Methodological options for setting the health benefits package

Peter C. Smith

Imperial College London and

International Decision Support Initiative

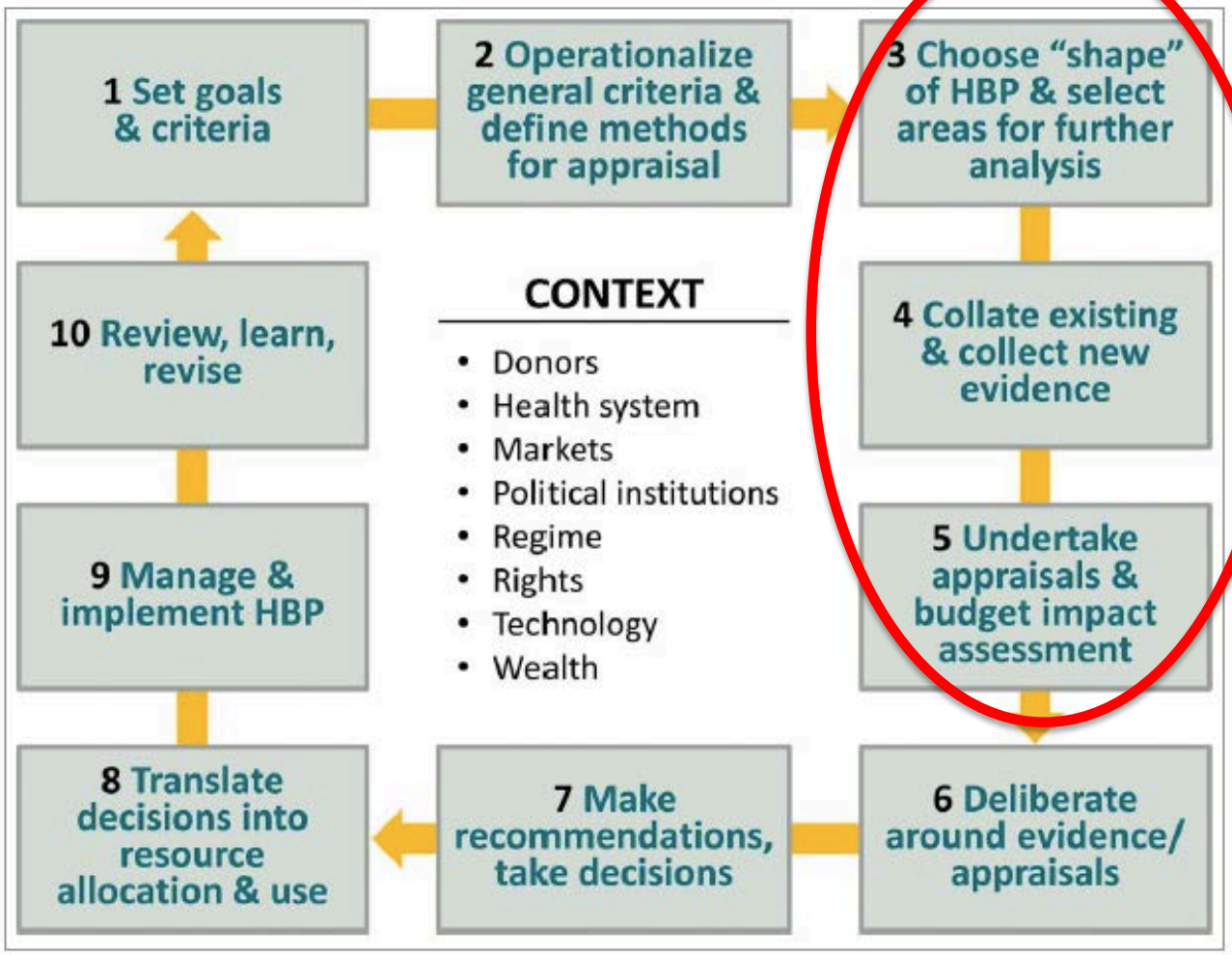
“Nobody knew that healthcare could be so complicated.”

Donald J. Trump, 27 February 2017

Purpose of this session

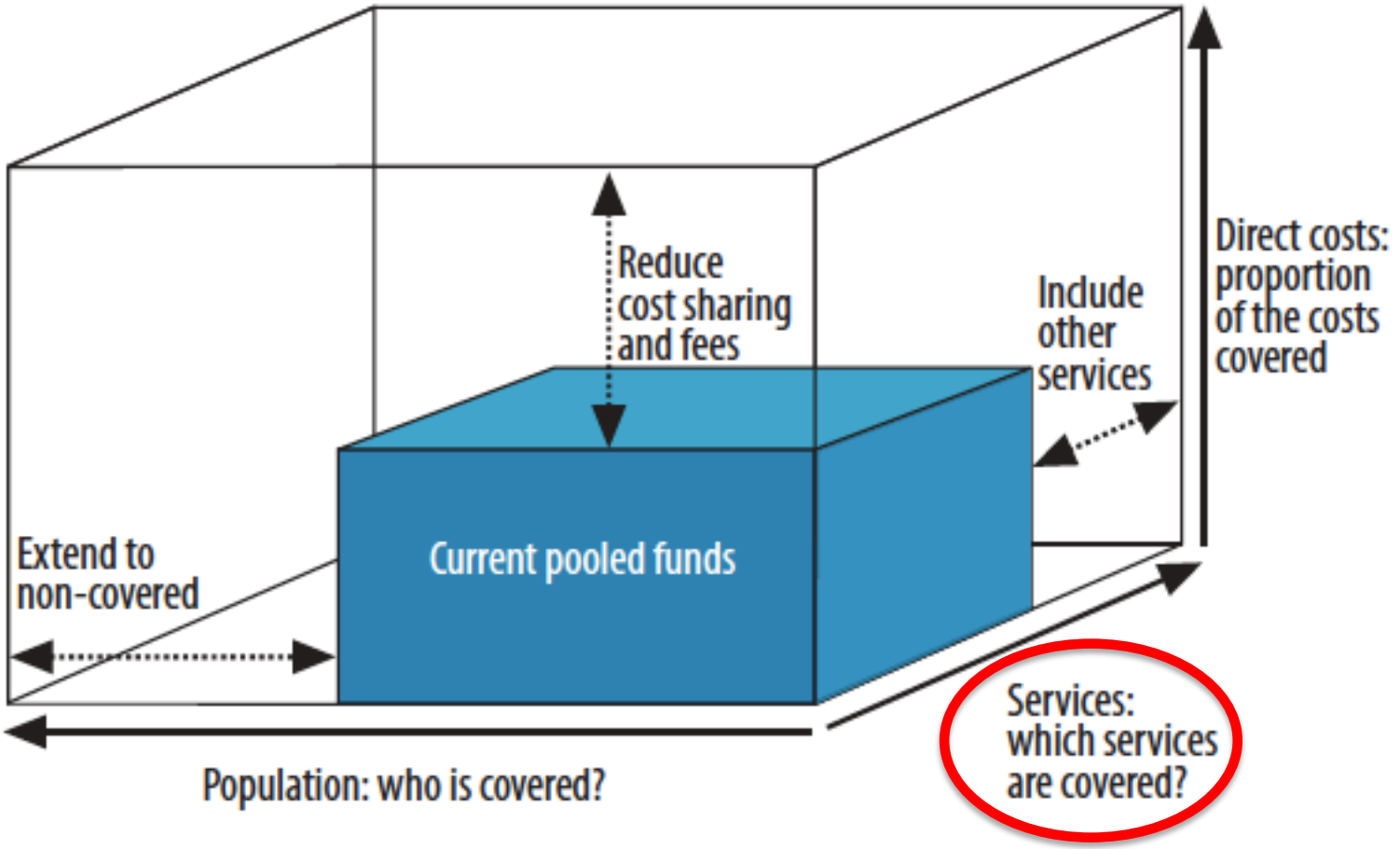
- To explain the role, the strengths and the limitations of analytic methods in informing the specification of the health benefits package
- To explore the most promising avenues for future development of methods
- Not intended as a methods tutorial

Ten Core Elements of Setting a Health Benefits Package



Glassman, A., Giedion, U., Sakuma, Y. and Smith, P. (2016), "Creating a health benefits package: what are the necessary processes?", *Health Systems and Reform,* 2(1), 31-50.

Three dimensions to consider when moving towards universal coverage



World Health Organization (2010), *World Health Report 2010*, WHO: Geneva

The role of analytic methods in informing the HBP

- Creation of HBP serious issue, with consequences for the health, life prospects and finances of affected individuals
- Ultimately a profound political problem
- Analytic methods can contribute by:
  - Acting as a 'referee' between competing claims for limited resources
  - Protecting politicians and other policy makers from impossible demands of competing claims for health services
  - Clarifying priorities and trade-offs (e.g. equity)
  - Facilitating accountability, transparency and consistency
  - Using evidence to best effect
  - Focusing attention where it is most needed
  - Demonstrating that health service funds are spent wisely

Analytic methods in context

- Should always be informed by legitimate policy choices
- Their key role is to apply chosen criteria consistently and universally
- Methods seek to maximize the ‘value’ obtained from limited health system resources
- Transparency should be intrinsic to analysis
- Recognize limitations to data, research and analytic capacity
- Analytic evidence should usually be considered alongside other contextual evidence and constraints.

Key choices when applying analytic methods

- What is ‘value’?
  - Health
  - Financial protection
  - Other
- What are available resources?
- What are other constraints to choices?
- How is ‘equity’ to be interpreted?
- What time period is under consideration?

The key concept of ‘opportunity cost’

- Whatever methods are used, some groups will gain (from inclusions in the HBP) and others will lose (through exclusions from the HBP)
- Gains from inclusions may be reflected in:
  - health (access to services that would not otherwise have been available)
  - finance (zero charges for services that would otherwise be charged for)
- These gains must be compared with the opportunity costs for those whose medical needs are excluded from the HBP
- ‘Externalities’ and other considerations complicate matters for infectious diseases

Outline of methods

1. Cost-effectiveness analysis
   - Measurement of health benefits
   - Measurement of costs
   - The cost-effectiveness threshold
2. Extended cost-effectiveness analysis
3. Multiple objectives
4. Non-budgetary constraints
5. Assessment of evidence relevance and limitations
6. Setting analytic priorities

1. Cost-effectiveness analysis (CEA)

- Based on the principle of *constrained maximization* of benefits with respect to a fixed budget
- Seeking to capture the *incremental* costs and benefits of a health service intervention
- So must always evaluate with respect to a *comparator* (which may often be 'do nothing')
- Usually assumes interventions are *independent* of each other
- The *incremental cost-effectiveness ratio* (ICER) is a key metric for any intervention
- Interventions are *ranked* according to their ICERs, and included until the budget is exhausted
- Resources:
  - Drummond, Michael F., Mark J. Sculpher, George W. Torrance, Bernie J. O'Brien, and Greg L. Stoddart. Methods for the Economic Evaluation of Health Care Programmes. 3 edition. Oxford; New York: Oxford University Press, 2005.
  - Jamison, Dean T., Joel G. Breman, Anthony R. Measham, George Alleyne, Mariam Claeson, David B. Evans, Prabhat Jha, Anne Mills, and Philip Musgrove. Disease Control Priorities in Developing Countries. The International Bank for Reconstruction and Development / The World Bank, 2006. https://www.ncbi.nlm.nih.gov/books/NBK11728/

CEA – Measuring benefits

- Challenging to model lifetime health gains, but methods well developed for many NCDs; increasing use for infectious diseases
- Generally accepted principles of quality-adjusted life years (QALYs) or DALYs
  - Life expectancy
  - Health-related quality of life
- Have to make some assumption about 'quality' (effectiveness of service delivery)
- Health gains to identical individuals should be universally similar (subject to similar service quality) so benefits calculations often transferrable between settings
- Special challenges for infectious diseases
  - Externalities
  - Dependent on epidemiology, behaviour etc

CEA – Measuring costs

- In principle should seek out opportunity costs
- Accounting costs usually used as a proxy
- Cost structures complex for many health services
  - Much early CEA work on pharmaceuticals, for which infrastructure costs relatively low
  - Infrastructure costs shared by many interventions
  - Economies of scale and scope
  - Often highly dependent on local service organization, so may not be readily transferrable between settings
- Costing tools beginning to emerge:
  - WHO OneHealth http://www.who.int/choice/onehealthtool/en/
  - JLN Costing Toolkit http://www.jointlearningnetwork.org/resources/costing-manual-tool-kit
- Costing also essential for
  - calculating budget impact
  - pricing and provider payment

CEA – the cost-effectiveness threshold

- The threshold indicates the ICER of the *marginal intervention*, just included in the HBP
- Any intervention with a higher ICER should be excluded
- The *level of the threshold* depends on a country's epidemiology, budget availability, and the range of therapies under consideration
- The threshold is useful because it acts as a rigorous *rule of thumb* for considering interventions piecemeal, not requiring re-assessment of the entire HBP
- The level of the threshold may change (reduce) if a treatment with *high budget impact* is introduced into the HBP

Quantifying and handling uncertainty in CEA

- Uncertainty intrinsic to all analysis
- Can arise from numerous sources:
  - Limitations in evidence from cost-effectiveness studies (e.g. sample size; target population; country setting; date of study)
  - Limitations in modelling methods used (model structure, parameters used)
  - Uncertainty about effectiveness with which health services will be delivered
  - Uncertainty about which population groups will use the treatment and heterogeneity in their benefits or costs
- Increasingly sophisticated methods for modelling and presenting uncertainty
- Often an important factor in decision-making, especially when deferral of decision is possible

- Griffin, S. and Claxton, K. "Analyzing uncertainty in cost-effectiveness for decision-making", in Glied, S. and Smith, P. (eds) (2011), *The Oxford handbook of health economics*, Oxford: Oxford University Press.

2. Extended CEA (Verguet and Jamison chapter)

- Extends the principle of conventional CEA to reflect (a) equity and (b) financial protection
- Calculates measures of financial loss averted by including the treatment in the HBP
- Reports health gains and financial gains by income group
- Leaves reporting disaggregated to allow decision-makers to take the different outcomes into account – does not seek to summarize benefits

Stylized example of ECEA from Verguet and Jamison

Table 2. Extended cost-effectiveness analysis (ECEA) results for universal public finance of tuberculosis treatment to 40 + 10% coverage (per 1,000,000 population).

Outcome	Total	Income Quintile I	Income Quintile II	Income Quintile III	Income Quintile IV	Income Quintile V
TB deaths averted	90	36	27	18	9	0
Private expenditures averted	40,000	16,000	12,000	8,000	4,000	0
Poverty cases averted	34	34	0	0	0	0

Examining the efficient purchase of health, equity, and non-health benefits, we find: $ICER = \$520$ per death averted, $ICER_{FRP} = \$1{,}470$ per poverty case averted, and $ICER_{Eq} = \$125{,}000$ per equity ratio (when simple metric of the ratio between the health benefits among the poorest and the total sum of the health benefits is used). Scaling per \$1,000,000 spent, we obtain 1,800 deaths averted, 720 of which among the bottom income quintile, and 680 poverty cases averted, all of which among the bottom income quintile.

3. Multiple objectives in CEA (Morton and Lauer chapter)

- Increased interest in 'multi-criteria decision analysis' (MCDA)
- Reflects concern that health improvement may not be the only objective of concern
  - E.g. workforce productivity
- ECEA first steps toward a theoretically coherent approach
- MCDA a more heuristic and flexible approach that allows inclusion and aggregation of multiple objectives
  - Well-established outside health care sector
  - Guidelines on good practice

Issues with implementing MCDA

- Who should influence choice and weight of criteria?
- What should those criteria be?
- How should attainment on the criteria be measured?
- What should be the weight placed on each additional unit of attainment for each criterion?
- Can MCDA be applied to all interventions under consideration?
- Profound methodological challenges
- Profound implementation challenges

From Morton and Lauer chapter

Table 4. Comparison of different methods

	CEA	MCDA	ECEA	CBA
Reflective of social values.	Methods assume that population health gain is the overriding objective.	In principle, method can take into account any possible social values, but care should be taken in structuring the criteria.	Method reflects a key concern in LMICs where avoidance of catastrophic financial payments is important alongside population health gain.	Methods involve modelling all-welfare relevant consequences. Opponents argue that CBA embeds unacceptable value tradeoffs.
Technically robust and justifiable.	Method is very well-established within the healthcare sector. Guidelines for good practice exist, including the international reference case, although methodological controversies remain.	Method is well-established outside the healthcare sector and popular within the healthcare sector. Several general (ie nonhealthcare specific) good practice guidelines exist, but healthcare specific guidelines are under in a process of development.	Method is new and established guidelines on good practice do not yet exist.	Method is well-established outside the healthcare sector Several general (ie nonhealthcare specific) good practice guidelines exist, but there is not yet a strong body of healthcare specific guidelines.
Easy to understand	Methods can be implemented at various levels of sophistication: more complicated models will be harder for lay people to engage with.	Ease of understanding is one of the principle selling points for these methods. However, appropriately structuring criteria and choosing aggregation rules is subtler than is often appreciated.	Same comments apply as in the case of CEA but with the proviso that some of the additional financial modelling (in particular the concept of insurance value) adds an additional layer of complexity.	Models can be very technical and expression of costs and benefits in monetary terms is often a stumbling block for lay engagement.
Have low cost of implementation.	Can be done at varying levels of intensity, from "quick and dirty" to more expensive and robust analyses.	Does not require specialised modelling resources but requires relatively intensive engagement from stakeholders to supply scores and weights.	Same comments apply as in the case of CEA but with the additional proviso that modelling of financial and payment aspects is required. Equity analysis requires disaggregated data which is often demanding.	Same comments apply as in the case of CEA and ECEA but requires a more extensive modelling of welfare consequences across a broader range of economic sectors.

4. Non-budgetary constraints (Hauck, Thomas and Smith chapter)

- Six categories of impediment to implementing CEA recommendations:
  - Design of the health system (eg human resource constraints)
  - Costs of implementing change
  - System interdependencies between interventions (eg shared platforms)
  - Uncertainty
  - Weak governance
  - Political constraints

An example: system interdependencies

	Treatment 1		Total			
Allocation fixed costs per case	882.4		882.4		Fixed costs	7,500,000
Variable costs per case	200.0		200.0		Var costs	1,700,000
Incremental benefits (QALYs)	7.0				TOTAL	9,200,000
Number of cases	8500		8500			
Total Cost/QALY	154.6					
Variable Cost/QALY	28.6					
	Treatment 1	**Treatment 2**	**Total**			
Allocation fixed costs per case	357.1	357.1	357.1		Fixed costs	7,500,000
Variable costs per case	200.0	500.0	378.6		Var costs	7,950,000
Incremental benefits (QALYs)	7.0	5.0			TOTAL	15,450,000
Number of cases	8500	12500	21000			
Total Cost/QALY	79.6	171.4	134.3			
Variable Cost/QALY	28.6	100.0	71.1			
Threshold	140					

Table 1: Six constraints and proposed solutions to incorporate them into Cost-effectiveness Analysis

Constraint	Solution
Health system design constraint	• Requires institutional adjustments, but can be incorporated into CEA analytically via: • Analyse supply- and demand-side responses • Incorporate multiple resource constraints into the mathematical modelling
Implementation costs	• Incorporate transition costs into the mathematical modelling • Disaggregate costs to highlight major cost components
System interactions	• Model interactions between interventions by incorporating economies of scope • Model intervention under alternative scenarios (with and without complementary intervention) • Present rage of CE ratios dependant on prevailing system configuration
Uncertainty	• Conduct probabilistic sensitivity analysis • Present extent of uncertainty via cost-effectiveness acceptability curves • Address structural uncertainty with sensitivity analyses • Commission additional research • Evaluate robustness of decisions under alternative future scenarios
Governance constraints	• Requires institutional adjustments, and difficult to incorporate into CEA analytically, but possibly: • Constrain the number of decisions that can be made in a given time period
Political constraints	• Requires institutional adjustments, possibly: • Devolve process of priority setting to agencies with politically determined terms of reference • Public involvement in decision making

5. Assessment of evidence relevance and limitations
Hawkins, Heggie and Wu chapter

- Increased interest in what constitutes ‘relevant’ evidence for CEA, and how it might be incorporated into creation of the HBP
- Relevance might be related to:
  - Treatment under scrutiny and its comparator
  - Quality of study
  - Population group
  - Geography
  - Date of study
  - Health system setting
- General principle is to allow all ‘relevant’ evidence to inform decision

Analytic approaches towards assessment of evidence

- Systematic reviews and searches
  - Eg snowballing; pearl growing
- Assessment of internal and external validity
  - validity testing tools eg EVAT external validity assessment tool
- Meta-analysis and other aggregation tools
- Sensitivity analysis
- 'Value of information' analysis
  - Identifying priorities for new or augmented data
- Creating evidence
  - Commissioning research
  - Monitoring and evaluation after implementation

6. Setting analytic priorities

- Limited local analytic capacity
- Need to prioritize topics
  - Always political priority topics!
  - But also topics where the budget impact is large
  - … or the cost-effectiveness is close to your likely threshold
- In principle, treatments currently in the HBP but candidates for exclusion should also be considered
- New evidence may prompt reconsideration
- New research studies
- Assessing monitoring evidence from implementation

Towards standardizing CEA – the international reference case

- Principles of Economic Evaluation
  - Transparency
  - Comparators
  - Use of Evidence
  - Measure of outcome
  - Measurement of costs
  - Time horizon for costs and effects
  - Costs and Effects outside health
  - Heterogeneity
  - Uncertainty
  - Impact on other constraints and budget impact
  - Equity implications

The Reference Case for Economic Evaluation (2015)
Tommy Wilkinson, Kalipso Chalkidou, Karl Claxton, Paul Revill, Mark Sculpher, Andrew Briggs, Yot Teerawattananon, Waranya Rattanavipapong

http://www.idsihealth.org/knowledge_base/the-reference-case-for-economic-evaluation/

Contribution of methods to creation of the HBP

- Clarify nature of choices to be made
- Make political preferences operational
- Create a ‘level playing field’ for patients, providers and manufacturers
- Promote consistency, transparency and stability
- Synthesize available evidence
- Identify priorities for new evidence
- Maximize ‘value’ secured from health system
- Promote confidence that health system finances are spent wisely

Acknowledgements

- Amanda Glassman, Ursula Giedion, Kalipso Chalkidou, Tommy Wilkinson, Paul Revill
- Book chapter authors:
  - Mark Sculpher; Paul Revill; Jessica Ochalek; Karl Claxton; Cheryl Cashin; Annette Özaltın; Alec Morton; Jeremy A. Lauer; Stéphane Verguet; Dean T. Jamison; Katharina Hauck; Ranjeeta Thomas; Neil Hawkins; Robert Heggie; Olivia Wu
- Commentaries
  - Gerald Manthalu; Dominic Nkhoma; Jessica Ochalek; Andrew Phillips; Paul Revill; Yot Teerawattananon; Nattha Tritasavit; Sitaporn Youngkong; Suradech Doungthipsirikul; Tran Thi Mai Oanh; Khuong Anh Tuan; Nguyen Khanh Phuong; Waranya Rattanavipapong
- International Decision Support Initiative (iDSI)
  - funded by the Bill & Melinda Gates Foundation, the UK Department for International Development, and the Rockefeller Foundation.