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The Value of "New": Attitudes to and Consideration of Treatment Novelty in Health Technology Assessment

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Treatment Novelty in HTA

- Do HTA bodies explicitly consider novel aspects of treatment in their data syntheses and/or deliberations?
- Should they?
- What improvements are possible in whether and how novelty is considered in HTA processes?

Agenda

1. New research on novelty consideration in HTA
2. Comments from panelists representing patient, academic, and HTA perspectives
3. Panel discussion
4. Your questions and comments

Panelists

Valentina Strammiello, MSc

European Patients' Forum

Panos Kanavos, PhD

London School of Economics & Political Science

Nick Crabb, PhD

National Institute for Health & Care Excellence

Polling Question 1

- Do you think treatment novelty should be a distinct and explicit criterion for HTA decision-making?
  - Yes
  - No

Advance to next slide for the poll

HTA Consideration of Novelty

Goals

- To examine HTA approaches to product novelty in diverse settings and systems
- To characterize:
  - Domains of novelty considered
  - Methods to value product novelty
  - Variation by disease and/or variation type
  - Variation in evidence standards
  - Influence of novelty on deliberation and outcomes of HTA
- Manuscript in press at Applied Health Economics & Health Policy

Approach

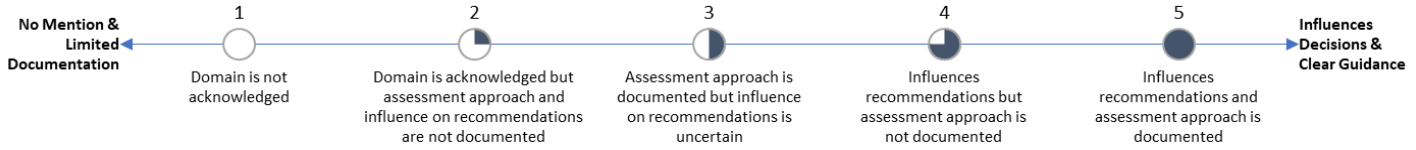
- 8 countries analyzed (AU, CA, FR, GB, NL, NO, SE, US)
- Published and grey literature
- Novelty definition:

Domain	Examples
New approach to treatment	Mechanism of action; method of administration; modified dosing
Addresses unmet need	No effective Rx; severe disease; resistance to alternatives
Broader impact beyond what is traditionally measured	Non-quantifiable benefits; benefits beyond patient; major shift in clinical practice

Key Findings

- “Novelty” not specifically called out in HTA documentation, but…
- Elements of definition often associated with adjustments to HTA assessment approach
- Some influence on deliberations and recommendations

Findings by Organization



Novelty domain		Australia PBAC	Canada CADTH	England NICE	France HAS	Netherlands ZIN	Norway NoMA	Sweden TLV/SBU	United States ICER
New Approach to Treatment	New molecular mechanism of action	3	1	1	5	1	1	1	1
	New method of administration	1	3	2	1	1	1	1	4
	Modified dosing schedule	1	1	2	1	1	1	1	4
Addresses Unmet Need	Targets a condition without effective treatments	3	4	1	5	5	1	4	1
	Targets a severe condition	4	4	5	5	5	5	4	4
	Addresses substantial residual morbidity from existing treatments	1	4	1	1	1	1	1	1
	Effective in patients whose condition is resistant to current therapies	1	1	1	1	1	1	1	1
	Intended for a group that requires special consideration	4	4	4	5	5	5	1	3
Broader Impact	Provides benefit not captured in measure of health gain	4	3	5	1	1	1	3	4
	Confers benefit beyond targeted population	2	1	4	1	1	1	1	4
	Marks major shift in clinical practice	4	3	2	1	1	1	1	3

Figure Note: HTA: Health technology assessment, PBAC: Pharmaceutical Benefit Advisory Committee, CADTH: Canadian Agency for Drugs and Technologies in Health, NICE: National Institute for Health and Care Excellence, HAS: Haute Autorité de Santé, ZIN: Zorginstituut Nederland, NoMA: Norwegian Medicines Agency, TLV: Dental and Pharmaceutical Benefits Agency, SBU: Swedish Agency for Health Technology Assessment and Assessment of Social Services, ICER: Institute for Clinical and Economic Review

Case Study: Voretigene Neparvovec

- Gene therapy for rare inherited condition leading to complete blindness in children and adolescents
- Reviewed by all 8 HTA organizations of interest:
  - 7/8 described unmet need for severe/rare disease w/no alternatives
  - Several mentioned positive spillover to caregivers/families
  - Potential downside of impaired equity described
  - Uncertain long-term outcomes and cost-effectiveness
- Recommendations:
  - Fully positive: 3 (FR, GB, US)
  - Conditional: 3 (AU, CA, NL)
  - Negative: 2 (NO, SE)

Case Study: Ocrelizumab

- Monoclonal antibody indicated for both relapsing (RMS) and primary progressive (PPMS) forms of multiple sclerosis
- Evaluated by 6 of 8 HTA bodies of interest:
  - Minimal consideration of novelty in RMS given # of alternatives
  - Reduced dosing frequency highlighted by 3 of 6 organizations
  - Greater consideration of unmet need in PPMS
  - Discussion of broader impacts (caregiver benefits, ⬆hospital care) mixed
- Recommendations:

	RMS	***PPMS***
— Fully positive:	4 (AU, FR, GB, NO)	4 (FR, GB, NO, US)
— Conditional:	2 (CA, US)	1 (CA)
— Negative:	0	1 (AU)

Summary

- Activities of 8 illustrative HTA bodies evaluated
- No explicit mention of treatment novelty as explicit criterion for value assessment and/or summary judgment
- Special consideration often given for novel aspects of treatment in appraisal and deliberation, however
- Are potentially *negative* aspects of novelty given equal weight?
- Improvements warranted in:
  - Documentation of criteria used and how applied
  - Transparency in how novelty affects recommendations and decisions

Panel Comments & Discussion

Poll Question 2

- Where should treatment novelty play a role in HTA processes (check all that apply)?
  - Appraisal of clinical evidence
  - Quantitative value assessment
  - Quantitative deliberation
  - Qualitative deliberation
  - Judgment/recommendation/decision
  - None of these

Advance to next slide for the poll

Questions for Panel

- What are the potentially positive and negative implications of explicit consideration of treatment novelty from your perspective?
- Could/should novelty play a role in:
  - Appraisal of clinical evidence
  - Quantitative value assessment
  - Quantitative deliberation
  - Qualitative deliberation
  - Judgment/recommendation/decision

Thank you!

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