Max weight
≤10	1
11 - 29	1 – 3 (sliding scale)
≥30	3

- As part of consideration of value for money
- Other factors still contribute to decisionmaking

Decision-making in HST

• "More to decision-making rather than strict application of costeffectiveness methods"

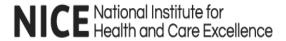




Why this approach?

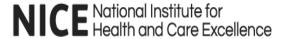
- Acknowledged that treatments for very rare conditions command a premium in the health system
- assessed against our current standard threshold - None of these treatments is cost effective
- Need to offer an objective, systematic, transparent and repeatable approach to deciding whether to fund new treatments
- Stakeholders seeking clarity

- Using incremental QALY gain as a way of illustrating, quantitatively, what actually matters to patients (incremental therapeutic benefit)
- Higher ICERs are only acceptable when associated with higher QALY gain
- What matters most and what will attract the highest premium, is therapeutic benefit.



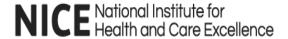
Evaluation - Challenges

- Defining the patient population
 - Population defined in Marketing Authorisation
 - Clarity on the patient population that will most benefit
- Uncertainty on outcomes
 - Create solutions to bridge gap
 - Give assurance to NICE that these will be addressed
- Impact on carers/ family members
 - Quantify this impact in submission
- Lack of Natural History Data
 - Use of surveys/ interviews
 - Patient group data



Observations

- At NICE QALY is recognised currency to operate in for all programs
- Rare diseases do not always have validated quality of life tools that can be assessed in evaluations
- How can we incorporate and value other criteria what is fair and should it only apply to rare diseases
- Why does the QALY not capture empirical preferences what needs to change



Thank you

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