Reflections on Value Based Pricing / Assessment

Professor Adrian Towse

Director, Office of Health Economics

AES 18th June 2015
Granada

Office of Health Economics
Research & Consulting

Agenda

- VBP context
- Regulating pharmaceuticals
  - VBP versus VBA, PBRSA's, MIPS
- What do we value?
  - Eliciting social preferences
- Aggregating elements of value
- Threshold and decision making in the NHS
- Reforming the Cancer Drugs Fund
- Conclusions

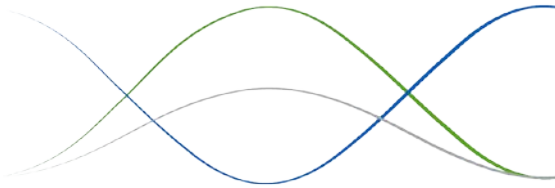
Value Based Pricing (VBP) Context

VBP was initially proposed in an Office of Fair Trading Report on the Pharmaceutical Price Regulation Scheme (PPRS)

VBP initially proposed by the Government was intended to:

1. Introduce a broader definition of value
2. Replace NICE appraisal with an algorithm
3. Impose / negotiate prices with the industry
4. End the 5 year PPRS negotiated agreements
5. Get rid of “no” or “restricted/optimised” decisions from NICE (and so get rid of anti-NICE, anti-DH Daily Mail headlines)
6. Enable the Cancer Drugs Fund to be got rid of

Agenda



- VBP context
- **Regulating pharmaceuticals**
  - **VBP versus VBA, PBRSA's, MIPS**
- What do we value?
  - Eliciting social preferences
- Aggregating elements of value
- Threshold and decision making in the NHS
- Reforming the Cancer Drugs Fund
- Conclusions

VBP versus Value Based Assessment

- Health systems should set price (WTP) for health gain reflecting insurees preferences
- Optimal global R&D comes from prices reflecting value at local CE thresholds for patent duration
- Price setting by governments/HTA bodies can lead to:
  - commercial uncertainty
  - opportunistic behaviour

HEALTH ECONOMICS
Health Econ. (2013)
Published online in Wiley Online Library (wileyonlinelibrary.com). DOI: 10.1002/hec.3021

VALUE-BASED DIFFERENTIAL PRICING: EFFICIENT PRICES FOR DRUGS IN A GLOBAL CONTEXT

PATRICIA DANZON[a,*], ADRIAN TOWSE[b] and JORGE MESTRE-FERRANDIZ[b]
[a]*The Wharton School, University of Pennsylvania, Philadelphia, PA, USA*
[b]*Office of Health Economics, London, UK*

ABSTRACT

This paper analyzes pharmaceutical pricing between and within countries to achieve second-best static and dynamic efficiency. We distinguish countries with and without universal insurance, because insurance undermines patients' price sensitivity, potentially leading to prices above second-best efficient levels. In countries with universal insurance, if each payer unilaterally sets an incremental cost-effectiveness ratio (ICER) threshold based on its citizens' willingness-to-pay for health; manufacturers price to that ICER threshold; and payers limit reimbursement to patients for whom a drug is cost-effective at that price and ICER, then the resulting price levels and use within each country and price differentials across countries are roughly consistent with second-best static and dynamic efficiency. These value-based prices are expected to differ cross-nationally with per capita income and be broadly consistent with Ramsey optimal prices. Countries without comprehensive insurance avoid its distorting effects on prices but also lack financial protection and affordability for the poor. Improving pricing efficiency in these self-pay countries includes improving regulation and consumer information about product quality and enabling firms to price discriminate within and between countries. © 2013 The Authors. *Health Economics* published by John Wiley & Sons Ltd.

Received 12 June 2012; Revised 11 July 2013; Accepted 6 November 2013

KEY WORDS: differential pricing; ICER thresholds; value-based pricing

1. INTRODUCTION

Achieving efficient pricing of pharmaceuticals between and within countries is a complex conceptual and policy problem. In any industry, pricing to maximize social welfare must consider both static efficiency (optimal use of existing products) and dynamic efficiency (optimal investment in research and development [R&D]). Reconciling these objectives is problematic for pharmaceuticals, for three reasons.

First, R&D is roughly 17% of sales for the US-based pharmaceutical industry, compared with 4% for other US industries, and other quasi-fixed costs of production are significant. Marginal cost pricing to achieve first-best static efficiency would fail to cover total costs and violate the dynamic efficiency requirement that producers capture the full social surplus produced by innovation. Patents enable firms to price above marginal cost and thus potentially achieve dynamic efficiency. This is 'second best' if pricing above marginal cost reduces utilization.

Second, in the case of pharmaceuticals, the effect of patents is both mitigated and distorted by insurance coverage in most industrialized countries. By lowering out-of-pocket prices to patients, insurance potentially brings utilization closer to first-best levels. However, by making patient demand highly price-inelastic, insurance creates the potential and incentives for manufacturers to set prices above second-best optimal levels. By contrast, patients in self-pay markets (including many middle and lower income countries [MLICs]) lack the financial protection of insurance but also avoid its distorting effects on prices. However, other factors—including uncertain product quality and skewed income distributions—contribute to drug prices that may exceed second-best optimal levels (Hynn *et al.*, 2009; Danzon *et al.*, 2011).

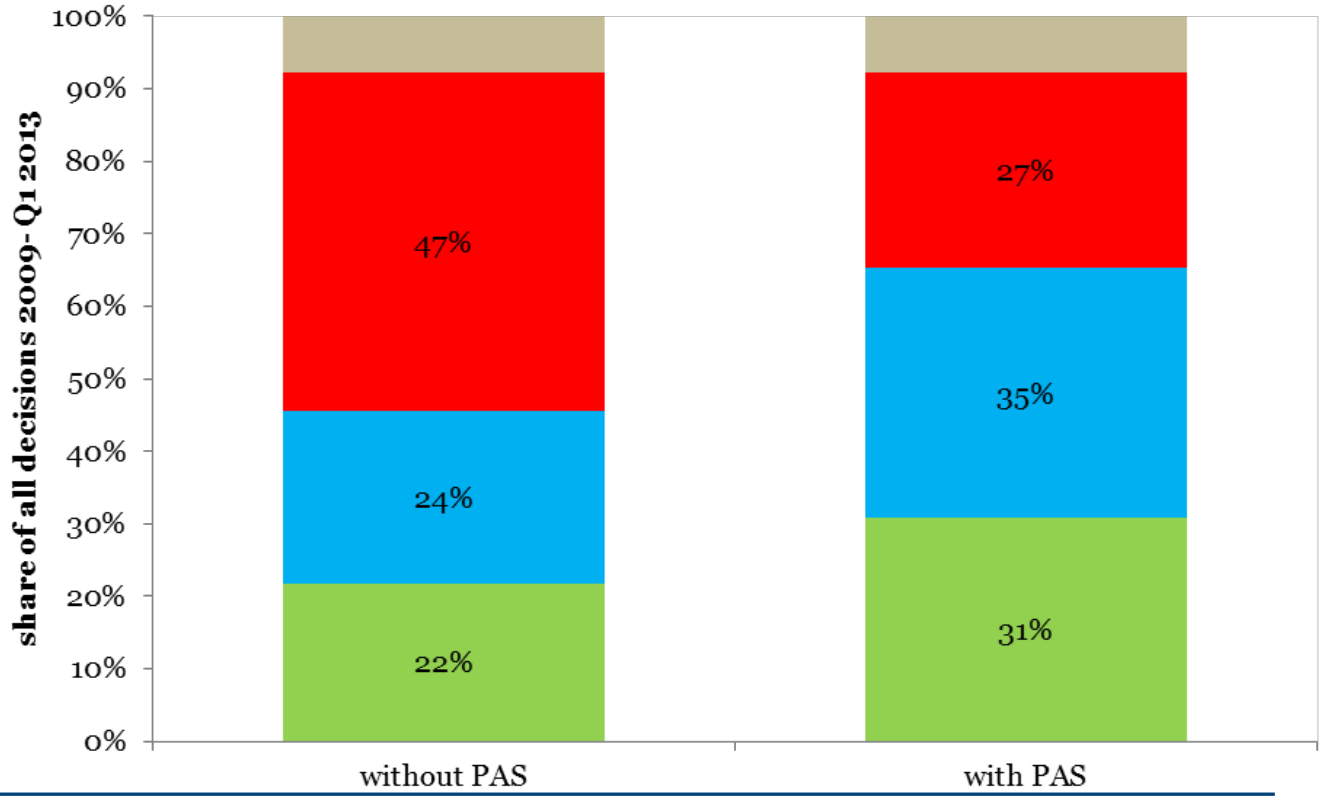
*Correspondence to: Wharton Health Care Systems Department, University of Pennsylvania, 3641 Locust Walk, Philadelphia, PA, 19104, USA. E-mail: danzon@wharton.upenn.edu

Office of Health Economics
Research & Consulting

Impact of patient access schemes

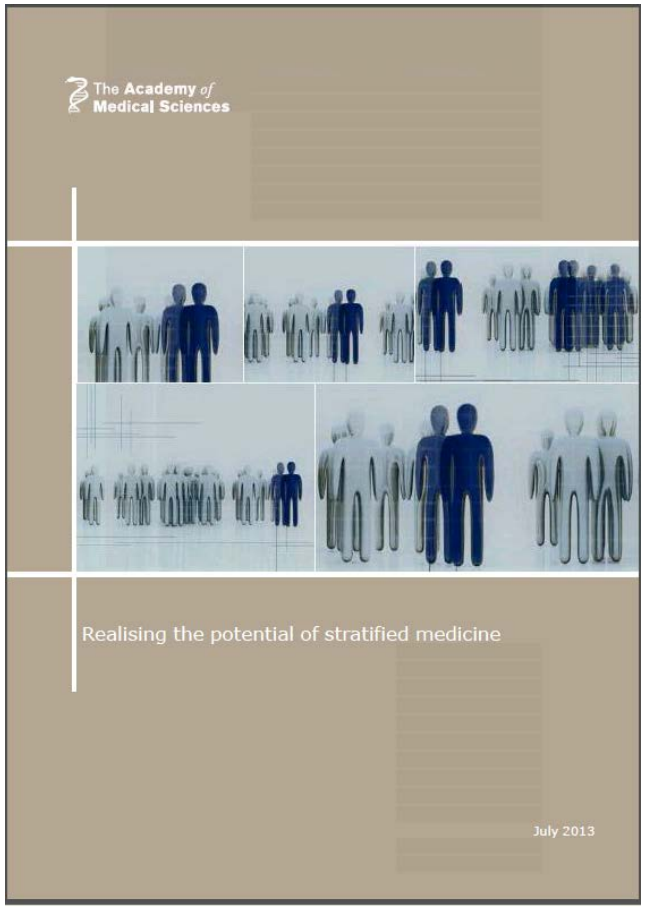
If all positive decisions since 2009 where a PAS was implemented were assumed to be a "not recommended" decision in the absence of a PAS (bar labelled "without PAS") the share of not recommended decisions increases to 47%

Chart: share of decision outcome for all medicines decisions from 2009 to Q3 2013, with and "without" PAS



Source: OHE analysis from NICE website

Need for flexible pricing, multi-indication pricing and more Performance-based risk sharing agreements (outcomes-based Patient Access Schemes)



VALUE IN HEALTH 16 (2013) 703–719

Available online at www.sciencedirect.com

ScienceDirect

ELSEVIER

journal homepage: www.elsevier.com/locate/jval

ISPOR TASK FORCE REPORTS

Performance-Based Risk-Sharing Arrangements—Good Practices for Design, Implementation, and Evaluation: Report of the ISPOR Good Practices for Performance-Based Risk-Sharing Arrangements Task Force

Louis P. Garrison Jr., PhD (co-chair)[1,*], Adrian Towse, MA, MPhil (co-chair)[2], Andrew Briggs, MSc, DPhil[3], Gerard de Pouvourville, PhD[4], Jens Grueger, PhD[5], Penny E. Mohr, MA[6], J.L. (Hans) Severens, PhD[7], Paolo Siviero, BA[8], Miguel Sleeper, ACMA[9]

[1]Pharmaceutical Outcomes Research & Policy Program, Department of Pharmacy, University of Washington, Seattle, WA, USA; [2]Office of Health Economics, London, UK; [3]Institute of Health and Wellbeing, University of Glasgow, Glasgow, UK; [4]ESSEC Business School, Cergy Pontoise, France; [5]F. Hoffmann - La Roche AG, Basel, Switzerland; [6]Center for Medical Technology Policy, Baltimore, Maryland, USA; [7]Institute of Health Policy & Management, Erasmus University Rotterdam, Rotterdam, The Netherlands; [8]Agenzia Italiana del Farmaco, Rome, Italy; [9]Access to Medicines GlaxoSmithKline plc, Brentford, UK

ABSTRACT

There is a significant and growing interest among both payers and producers of medical products for agreements that involve a "pay-for-performance" or "risk-sharing" element. These payment schemes—called "performance-based risk-sharing arrangements" (PBRSAs)—involve a plan by which the performance of the product is tracked in a defined patient population over a specified period of time and the amount or level of reimbursement is based on the health and cost outcomes achieved. There has always been considerable uncertainty at product launch about the ultimate real-world clinical and economic performance of new products, but this appears to have increased in recent years. PBRSAs represent one mechanism for reducing this uncertainty through greater investment in evidence collection while a technology is used within a health care system. The objective of this Task Force report was to set out the standards that should be applied to "good practices"—both research and operational—in the use of a PBRSA, encompassing questions around the desirability, design, implementation, and evaluation of such an arrangement. This report provides practical recommendations for the development and application of state-of-the-art methods to be used when considering, using, or reviewing PBRSAs. Key findings and recommendations include the following. Additional evidence collection is costly, and there are numerous barriers to establishing viable and cost-effective PBRSAs: negotiation, monitoring, and evaluation costs can be substantial. For good research practice in PBRSAs, it is critical to match the appropriate study and research design to the uncertainties being addressed. Good governance processes are also essential. The information generated as part of PBRSAs has public good aspects, bringing ethical and professional obligations, which need to be considered from a policy perspective. The societal desirability of a particular PBRSA is fundamentally an issue as to whether the cost of additional data collection is justified by the benefits of improved resource allocation decisions afforded by the additional evidence generated and the accompanying reduction in uncertainty. The ex post evaluation of a PBRSA should, however, be a multidimensional exercise that assesses many aspects, including not only the impact on long-term cost-effectiveness and whether appropriate evidence was generated but also process indicators, such as whether and how the evidence was used in coverage or reimbursement decisions, whether budget and time were appropriate, and whether the governance arrangements worked well. There is an important gap in the literature of structured ex post evaluation of PBRSAs. As an innovation in and of themselves, PBRSAs should also be evaluated from a long-run societal perspective in terms of their impact on dynamic efficiency (eliciting the optimal amount of innovation).

Keywords: access with evidence development, conditional licensing, coverage with evidence development, managed entry schemes, outcomes-based, patient access schemes, pay for performance, performance-based risk-sharing arrangements, risk-sharing.

* Address correspondence to: Louis P. Garrison Jr., Pharmaceutical Outcomes Research & Policy Program, Department of Pharmacy, Health Sciences Building, H375 1959 NE Pacific Street, H-375A, Box 357630, University of Washington, Seattle, WA 98195, USA.
E-mail: lgarrison@u.washington.edu.

1098-3015/$36.00 – see front matter Copyright © 2013, International Society for Pharmacoeconomics and Outcomes Research (ISPOR). Published by Elsevier Inc.
http://dx.doi.org/10.1016/j.jval.2013.04.011

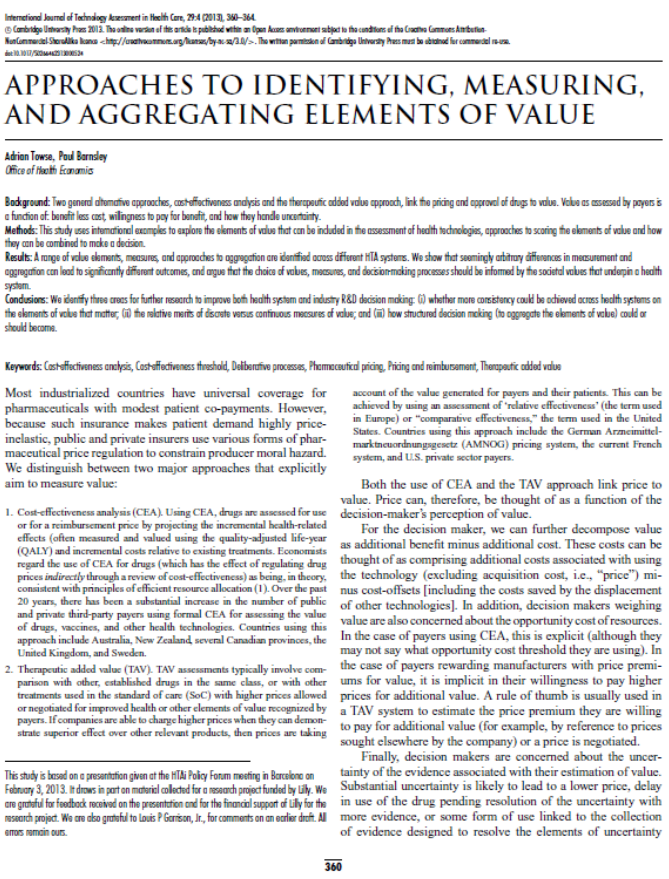
Office of Health Economics
Research & Consulting

Agenda

- VBP context
- Regulating pharmaceuticals
  - VBP versus VBA, PBRSA's, MIPS
- **What do we value?**
  - **Eliciting social preferences**
- Aggregating elements of value
- Threshold and decision making in the NHS
- Reforming the Cancer Drugs Fund
- Conclusions

What do we value?

- A lot of variation in what is valued by payers / HTA
- Core is (i) health gain (life extending, improved health status) (ii) reducing system cost
- How far beyond this?
- Is this decided by:
  1. The (extra-welfarist) decision maker
  2. The (welfarist) search for social / individual preferences
  3. Or 1. informed by 2.?



International Journal of Technology Assessment in Health Care, 29:4 (2013), 360–364.

doi:10.1017/S0266462313000524

APPROACHES TO IDENTIFYING, MEASURING, AND AGGREGATING ELEMENTS OF VALUE

Adrian Towse, Paul Barnsley
Office of Health Economics

Background: Two general alternative approaches, cost-effectiveness analysis and the therapeutic added value approach, link the pricing and approval of drugs to value. Value as assessed by payers is a function of: benefit less cost, willingness to pay for benefit, and how they handle uncertainty.
Methods: This study uses international examples to explore the elements of value that can be included in the assessment of health technologies, approaches to scoring the elements of value and how they can be combined to make a decision.
Result: A range of value elements, measures, and approaches to aggregation are identified across different HTA systems. We show that seemingly arbitrary differences in measurement and aggregation can lead to significantly different outcomes, and argue that the choice of values, measures, and decision-making processes should be informed by the societal values that underpin a health system.
Conclusions: We identify three areas for further research to improve both health system and industry R&D decision making: (i) whether more consistency could be achieved across health systems on the elements of value that matter; (ii) the relative merits of discrete versus continuous measures of value; and (iii) how structured decision making (to aggregate the elements of value) could or should become.

Keywords: Cost-effectiveness analysis, Cost-effectiveness threshold, Deliberative processes, Pharmaceutical pricing, Pricing and reimbursement, Therapeutic added value

Most industrialized countries have universal coverage for pharmaceuticals with modest patient co-payments. However, because such insurance makes patient demand highly price-inelastic, public and private insurers use various forms of pharmaceutical price regulation to constrain producer moral hazard. We distinguish between two major approaches that explicitly aim to measure value:

1. Cost-effectiveness analysis (CEA). Using CEA, drugs are assessed for use or for a reimbursement price by projecting the incremental health-related effects (often measured and valued using the quality-adjusted life-year (QALY) and incremental costs relative to existing treatments. Economists regard the use of CEA for drugs (which has the effect of regulating drug prices *indirectly* through a review of cost-effectiveness) as being, in theory, consistent with principles of efficient resource allocation (1). Over the past 20 years, there has been a substantial increase in the number of public and private third-party payers using formal CEA for assessing the value of drugs, vaccines, and other health technologies. Countries using this approach include Australia, New Zealand, several Canadian provinces, the United Kingdom, and Sweden.
2. Therapeutic added value (TAV). TAV assessments typically involve comparison with other, established drugs in the same class, or with other treatments used in the standard of care (SoC) with higher prices allowed or negotiated for improved health or other elements of value recognized by payers. If companies are able to charge higher prices when they can demonstrate superior effect over other relevant products, then prices are taking account of the value generated for payers and their patients. This can be achieved by using an assessment of 'relative effectiveness' (the term used in Europe) or "comparative effectiveness," the term used in the United States. Countries using this approach include the German Arzneimittelmarktneuordnungsgesetz (AMNOG) pricing system, the current French system, and U.S. private sector payers.

Both the use of CEA and the TAV approach link price to value. Price can, therefore, be thought of as a function of the decision-maker's perception of value.

For the decision maker, we can further decompose value as additional benefit minus additional cost. These costs can be thought of as comprising additional costs associated with using the technology (excluding acquisition cost, i.e., "price") minus cost-offsets [including the costs saved by the displacement of other technologies]. In addition, decision makers weighing value are also concerned about the opportunity cost of resources. In the case of payers using CEA, this is explicit (although they may not say what opportunity cost threshold they are using). In the case of payers rewarding manufacturers with price premiums for value, it is implicit in their willingness to pay higher prices for additional value. A rule of thumb is usually used in a TAV system to estimate the price premium they are willing to pay for additional value (for example, by reference to prices sought elsewhere by the company) or a price is negotiated.

Finally, decision makers are concerned about the uncertainty of the evidence associated with their estimation of value. Substantial uncertainty is likely to lead to a lower price, delay in use of the drug pending resolution of the uncertainty with more evidence, or some form of use linked to the collection of evidence designed to resolve the elements of uncertainty

This study is based on a presentation given at the HTAi Policy Forum meeting in Barcelona on February 3, 2013. It draws in part on material collected for a research project funded by Lilly. We are grateful for feedback received on the presentation and for the financial support of Lilly for the research project. We are also grateful to Louis P Garrison, Jr., for comments on an earlier draft. All errors remain ours.

Eliciting social preferences: End of life findings highlight the challenges

EEPRU Policy Research Unit in Economic Evaluation of Health and Social Care Interventions

Research Report

Title

Eliciting societal preferences for burden of illness, therapeutic improvement and end of life for value based pricing: a report of the main survey

Authors

John Brazier[a], Donna Rowen[a], Clara Mukuria[a], Sophie Whyte[a], Anju Keetharuth[a], Arne Rise Hole[b], Aki Tsuchiya[a,b], Phil Shackley[a]

Number 01/13

Date: 10 October 2013

The University Of Sheffield.

THE UNIVERSITY of York

Eur J Health Econ
DOI 10.1007/s10198-013-0482-3

ORIGINAL PAPER

Valuing health at the end of life: an empirical study of public preferences

Koonal K. Shah · Aki Tsuchiya · Allan J. Wailoo

Received: 29 June 2012 / Accepted: 8 April 2013

Abstract In 2009, the National Institute for Health and Clinical Excellence (NICE) issued supplementary advice to its Appraisal Committees to be taken into account when appraising life-extending, 'end-of-life' treatments. This indicated that if certain criteria are met, it may be appropriate to recommend the use of such treatments even if they would not normally be considered cost-effective. However, NICE's public consultation revealed concerns that there is little scientific evidence to support such a policy. This study examines whether there is public support for giving higher priority to life-extending, end-of-life treatments than to other types of treatment. In face-to-face interviews, respondents answered six questions asking them to choose which of two hypothetical patients they would prefer to treat, assuming that the health service has enough funds to treat one but not both of them. The various scenarios were designed so as to control for age- and time-related preferences. Fifty members of the general public in England were interviewed in July 2011. We find some evidence of support for giving priority to the patient with shorter remaining life expectancy, but note that a nontrivial minority of respondents expressed the opposite preference. Substantial preference for quality-of-life improvement over life extension was observed. V respondents expressed indifference or unwilling choose between the patients. Whilst there cannot b bed to be a single 'consensus' set of preferences, wee that there are ways in which the results suggest current NICE policy may be insufficient.

Keywords UK · End of life · NICE · Distributio preferences · Severity

JEL Classification I18

Introduction

Background

The National Institute for Health and Clinical Ex (NICE) is responsible for producing advice on th new and existing health technologies to the Nationa Service (NHS) in England and Wales. NICE's Tec Appraisals are guided by clinical and cost-effec analyses, usually using the quality-adjusted li (QALY) [1] to measure health outcomes. Curren lines used by NICE [2] define a 'reference case' whereby all equal-sized health gains are of equa value, regardless of to whom they accrue and the co which they are enjoyed. As well as evaluating the s evidence, those responsible for formulating NICE also need to make social value judgements [3]. Th concerned with what is appropriate and accept society in delivering health care across the NHS.

In January 2009, NICE issued supplementary a its Appraisal Committees (independent con responsible for formulating NICE guidance based

K. K. Shah (✉)
Office of Health Economics, 7th floor Southside, 105 Victoria Street, London SW1E 6QT, UK
e-mail: kshah@ohe.org

K. K. Shah · A. Tsuchiya · A. J. Wailoo
School of Health and Related Research, University of Sheffield, Regent Court, 30 Regent Street, Sheffield S1 4DA, UK

A. J. Wailoo
e-mail: a.j.wailoo@sheffield.ac.uk

A. Tsuchiya
Department of Economics, University of Sheffield, 9 Mappin Street, Sheffield S1 4DT, UK
e-mail: a.tsuchiya@sheffield.ac.uk

Published online: 09 May 2013

HEALTH ECONOMICS
Health Econ. (2012)
Published online in Wiley Online Library (wileyonlinelibrary.com). DOI: 10.1002/hec.2872

SOCIETAL VIEWS ON NICE, CANCER DRUGS FUND AND VALUE-BASED PRICING CRITERIA FOR PRIORITISING MEDICINES: A CROSS-SECTIONAL SURVEY OF 4118 ADULTS IN GREAT BRITAIN

WARREN G. LINLEY and DYFRIG A. HUGHES*
Centre for Health Economics & Medicines Evaluation, Institute of Medical and Social Care Research, Bangor University, Bangor, UK

ABSTRACT

The criteria used by the National Institute for Health and Clinical Excellence (NICE) for accepting higher incremental cost-effectiveness ratios for some medicines over others, and the recent introduction of the Cancer Drugs Fund (CDF) in England, are assumed to reflect societal preferences for National Health Service resource allocation. Robust empirical evidence to this effect is lacking. To explore societal preferences for these and other criteria, including those proposed for rewarding new medicines under the future value-based pricing (VBP) system, we conducted a choice-based experiment in 4118 UK adults via web-based surveys. Preferences were determined by asking respondents to allocate fixed funds between different patient and disease types reflecting nine specific prioritisation criteria. Respondents supported the criteria proposed under the VBP system (for severe diseases, address unmet needs, are innovative—provided they offered substantial health benefits, and have wider societal benefits) but did not support the end-of-life premium or the prioritisation of children or disadvantaged populations as specified by NICE, nor the special funding status for treatments of rare diseases, nor the CDF. Policies introduced on the basis of perceived—and not actual—societal values may lead to inappropriate resource allocation decisions with the potential for significant population health and economic consequences. Copyright © 2012 John Wiley & Sons, Ltd.

Received 30 November 2011; Revised 17 July 2012; Accepted 14 August 2012

KEY WORDS: NICE; value-based pricing; cancer drugs fund; orphan drugs; equity; health care rationing; public preferences

1. INTRODUCTION

The UK National Health Service (NHS) has legal and moral obligations to provide fair, comprehensive, needs-based care for all [Department of Health, 2010a]. Given the unprecedented efficiency savings demanded across the NHS in recent and coming years [Department of Health, 2009; Institute for Fiscal Studies/Nuffield Trust, 2012], it is imperative that resource allocation decisions provide the most effective and sustainable use of finite resources. The National Institute for Health and Clinical Excellence (NICE) makes compulsory recommendations on the use of medicines and other health technologies in the NHS in England and Wales, with reference to their clinical and cost effectiveness. The funding of new medicines requires that other existing medicines or services are displaced, the opportunity cost of which is reflected in NICE's cost-effectiveness threshold, set at £20,000–£30,000 per quality-adjusted life-year (QALY) gained [National Institute for Health and Clinical Excellence, 2008a]. However, several medicines with incremental cost-effectiveness estimates in excess of this threshold range have been approved by NICE for use via the NHS (e.g., sunitinib for advanced renal cancer and rilruzole for motor neurone disease) [Rawlins *et al.*, 2010].

Justification for this departure from the usual cost-effectiveness threshold range includes the social value judgements of NICE's Citizen Council. On the basis of its views, six specific criteria besides clinical and

*Correspondence to: Centre for Health Economics & Medicines Evaluation, Institute of Medical and Social Care Research, Bangor University, Dean Street, Bangor, Gwynedd, UK. E-mail: d.a.hughes@bangor.ac.uk

Office of Health Economics Research & Consulting

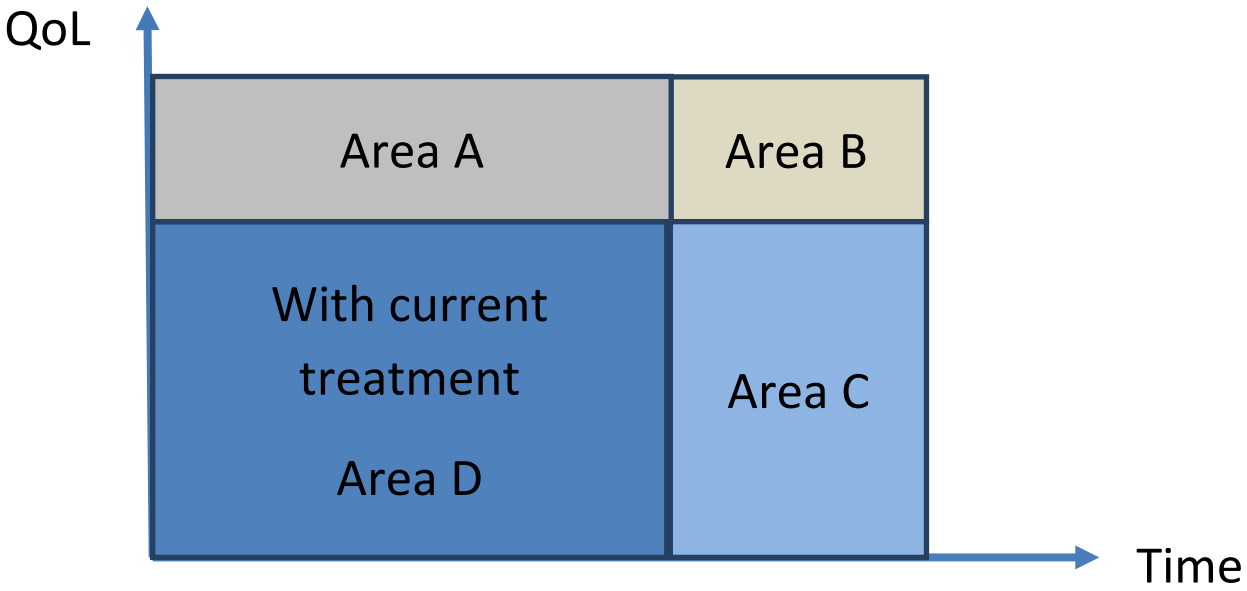
EEPRU work – approach and findings (i)

- Discrete Choice Experiment (DCE) with 3669 respondents
- Chose whether NHS should treat patient group A or B, who differed in terms of four attributes: life expectancy without treatment; HRQOL without treatment; survival gain from treatment; and HRQOL gain from treatment.
- These attributes were used to explore Therapeutic Improvement (TI), derive Burden of Illness (BOI), QALY gain and End of Life (EOL).
- Respondents preferred to treat patients with larger QALY gains, but at a diminishing rate meaning there was no support for TI
- Respondents preferred to treat patients with a shorter life expectancy (EOL)

EEPRU work – approach and findings (ii)

- Results suggested some support for BOI. Excluding respondents "misunderstanding" the DCE task (remaining sample 2247) had positive, significant and robust BOI coefficients
- Using the marginal rate of substitution to estimate weights indicated that 1 unit of BOI is equivalent to 0.04 QALYs gained, and EOL is equivalent to 3.331 QALYs gained
- Robust and consistent support for EOL in general (but this conceptually overlaps with BOI and the two should not be used together)
- Overall the results indicate that a QALY is not a QALY and provide a basis for determining QALY weights.

Absolute and Proportional QALY Shortfall Definitions



Absolute QALY shortfall is total potential health going forwards (Areas A+B+C+D) minus current health prospects (Area D), i.e. Areas A+B+C.

Proportional QALY shortfall is the ratio of health lost to total potential health going forwards, i.e. Areas A+B+C as a proportion of Areas A+B+C+D.

Fair Innings (Proportional QALY shortfall from birth) is not shown in Figure 1.

Rationale

Absolute shortfall - Gavin Roberts

"The rationale behind this approach is simply that society cares about the absolute loss of quality of life and duration of illnesses. That is, larger losses of quality of life are more important than smaller losses. Longer durations of disease are more important than shorter durations of disease. Diseases which cause very premature death are more important than those which cause less premature death."

Proportional shortfall - Stolk et al (2004)

"The trouble with the [absolute shortfall] approach may be that substantial differences in health prospects may exist not only because of different illnesses, but also because of age differences. Hence, unequal health prospects may not always be considered unfair and inequitable."

Preferences and Value based pricing / assessment: where have we got to/

- EEPRU study showed incremental innovation had a higher value than breakthrough, so DH dropped it
- Operational model of EEPRU-based severity weights and DH societal value given to NICE in 2012 with DH instruction to have positive and negative effects.
- 2013 PPRS includes commitment to keep current NICE thresholds in place for 5 years (2013-18)
- NICE consults in early 2013 on “severity” weight using proportional QALY shortfall and on “social impact” weight using absolute QALY shortfall with £20K - £50K threshold range. Only positive effects. Will replace End of Life (EoL)
- October 2013 NICE announces no mandate for change. EoL will stay. NICE will discuss with DH.

Agenda

- VBP context
- Regulating pharmaceuticals
  - VBP versus VBA, PBRSA's, MIPS
- What do we value?
  - Eliciting social preferences
- **Aggregating elements of value**
- Threshold and decision making in the NHS
- Reforming the Cancer Drugs Fund
- Conclusions

A reordering of process?

Criteria: broader definition of value (risks, benefits)

Safety

Efficacy, effectiveness

Value for money (CE)

Affordability (BIA)

Other factors of value to D-M (ethical issues, social values, feasibility of implementation, unmet needs, innovation value, legal issues, ...)

Overall D-M Framework: Opportunity costs (value for money)

Source: Ron Goeree, Director PATH Research Institute, Professor, McMaster University

PATH RESEARCH INSTITUTE

Different types of judgement

Scientific judgment is usually about an effect (positive or negative), its size, the ways in which it can be achieved, for whom, for how long, ...

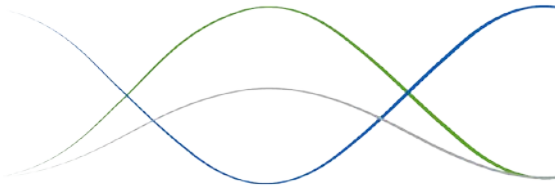
Value judgments tend to be in a different territory but they might be about, for example, how worthwhile a technology is, how defensible the tough bits of the decision are, how tolerant of uncertainty the committee ought to be, ...inter-personal comparisons ... whether the [outcome measure] was a good tracker of the relative health benefits of the interventions that were compared.

Source: A.J. Culyer. *Deliberative Processes in Decisions about Health Care Technologies.* OHE Briefing , 2009

Aggregating elements of value

- Weighting multiple criteria relevant to the decision (MCDA):
- A pure deliberative process does not use any formal structure and so is a “black box” to outsiders and potentially to itself over time (may lead to a lack of consistency and a lack of clear signals as to what matters)
- A pure algorithmic approach does not need a Committee
- Is there something workable (theoretically robust and practical) in between?

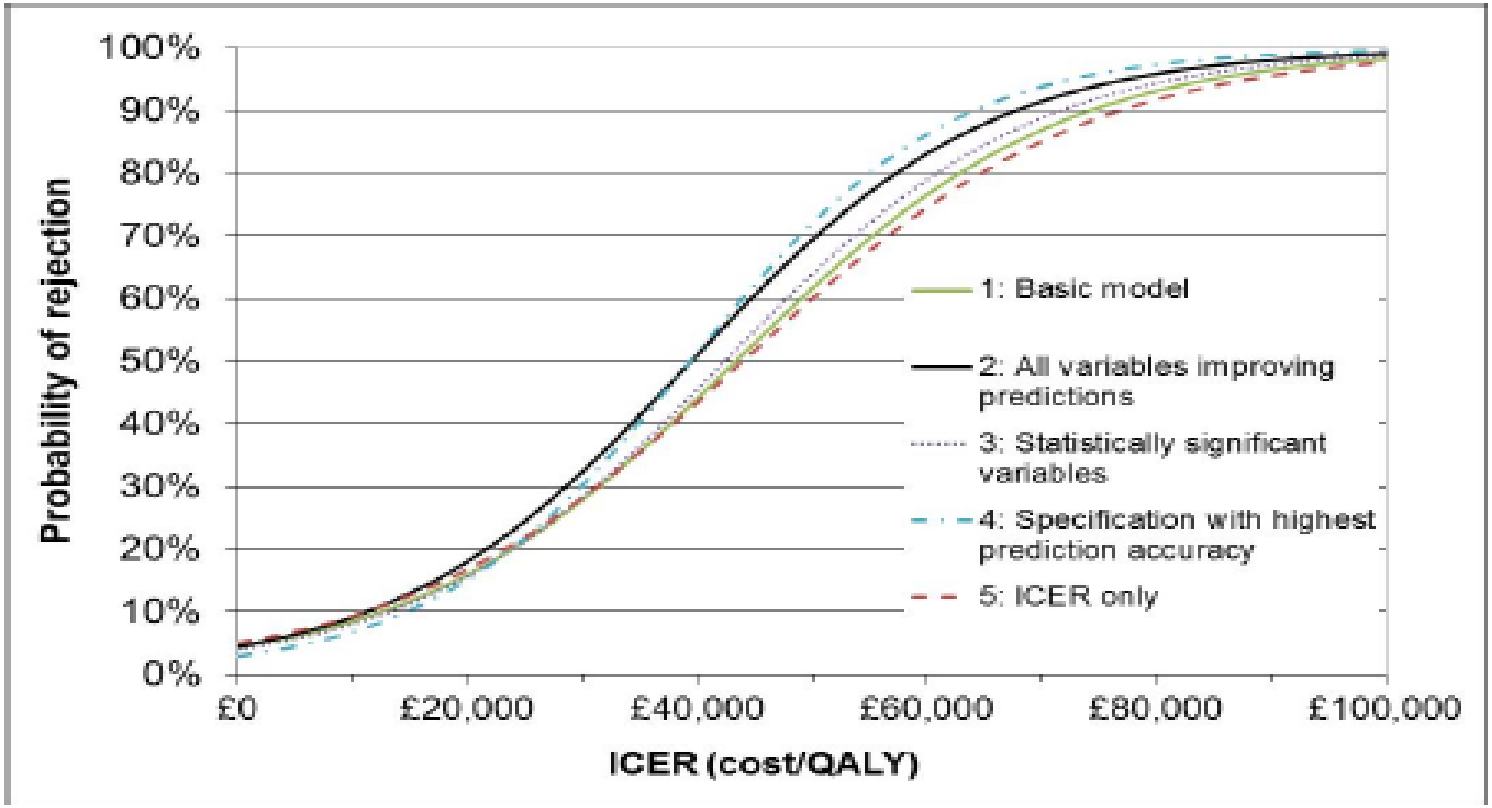
Agenda



- VBP context
- Regulating pharmaceuticals
  - VBP versus VBA, PBRSA's, MIPS
- What do we value?
  - Eliciting social preferences
- Aggregating elements of value
- **Threshold and decision making in the NHS**
- Reforming the Cancer Drugs Fund
- Conclusions

The cost-effectiveness threshold (i)

Figure 5. Predicted probability of NICE rejections at different ICER values for Models 1-5, holding all other variables at mean levels



Source: Dakin et al, OHE Research Paper, November 2013

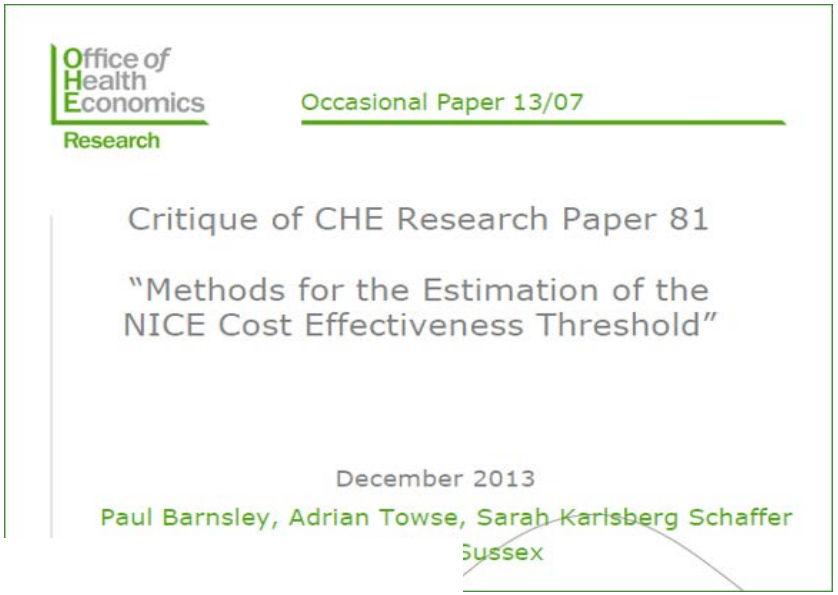
The cost-effectiveness threshold (ii)


HEALTH TECHNOLOGY ASSESSMENT

VOLUME 19 ISSUE 14 FEBRUARY 2015
ISSN 1366-5278

Methods for the estimation of the National Institute for Health and Care Excellence cost-effectiveness threshold

Karl Claxton, Steve Martin, Marta Soares, Nigel Rice, Eldon Spackman, Sebastian Hinde, Nancy Devlin, Peter C Smith and Mark Sculpher


Office of Health Economics Research

Occasional Paper 13/07

Critique of CHE Research Paper 81

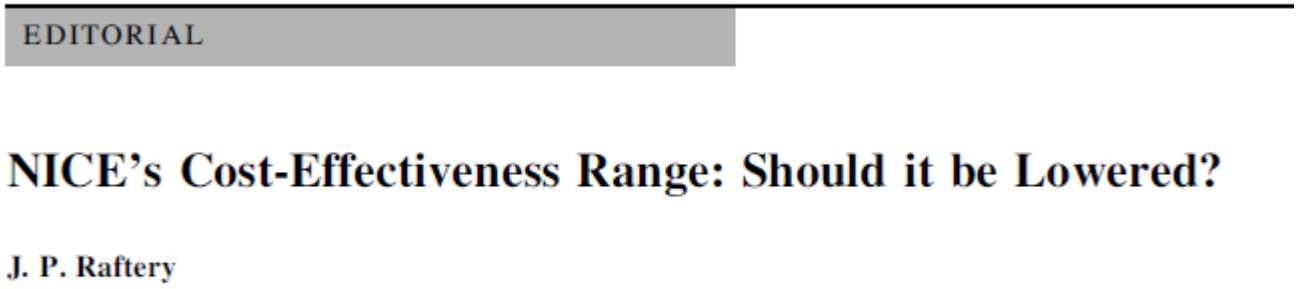
"Methods for the Estimation of the NICE Cost Effectiveness Threshold"

December 2013

Paul Barnsley, Adrian Towse, Sarah Karlsberg Schaffer

Sussex

Cla


EDITORIAL

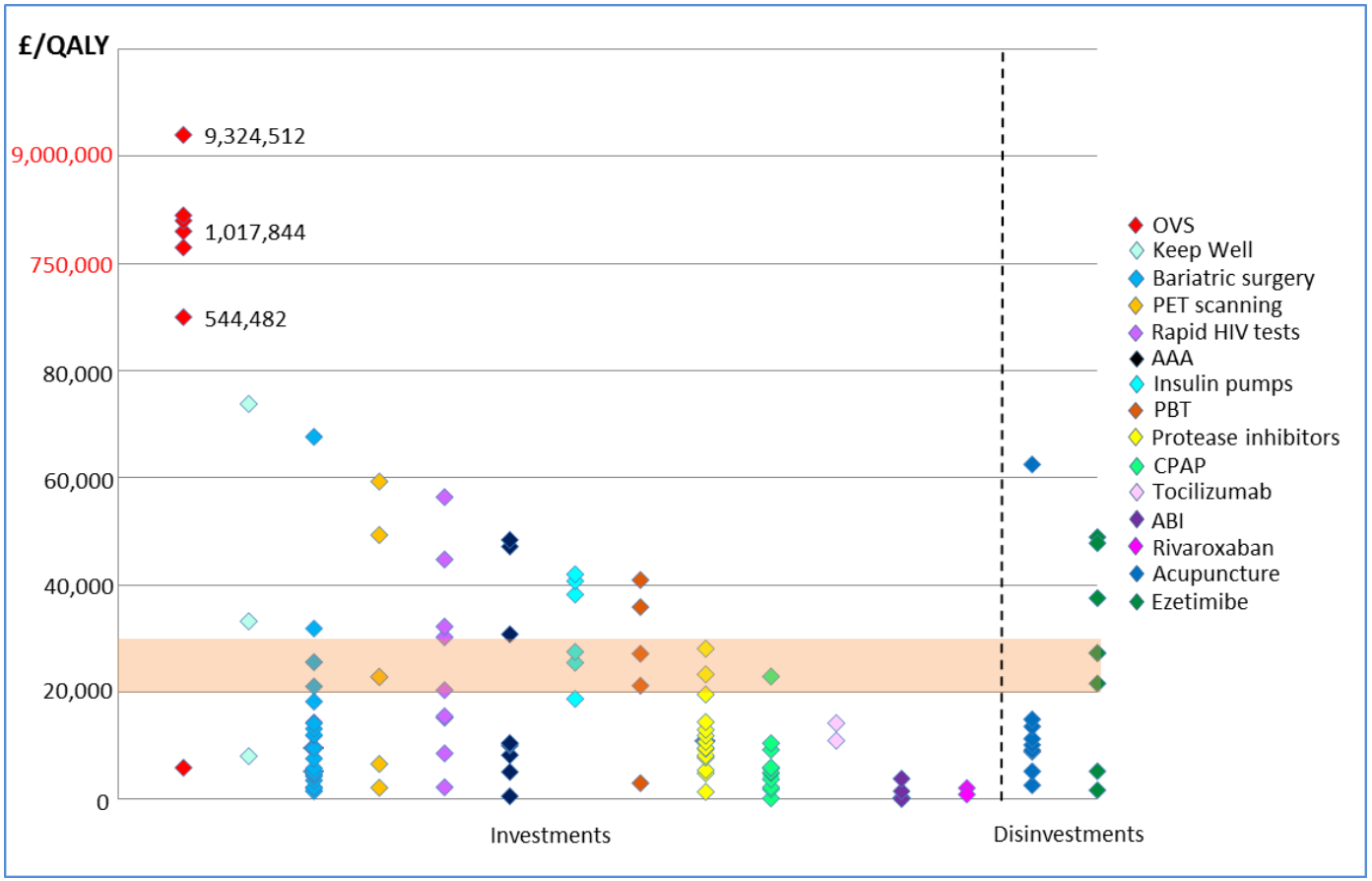
NICE's Cost-Effectiveness Range: Should it be Lowered?

J. P. Raftery

The cost-effectiveness threshold (iii)

- The DH is “unofficially” using £15K as its version of the CHERP81 £13K figure
- The PPRS guarantees NICE use the existing threshold of £20K-£30K plus up to £50K for EoL
- We are struggling to understand what an appropriate threshold might be:
  - OHE work in Scotland and Wales
  - Use of “local” PBMA and MCDA approaches
  - Better data measurement is key (PROMS?)

Reality of marginal service decisions – costs per QALY ranges



Schaffer, S.K., Sussex, J., Devlin, N. and Walker, A. (2013) Searching for cost-effectiveness thresholds in NHS Scotland. Research Paper 13/07. London: Office of Health Economics.

Agenda

- VBP context
- Regulating pharmaceuticals
  - VBP versus VBA, PBRSA's, MIPS
- What do we value?
  - Eliciting social preferences
- Aggregating elements of value
- Threshold and decision making in the NHS
- **Reforming the Cancer Drugs Fund**
- Conclusions

thebmj

BMJ 2014;349:g7276 doi: 10.1136/bmj.g7276 (Published 28 November 2014) Page 1 of 2

EDITORIALS

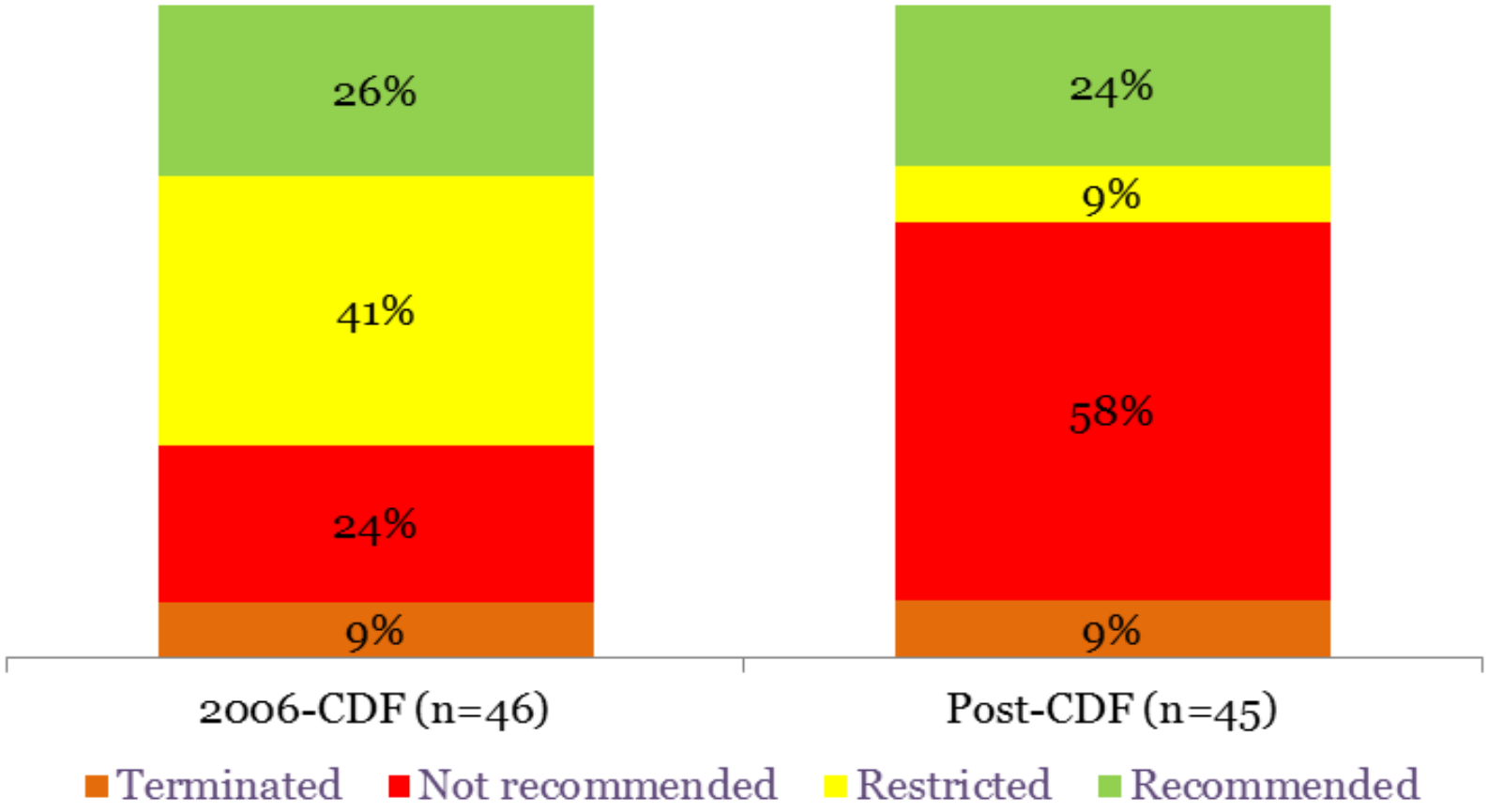
Reforming the Cancer Drug Fund

Focus on drugs that might be shown to be cost effective

Martin Buxton *emeritus professor* [1], Louise Longworth *reader in health economics* [1], James Raftery *professor of health technology assessment* [2], Mark Sculpher *professor of health economics* [3], Adrian Towse *director* [4]

- Access to cancer drugs not approved by NICE.
- Set up in 2010-11 with a budget of £50 million, increased to £200 million for next three years, and to £280m for 2014-5 and 2015-6
- Underspent for first three years, last year (2014-5) overspent
- NHS England has introduced rationing criteria for the CDF

Trends in decision for cancer medicines pre and post establishment of cancer drugs fund (Q4 2010- Q3 2013)



Source: OHE analysis from NICE website

Agenda

- VBP context
- Regulating pharmaceuticals
  - VBP versus VBA, PBRSA's, MIPS
- What do we value?
  - Eliciting social preferences
- Aggregating elements of value
- Threshold and decision making in the NHS
- Reforming the Cancer Drugs Fund
- **Conclusions**

Conclusions

1. VBA is a better way forward than VBP. Renamed VBA in 2012 PPRS agreement. So VBA is alive! (… just)
2. Work on the broader definition of value needs to continue. It requires better understanding of the preferences of the public and of patients. We need to invest in preference elicitation
3. Price flexibility by indication / subgroup and outcomes-based CED/ PBRSA schemes are important for getting dynamic and static efficiency from the use of drugs. Reform of the CDF offers a way forward to try more of these approaches
4. A deliberative process is necessary in value assessment. Introducing structure to this process (MCDA) is a challenge
5. We need to thinking about decision making in the other 90% of NHS spending. We might have a better basis for understanding the relevant cost-effectiveness threshold and improve NHS efficiency.

References

- Buxton, M., Longworth, L., Raftery, J., Schulpher, M. and Towse, A., 2014. Reforming the Cancer Drug Fund. BMJ 2014; 349 doi: (Published 28 November 2014) Cite this as: BMJ 2014;349:g7276.
- Claxton et al. Methods for the estimation of the National Institute for Health and Care Excellence cost-effectiveness threshold
- Dakin et al, OHE Research Paper, November 2013
- Danzon, P.; Towse, A.; Mestre-Ferrandiz, J 2015. Value-based Differential Pricing: Efficient Prices For Drugs In A Global Context. Health Economics Volume: 24 Issue: 3 Pages: 294-301
- Linley and Hughes. Societal views on NICE, cancer drugs fund and value-based pricing criteria for prioritising medicines: a cross-sectional survey of 4118 adults in Great Britain. Health Econ. 2013 Aug;22(8):948-64. doi: 10.1002/hec.2872. Epub 2012 Sep 7
- Rowen et al. 2014 Update: Eliciting societal preferences for weighting QALYs according to burden of illness, size of gain and end of life. EEPRU Policy Research Unit in Economic Evaluation of Health and Social Care Interventions. RR018 Date March 2014
- Raftery, J.P. (2014) NICE's cost-effectiveness range: should it be lowered? PharmacoEconomics, 32, (7), 613-615. (doi:10.1007/s40273-014-0158-6). (PMID:24811892).
- Schaffer, S.K., Sussex, J., Devlin, N. and Walker, A. (2013) Searching for cost-effectiveness thresholds in NHS Scotland. Research Paper 13/07. London: Office of Health Economics.
- Shah, K.K., Tsuchiya, A. and Wailoo, A.J., 2014. Valuing health at the end of life: A stated preference discrete choice experiment. Social Science & Medicine, 124, pp.48-56.
- Towse and Barnsley. Approaches To Identifying, Measuring, And Aggregating Elements Of Value. International Journal of Technology Assessment in Health Care Volume: 29 Issue: 4 Pages: 360-364 Published: 2013 Health Technology Assessment Volume: 19 Issue: 14