Collaboration for Leadership in Applied Health Research and Care South London (CLAHRC South London)

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No implementation without operationalization: Comparing the role of actors, paradigms and rules of evidence in England and Germany

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● The Collaboration for Leadership in Applied Health Research and Care (CLAHRC) South London is investigating the best way to make tried and tested treatments and services routinely available. University-based researchers, health professionals, patients and service users are working together to make this happen. ● The collaborating organisations are Guy's and St Thomas' NHS Foundation Trust, Health Innovation Network (the NHS England-funded academic health science network in south London), King's College Hospital NHS Foundation Trust, King's College London, King's Health Partners, St George's University Hospitals NHS Foundation Trust, St George's, University of London and South London and Maudsley NHS Foundation Trust. ● The work of the CLAHRC South London is funded for five years (from 1 January 2014) by the National Institute for Health Research, collaborating organisations and local charities. It is hosted by King's College Hospital NHS Foundation Trust. ● The CLAHRC is also working closely with GPs, local authorities (responsible for public health) and commissioners of health services in south London.

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What is the project about?

- Comparative study into the health technology assessment (HTA) outcomes in England and Germany: Qualitative analysis of 10 of the same products that were assessed in 2011 and 2012; stakeholder interviews
- HTAs summarise and compare evidence on medicines and clinical interventions to assess clinical and cost effectiveness → Organisations have been established to inform (reimbursement & coverage) decisions/policies
- HTA used as a policy instrument to help decision-makers in the process of making difficult decisions (delegation of decision-making to expert bodies)
- Puzzle: HTA outcomes on the same medicines and interventions vary from country to country

→ Can we explain variation by looking at how HTA policies are implemented?

Health care in England and Germany (Implementation context)

England

- National Health Service (NHS) financed through taxation, free at the point of use
- Health care provision is commissioned by 207 regional clinical commissioning groups (CCGs), NHS England and local authorities
- Remains a highly centralized and politicized system
- History of priority-setting in health care

Germany

- Self-governing system: Statutory health insurance (sickness) funds, provider associations including doctors, hospitals and pharmacies → Negotiation of contracts
- Financed through mandatory employer and employee contributions to sickness insurance funds, supplemented through taxation
- Government only sets overall statutory framework; state oversight limited by principle of self-governance
- Priority-setting in health care is a controversial topic

Implementation object: HTA in England and Germany

	National Institute for Health and Care Excellence (England)	Federal Joint Committee (Germany)	Institute for Quality and Efficiency in Health Care (IQWiG) (Germany)
Composition	Appraisal Committee (AC): Members from the NHS, patient organizations, academia, pharmaceutical industry	Representatives of the self-governing health care system	Salaried employees
Decision-making mandate	Positive recommendations are binding	Final decision-making on pharmaceutical benefit assessment	Recommendations to FJC, non-binding
Purpose of assessment & appraisal	**To recommend the use or non-use of a medicine based on clinical and cost effectiveness criteria**	**To inform price negotiations between sickness funds and pharmaceutical manufacturers**	To assess the pharmaceutical manufacturer's dossier
Who commissions an appraisal?	Commissioned by the Secretary of Health, based on topic recommendations	All pharmaceutical products with a new active substance must be appraised (§135a Social Code Book V)	Commissioned by the FJC
What gets appraised?	Topics referred to by the Secretary of State	All pharmaceutical products with a new active substance	All pharmaceutical products with a new active substance
Appraisal outcome	**Recommended, not recommended, only in research**	**Assignment to one of six 'additional benefit' categories**	**Assignment to one of six 'additional benefit' categories**

Target and implementers

Target/goal:

- England: To address postcode lottery; to ensure cost effective use of resources; to identify potential interventions for disinvestment?
- Germany: To set a limit on the prices that pharmaceutical companies can set for new products

Implementers:

- Newly established institutions (NICE and IQWiG)
- Health care providers (England) and self-governing bodies (Germany)

Source: http://econitynepal.com/tag/bureaucracy/

Operationalization in implementation: Paradigms and rules of evidence

- Differences in HTA outcomes can be explained by differences in how policy (ideas) are implemented; concepts of policy paradigms and rules of evidence can help explain the differences
- Policy paradigms are used in public policy literature to explain big policy changes; ***broadly, paradigms refer to the framework of ideas, concepts, norms and values, policy solutions that guide decision-makers and policymakers*** (Hall, 1993)
- Rules of evidence (Majone, 1989): “When the issues under discussion require complex patterns of reasoning and large amounts of data of doubtful reliability and relevance, explicit rules of evidence become […] important” (p. 10) → What counts as evidence, how is it interpreted, what thresholds does it need to meet?

Operationalization in implementation: Example

- Principles of HTA policy need to be operationalized in real life

Act on the Reform of the Market for Medicinal Products (Germany):

The benefit of a pharmaceutical product is: "[…] the **patient-relevant** therapeutic effect, in particular in respect of the *improvement in the state of health, the reduction of the duration of the disease, […] an improvement in the quality of life*" (Bundesgesetzblatt, 2010). (Emphasis added).

IQWiG:

- Benefit in relation to patient: How a patient feels, whether they can go about their daily lives, whether they survive
- Mortality, morbidity, Health-related quality of life (HRQoL)
- Assignment into one of six 'additional benefit' categories: Extent of significance of statistical results + quality of evidence

Example (continued): Additional Benefit Categories

Benefit Category	Definition
1. Major additional benefit	Sustained and large improvement; in particular a recovery from the disease, a considerable increase in life, long-term freedom from severe symptoms or extensive avoidance of severe side-effects […].
2. Significant additional benefit	Considerable improvement; in particular lessening of severe symptoms, moderate extension in life, an easing of the disease, which is noticeable to patients
3. Marginal additional benefit	Moderate improvement; in particular a reduction in non-severe symptoms of the disease or a relevant avoidance of side-effects.
4. Additional benefit, but not quantifiable	Lack of scientific data to quantify benefit
5. No additional benefit	
6. Benefit less than that of fit-for-purpose comparator	

Example (continued): Rules of evidence

Product & indication	FJC	NICE
Fingolimod (Multiple Sclerosis)	Different benefit categories for 3 different patient populations: 1. Patients with highly active relapsing-remitting MS/heavily pre-treated: **Additional benefit not substantiated** 2. Patients with highly active relapsing-remitting MS/not heavily pre-treated: **Additional benefit not substantiated** 3. Patients with rapidly evolving severe relapsing-remitting MS: **Hint of a marginal benefit** <u>Issues with evidence:</u> • Patient populations in trial more broadly defined than in marketing authorisation • Did not accept indirect comparisons • Did not acknowledge oral formulation as an innovation (**not a patient relevant endpoint**)	'Conditional' recommendation Recommended as an option for the treatment of highly active relapsing-remitting multiple sclerosis (MS) in adults, only if: they have an unchanged or increased relapse rate or ongoing severe relapses compared with the previous year despite treatment with beta interferon <u>Issues with evidence:</u> • Patient populations in trial more broadly defined than in marketing authorisation • Accepted mixed treatment comparison and indirect evidence • Oral formulation as an innovation (Patient views accepted as evidence)

Example (continued): Rules of evidence and patient relevance

"Laboratory parameters alone are not usually considered patient relevant. […] we had big discussions about this in the case of hepatitis and the virus load […] do I have hepatitis if I can detect it [in the blood] or not? […] what is symptomatic, what the patient feels, quality of life etc. […] that is patient relevant" (Interview, FJC representative, 2013)

"[…] typically the industry fights with the FJC or IQWiG about the patient relevance. We say one thing, they say another, but de facto only patients can answer the question whether something is relevant for him" (Interview, Pharmaceutical manufacturer, 2013)

Impact

Germany:

- Controversy amongst stakeholders about how patient relevance and benefit categories are operationalized → Criticism from stakeholders that methods for operationalization are not scientifically validated
- How useful are the benefit categories in (clinical) practice? Has the system reduced prices for new medicines?
- Patient involvement to help identify what is patient relevant?

England:

- NICE more flexible in applying rules of evidence; incremental change in methods
- Not much progress with regard to disinvestment
- Cost effectiveness versus budget impact

Conclusion

- (Policy) implementation requires defining and operationalizing key principles: Patient relevance in Germany, cost effectiveness in England
- Conceptualization and operationalization takes place at ‘implementer’ (HTA body) level: This may lead to unintended consequences of a policy
- One of the ways in which a policy is implemented can be explained with reference to paradigms:

  - Cost effectiveness paradigm (England) reflects ideas enshrined in a tax-based health system that seeks to provide best possible services for the largest number of people whilst ensuring ‘value for money’;
  - Patient relevance paradigm (Germany) reflects ideas enshrined in a self-governing system that has negotiation and bargaining at its core

References

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