

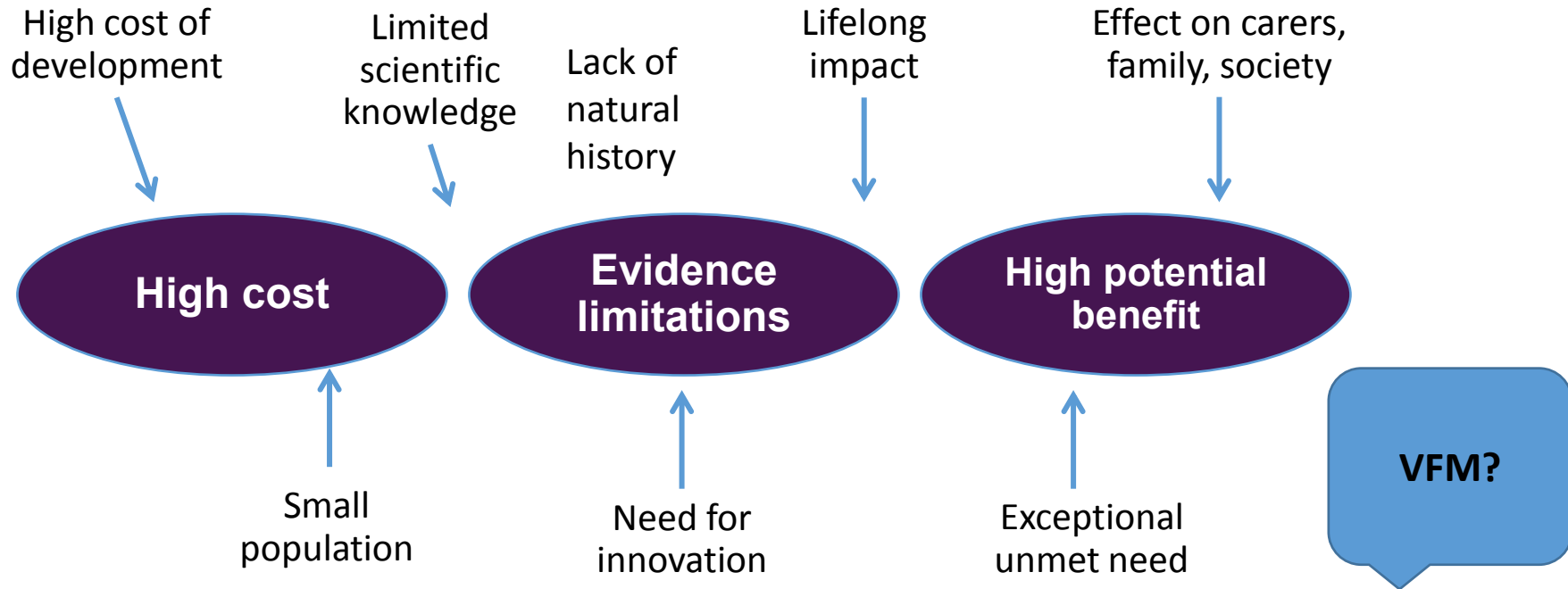
# Highly Specialised Technologies Programme at NICE

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# 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

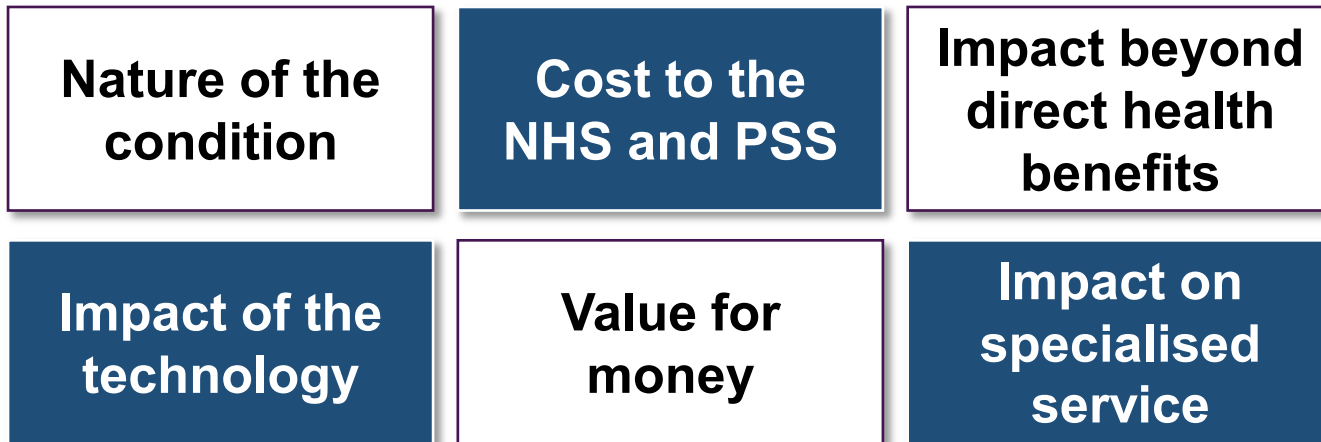
Incr QALYs	Max weight
≤10	1
11 - 29	1 - 3 (sliding scale)
≥30	3

❖ **As part of** consideration of value for money

❖ **Other factors** still contribute to decision-making

# Decision-making in HST

- *“More to decision-making rather than strict application of cost-effectiveness 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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